

**Assessing Self Efficacy
in Caregivers
of Children with Cystic Fibrosis**

*‘Thesis submitted in accordance with the requirements of the
University of Liverpool for the degree of Master of Philosophy
by Miss Latifa Bibi Patel.’*

August 2010

Abstract

Does the introduction of a parent reported outcome measure improve the self efficacy of caregivers of children with cystic fibrosis?

Background: *Parent Reported Outcome Measures (PROM) are an integral component of cystic fibrosis (CF) care yet there is little evidence supporting the role of PROM and their benefits. We are conducting a single centre pilot study to assess the impact of a PROM on the Self Efficacy (SE) of caregivers of children with CF.*

Aims: *The aims of this study were to explore the feasibility and benefits of introducing the Challenges of Living with Cystic Fibrosis-Questionnaire (CLCF-Q) into clinical practice. The anticipated outcome was an increase in the SE of the caregivers.*

Methods: *We are conducting a parallel randomised controlled intervention pilot study using the Cystic Fibrosis Self Efficacy-Questionnaire (CFSE-Q) as an outcome measure. All participants completed the CFSE-Q at 2 time points. Between these time points the intervention group completed the CLCF-Q as part of their annual assessment. They received feedback at their next clinic appointment from the team in the form of colour coded tables illustrating both the positive and negative issues raised in the CLCF-Q. They were invited to talk to members of the CF team and others (consultants, CF nurses, dieticians, physiotherapists, psychologists, pharmacists, family doctors and the school) about the issues raised in the CLCF-Q. Select participants (n=1) were also invited to participate in narrative interviews.*

Results: *Preliminary data from 18 cases; of children aged 5-13 years (11♂, 7♀) are reported, (8 control, 10 intervention). The SE score ranged from 26/40 to 35/40 at baseline. In the control group the SE score remained relatively static whilst the intervention group has shown an increase in SE. Baseline SE ranged from 26/40 – 39/40 using only validated items and 36/56 – 53/56 including non-validated items. Average change in SE was -0.1 in the control group and 1.3 in the intervention group. With the inclusion of the non-validated items, average increase in SE was 0.8 in the control group and 4.2 in the intervention group. The control group showed consistent improvement in 4 items and a decline in 3 items. In comparison the intervention group showed consistent improvement in 8 items and decline in only 1 item.*

Conclusions: *The CF team play a significant role in the lives of families of children with CF. They are recognised as invaluable by caregivers and are the first port of call when faced with a challenge. Potentially, the PROM CLCF-Q may have an important role in the annual assessment process. As well as extracting clinical data it raises unidentified concerns which may alert the CF team to otherwise un-recognised issues. A favourable consequence of routinely introducing the CLCF-Q into the annual assessment process may be an increase in the caregiver's SE through improved communication between the caregiver and the CF team.*

Table of Contents

Abstract.....	ii
Table of Contents.....	iii
List of Figures.....	vii
List of Tables.....	viii
List of Abbreviations.....	ix
Preface.....	x
Acknowledgements.....	xii

Chapter One: Introduction.....	1
1. The History of Cystic Fibrosis.....	3
1.1. Discovering Cystic Fibrosis.....	4
1.2. The Changing Methods of Diagnosing Cystic Fibrosis.....	6
1.3. The Significant Developments in Managing Cystic Fibrosis.....	7
1.3.1. The Four Main Concepts in Managing Cystic Fibrosis.....	8
1.3.1.1. Respiratory Relief.....	8
1.3.1.2. Maintaining Nutrition.....	8
1.3.1.3. Antibiotic Relief.....	8
1.3.1.4. Suppression of Inflammation.....	9
1.3.2. The Psychological Impact of Cystic Fibrosis.....	9
2. Cystic Fibrosis Today.....	10
2.1. Epidemiology of Cystic Fibrosis.....	11
2.2. Aetiology of Cystic Fibrosis.....	12
2.3. Diagnosing and Screening Cystic Fibrosis.....	13
2.3.1. Indications.....	13
2.3.2. Criteria.....	15
2.3.3. Newborn Screening – Immune Reactive Trypsin.....	15
2.3.4. Sweat Testing.....	16
2.3.5. Genotyping.....	16
2.3.6. Nasal Potential Difference.....	17
2.3.7. Other Methods.....	17
2.4. The Clinical Manifestations of Cystic Fibrosis.....	17
2.4.1. The Respiratory System.....	18
2.4.2. The Gastrointestinal System.....	18
2.4.3. Diabetes Mellitus and Glucose Intolerance.....	19
2.4.4. Liver Disease.....	19
2.5. Treating and Managing Cystic Fibrosis.....	19
2.5.1. The Respiratory System.....	19
2.5.1.1. Physiotherapy.....	20
2.5.1.2. Lung Transplantation.....	20
2.5.2. The Gastrointestinal System.....	21
2.5.3. Diabetes Mellitus and Glucose Intolerance.....	21
2.5.4. Liver Disease.....	21
2.5.5. Complications.....	21

2.6. Organisation of Cystic Fibrosis Care.....	22
2.6.1. Shared Care of Cystic Fibrosis.....	22
2.6.2. The Annual Assessment Process.....	22
<hr/>	
Chapter Two: Literature Review.....	25
3. Parent Reported Outcome Measures and Quality of Life.....	26
3.1. Defining Quality of Life.....	27
3.2. Measuring Health Related Quality of Life using Patient Reported Outcome Measures.....	27
3.2.1. The Utility Model.....	28
3.2.2. The Health Profile Model.....	28
3.2.3. The Disease Specific Model.....	28
3.3. Review of Commonly used Patient Reported Outcome Measures.....	29
3.3.1. Summary of Studies.....	29
3.3.2. Critical Appraisal.....	32
3.3.3. Analysis of Methods Employed.....	38
3.3.4. Analysis of commonly used Patient Reported Outcome Measures.....	39
3.4. The Challenges of Living with Cystic Fibrosis Questionnaire – A Parent Reported Outcome Measure.....	41
<hr/>	
4. Self Efficacy.....	44
4.1. The Concept of Self Efficacy.....	45
4.2. Influencing Self Efficacy.....	46
4.2.1. Performance Accomplishments.....	46
4.2.2. Vicarious Experience.....	46
4.2.3. Verbal Persuasion.....	47
4.2.4. Physiological States.....	47
4.3. Influencing Self Efficacy – In Practice.....	47
4.4. Assessing Self Efficacy.....	48
4.5. The Role of Self Efficacy in Stressful Situations.....	48
4.6. The Role of Self Efficacy in Promoting Quality of Life.....	50
4.7. The Role of Self Efficacy in Caregivers of Children with Cystic Fibrosis.....	51
<hr/>	
5. Chapter Three: Does the introduction of a Parent Reported Outcome Measure Influence the Self Efficacy of caregivers of children with cystic fibrosis?.....	54
5.1. Introduction.....	55
5.2. Objectives.....	58
5.2.1. Aim.....	58
5.2.2. Hypothesis.....	58
5.3. Methodology.....	59
5.3.1. Administrative Organisation.....	60
5.3.2. Ethical Approval.....	60
5.3.3. Study Population.....	60
5.3.3.1. Selection Procedure.....	60
5.3.3.1.1. Principal Inclusion Criteria.....	60
5.3.3.1.2. Principal Exclusion Criteria.....	61
5.3.3.2. Recruitment Procedure.....	61
5.3.3.3. Randomisation Process.....	61
5.3.4. Experimental Design.....	62
5.3.4.1. Narrative Interviews.....	62
5.3.5. Study Intervention.....	64
5.3.6. Study Intervention Points.....	65

5.3.7. Interview Schedule.....	65
5.3.8. Scoring the Challenges of Living with Cystic Fibrosis-Questionnaire.....	66
5.3.9. Scoring the CFSE-Q.....	67
5.3.10. Statistical analysis.....	67
<hr/>	
5.4. Results.....	68
5.4.1. Population Data.....	69
5.4.2. Quantitative Data.....	71
5.4.2.1. Cystic Fibrosis Self-Efficacy-Questionnaire.....	71
5.4.2.1.1. Item Analysis.....	71
5.4.2.1.1.1. Analysis of Validated Items in the CFSE-Q.....	71
5.4.2.1.1.2. Analysis of Non-Validated Items in the CFSE-Q.....	76
5.4.2.1.2. Analysis of all Items in the CFSE-Q.....	79
5.4.3. Qualitative Data.....	83
5.4.3.1. Challenge of Living with Cystic Fibrosis-Questionnaire.....	83
5.4.3.2. Interview Analysis.....	89
<hr/>	
Chapter Five: Discussion.....	95
5.5. Discussion.....	96
5.6. Limitations.....	99
5.7. Conclusion.....	100
<hr/>	
6. References.....	103
<hr/>	
7. Appendix.....	113
<hr/>	
7.1. Study Information.....	114
7.1.1. Protocol.....	114
7.1.2. Summary and Flow Diagram of Study.....	126
7.1.3. Interview Schedule.....	128
<hr/>	
7.2. Information for Caregivers – Final Versions.....	130
7.2.1. Letter to Parents, Caregivers and General Practitioner.....	130
7.2.2. Participant Information Sheet.....	131
7.2.3. Participant Consent Form.....	134
7.2.4. Participant Information Sheet – Interview Only.....	135
7.2.5. Participant Consent Form – Interview Only.....	138
<hr/>	
7.3. Study Questionnaire – Final Versions.....	139
7.3.1. Cystic Fibrosis Self Efficacy-Questionnaire.....	139
7.3.2. The Challenges of Living with Cystic Fibrosis-Questionnaire.....	142
7.3.3. Self-Feedback Form – Caregiver Copy.....	152
7.3.4. Feedback Form – Researcher Copy.....	155
7.3.5. Study Feedback Form.....	159
<hr/>	
7.4. Letters of Correspondence.....	160
7.4.1. Approval Letter for Governance Officer – University of Liverpool.....	160

7.4.2. Letter from Statistician – University of Liverpool.....	162
7.4.3. Approval Letter from Research and Development – Alder Hey.....	163
7.4.4. Validation Letter from Research Ethics Committee – Liverpool East.....	165
7.4.5. Provisional Approval from Research Ethics Committee – Liverpool East.....	168
7.4.6. Approval Letter from Research Ethics Committee – Liverpool East.....	172
7.5. Publications.....	175
7.5.1. Components of risk associated with home intravenous antibiotic therapy (H-IVAT) in the UK.....	175
7.5.2. The impact of pulmonary exacerbation on the challenge of living with cystic fibrosis.....	176
7.5.3. Does the introduction of a Parent Reported Outcome Measure Influence the Self Efficacy of caregivers of children with cystic fibrosis?.....	177

List of Figures

1.1. Milestones in the development of diagnosing, managing and treating cystic fibrosis.....	7
2.1. The changing median survival age since cystic fibrosis was first accurately described..	11
5.1. Study sample to date, August 2010.....	70
5.2. Self Efficacy Score at Baseline – Intervention Group.....	74
5.3. Self Efficacy Score at Baseline – Control Group.....	74
5.4. Self Efficacy Score at Baseline – Comparison.....	74
5.5. Self Efficacy Score at Endpoint – Intervention Group.....	75
5.6. Self Efficacy Score at Endpoint – Control Group.....	75
5.7. Self Efficacy Score at Endpoint – Comparison.....	75
5.8. Self Efficacy Score at Baseline and Endpoint – Intervention Group.....	76
5.9. Self Efficacy Score at Baseline and Endpoint – Control Group.....	76
5.10. Self Efficacy Score at Baseline and Endpoint – Comparison.....	76
5.11. Self Efficacy Score at Baseline and Endpoint – Intervention Group.....	78
5.12. Self Efficacy Score at Baseline and Endpoint – Control Group.....	78
5.13. Self Efficacy Score at Baseline and Endpoint – Comparison.....	78
5.14. Self Efficacy Score at Baseline and Endpoint – Comparison.....	78

List of Tables

Table 1: Mid-2003 cystic fibrosis population by age and sex.....	12
Table 2: Classes of CFTR mutations.....	13
Table 3: Presenting features in unscreened cystic fibrosis.....	15
Table 4: Levels of sweat chloride and diagnosis.....	16
Table 5: Summary of 15 studies into the use of patient reported outcome measures in clinical practice.....	29
Table 6: Study 1 –Validation of a disease-specific measure of health-related quality of life for children with cystic fibrosis.....	32
Table 7: Study 2 - Effect of disease-related pain on the health-related quality of life of children and adolescents with cystic fibrosis.....	33
Table 8: Study 3 – Quality of life in patients with cystic fibrosis: association with anxiety and depression.....	34
Table 9: Study 4 – Quality of life of children with cystic fibrosis.....	35
Table 10: Study 5 – Psychosocial problems in children with cystic fibrosis.....	36
Table 11: Disease-specific patient reported outcome measures for health related quality of life in children with Cystic Fibrosis.....	37
Table 12: Study Intervention Points.....	65
Table 13: Caregiver and Child Characteristics at Baseline.....	71
Table 14: Baseline Scores of Previously Validated Items on the CFSE-Q.....	72
Table 15: Endpoint Scores of Previously Validated Items on the CFSE-Q.....	73
Table 16: Baseline Scores of Non-Validated Items on the CFSE-Q.....	77
Table 17: Endpoint Scores of Non-Validated Items on the CFSE-Q.....	77
Table 18: Comparison of Consistent Changes in Items on the Cystic Fibrosis Self Efficacy-Questionnaire.....	80
Table 19: Challenges of Living with Cystic Fibrosis-Questionnaire.....	83

List of Abbreviations

ABPA	Allergic Bronchopulmonary Aspergillosis
ADL	Activities of Daily Living
BOC	Burden of Care
BMI	Body Mass Index
BYI	Beck Youth Inventories
CBAVD	Congenital Bilateral Absence of Vas Deferens
CF	Cystic Fibrosis
CFF	Cystic Fibrosis Foundation
CFQ	Cystic Fibrosis Questionnaire
CFQ-R	Cystic Fibrosis Questionnaire - Revised
CFQOL	Cystic Fibrosis Quality of Life
CFSE-Q	Cystic Fibrosis Self Efficacy - Questionnaire
CFTR	Cystic Fibrosis Transmembrane Regulator
CLCF-Q	Challenges of Living with Cystic Fibrosis - Questionnaire
CHQ	Child Health Questionnaire
CQOLCFS	Caregiver Quality of Life Cystic Fibrosis Scale
DIOS	Distal Intestinal Obstruction Syndrome
DM	Diabetes Mellitus
DNA	Deoxyribonucleic Acid
DNase	Deoxyribonuclease
FEV1	Forced Expiratory Volume in 1 minute
FVC	Forced Vital Capacity
FLZM-CF	Questions on Life Satisfaction - Cystic Fibrosis
GSE-S	General Self Efficacy - Scale
HADS	Hospital Anxiety and Depression Scale
HOPES	Hunter Opinions and Personal Expectations Scale
HRQOL	Health Related Quality of Life
IRT	Immunoreactive Trypsin
INR	International Normalising Ratio
MDT	Multidisciplinary Team
NBS	Newborn Screening
NPD	Nasal Potential Difference
PtRO	Patient Reported Outcome
PtROM	Patient Reported Outcome Measures
PRO	Parent Reported Outcome
PROM	Parent Reported Outcome Measures
PSE	Perceived Self Efficacy
RCT	Randomised Controlled Trial
QOL	Quality of Life
QWB	Quality of Well Being
SE	Self Efficacy
SF-36	Short Form – 36
UK	United Kingdom
WHO	World Health Organisation

*Likert scales are a type of scale to grade a statement which the subject deems favourable or not favourable. Respondents illustrate their degree of agreement towards the statement by scoring on a point scale.

Preface

“It is no sin to devise oversimplified working concepts of nature’s secrets. The scientific sinning comes with refusal to complicate such concepts to the degree dictated by irrefutable data or to discard them completely should they become untendable¹.”

- Anonymous

Chronic illness affects patients and their caregivers in many ways both physically and mentally. Unfortunately the majority of medicine aims to fulfil the needs of only one. This study concentrates on the other area which is frequently overlooked.

Over the last seven decades the management of cystic fibrosis has changed dramatically. Cystic fibrosis has progressed from a fatal childhood disease to a multisystem adult disease requiring multidisciplinary involvement. Over this period of change, the quality of life of patients and their caregivers has come under scrutiny. Consequently, increasing time and effort has been invested into the careful examination of their quality of life. Decreasing the burden of care and improving the quality of life of the caregivers and families was pivotal to this movement and is the focus of this study.

Employing quantitative and qualitative methods this study looks at the stress that the burden of caring for a child with cystic fibrosis brings to a family. Families of chronically ill children face challenges on a daily basis which are entirely foreign to the normal population. This project concentrates on the affect of these challenges on the caregivers of children with cystic fibrosis. It explores possible interventions which could ease burden.

The role of the caregiver is crucial to the well-being of the patient, particularly in paediatrics. Self efficacy was a term coined by Albert Bandura in 1977. It refers to one’s belief in one’s own ability to complete any given task. For caregivers of children with cystic fibrosis maintaining a good level of self efficacy may be important. Any change in self efficacy could affect the caregiver’s management of their child’s disease.

This study asks if it possible to improve the self efficacy of the caregiver. A recently developed and validated burden of care measure; the Challenges of Living with Cystic

Fibrosis-Questionnaire and a series of feedback tools were used to determine their role in improving the self efficacy of the caregiver and subsequently improving the care of the child with cystic fibrosis.

Latifa Patel

Acknowledgements

I would like to thank my primary supervisor Dr Kevin Southern for lending me his thoughts and ideas and allowing me to take his initial theory and expand on it. His supervision and support throughout the year including the endless e-mails and meetings were greatly appreciated. Further thanks are sent to my secondary supervisor Dr Claire Glasscoe whose passion for qualitative research was empirical to this study. I would like to thank Claire for all her support throughout the year and for passing on her enthusiasm for the Challenges of Living with Cystic Fibrosis-Questionnaire. I would also like to thank the Cystic Fibrosis Team and Mental Health Unit at Alder Hey Children's Hospital for answering my many queries and their continual guidance. Particular thanks go to Jenny, Elinor, Carol, Dr David Heaf, Dr Jon Couriel, Professor Rosalind Smyth, Dr Paul McNamara, Pamela, Alison, Ruth, Louisa, Jacqui, Holly, Christina and Clare Dixon. Alder Hey Cystic Fibrosis Trust Fund kindly helped fund myself and the study and for that I am very grateful. Most importantly my thanks are given to all the caregivers who participated in the study and all their children. Without their cooperation this study would not have been possible. Thank you all.

Chapter One

Introduction

“Understanding the life experiences of family members caring for a child with cystic fibrosis will provide the empiric underpinnings for appropriate clinical interventions².”

- Carpenter et al, 2004

This thesis builds on the theory discussed by Glasscoe *et al* introducing the Challenges of Living with Cystic Fibrosis-Questionnaire (CLCF-Q) as an intervention³. The effect, of introducing this Parent Reported Outcome Measure (PROM) into clinical practice, on the caregiver's Self Efficacy (SE) is mapped and supported by qualitative data.

Chapter 1 outlines the history of Cystic Fibrosis (CF) and details the major developmental milestones in the diagnosis and management of CF. Chapter 2 describes the findings of the literature review outlining the role of PROM in the measurement of a patient's Quality of Life (QOL). The concept of self efficacy and its relatively novel role in the management of children with CF is detailed; more specifically the role of assessing SE in caregivers.

The latter half of the thesis concentrates on the methods applied. The mixed methodology approach and the formation of the tools used are explained in detail in Chapter 3. Chapter 4 describes the preliminary raw results observed in the study. Chapter 5 concludes the thesis bringing together the background knowledge, the original hypothesis and deducing a conclusion from the preliminary qualitative and quantitative results.

History of Cystic Fibrosis

1.1. Discovering Cystic Fibrosis

“Woe to that child which kissed on the forehead tastes salty. He is bewitched and will soon die⁴.”

- Anonymous

This North European folklore originated between the 17th and 18th century. It was believed to have described the fate of children who had inherited the previously fatal disease now known as CF⁴. “Swollen, hardened, gleaming white pancreas,” is an extract describing the product of CF. It was written in 1595 by Professor Peter Pauw from Lieden and describes the pancreas of a girl believed to be bewitched⁵. This was not an isolated case. Over the years hundreds of documented findings have been uncovered, all describing the characteristics of typical CF. Works of the late Rochholz, Rokitansky and Landsteiner in the early nineteenth century resemble examples of the complications of CF; malabsorption, pancreatic insufficiency and meconium ileus⁵⁻⁶.

The aetiology of the similarly manifesting celiac disease, was unknown until 1950⁷. Prior to this discovery, it was largely associated with the symptoms of CF. An account dated 1888, written by Samuel Gee, describes severely malnourished infants with the classical features of CF; failure to thrive, distended abdomen, intermittent diarrhoea, pale stools and histological changes to both the lungs and the pancreas^{5 8}. These cases were misdiagnosed as celiac disease.

Prior to 1938, a cornerstone in the history of CF, a diagnosis of lone CF was rare and poorly understood. Whilst Margaret Harper in Sydney described pancreatic abnormalities of 14 children both clinically and at post mortem; which she believed were consistent with those of CF⁹⁻¹⁰, Americans Blackfan and Wolbach studying the effects of vitamin A deficiency discovered histological changes in the pancreases of 11 children^{5 11}. Following several post mortems on malnourished children, CF was differentially recognised from celiac disease. It was the collective work of a number of clinicians around the world which resulted in the recognition of CF as lone disease^{6 12}.

Further reports revealed thick, sticky mucus clogging the ducts of many mucus glands in the body. This arose to the term ‘mucoviscidosis,’¹³. It was construed that the disease was a result of a genetic abnormality giving rise to pancreatic damage causing a lack of pancreatic enzymes. Disease manifestations were believed to be a result of the consequent malabsorption. This led to growth failure. Poor nutritional status was assumed to be the cause of unusually high susceptibility to pulmonary infections. Respiratory failure was the primary cause of death in young patients. Whilst, ‘cystic fibrosis of the pancreas,’ was

widely recognised it remained referred to as, ‘generalised exocrinopathy,’ due to the extent of exocrine glands involvement⁵⁻⁶. Blackfan and May later documented similar pancreatic changes in 35 infants who also carried chronic lung infections⁵. Similarly, Fanconi described recently manifesting lung histology in children whom he believed had suffered from celiac disease⁵.

Pathologist Dorothy Anderson of Babies’ and Children’s Hospital, Columbia Presbyterian Medical Centre, New York laid another cornerstone with her work in 1938 when she published, ‘Cystic fibrosis of the pancreas and its relation to celiac disease: a clinical and pathological study¹².’ Though she initially aimed to evaluate the clinical, laboratory and histological findings of children with celiac disease, her work amalgamated the works of others identifying a definite link between, ‘celiac disease,’ intestinal obstruction, intestinal and respiratory complications and the prominent pancreatic histology¹². A colleague of Andersen’s Paul di Sant’Agnese made a promising discovery in 1948. During a heat wave in New York he discovered that many infants presenting with exhaustion were diagnosed with CF¹⁴. This led to the discovery of an abnormality in their sweat. He later found a 5-fold increase in both sodium and chloride concentrations¹⁴. Where it was previously thought that the fault was in the mucus production it now emerged that this was not the case. Furthermore this discovery made way for the diagnostic sweat test we now use. This was closely followed by the recognition of the familial incidence of CF. CF portrayed a Mendelian recessive inheritance pattern and it was now widely accepted that CF was a multisystem disease affecting organs other than the pancreas^{7 14}.

In 1983 Paul Quinton identified chloride transport as defect in CF using sweat ducts¹⁵. Knowles and Boucher also recognised an increased level of sodium reabsorption in the airways¹⁶⁻¹⁷. And in 1989 *Knowles et al* found that the Nasal Potential Difference (NPD) was also abnormal in patients with CF. CF patients had abnormalities concerning both sodium and chloride¹⁶. They experienced increased sodium reabsorption and decreased chloride secretion. Since this finding NPD measuring moved from a research tool to a crucial diagnosing tool. The use of cells from sweat ducts remained a common trend and it was use of these in 1989 that changed the management of CF completely¹⁸.

In 1989 the CF gene was isolated by positional cloning¹⁸⁻²⁰. It was the first gene to be identified by positional cloning whose use was previously completely unknown. It was discovered almost simultaneously by two parties; Lap-Chee Tsui and Jack Riordan (Hospital for Sick Children, Toronto) and Francis Collins (University of Michigan)¹⁸⁻²⁰. The gene sat on chromosome 7 and at 250 kb in length coded for a protein of 1480 amino acids; the Cystic Fibrosis Transmembrane conductance Regulator (CFTR) gene^{21,22}. Over the last

century the management of CF had evolved with great pace and by the late twentieth century the disease was very well understood and controlled with the prognosis nearing the age of 50 years.

1.2. The Changing Methods of Diagnosing Cystic Fibrosis

In the early twentieth century the diagnosis of CF was entirely pathological. By 1948, elevated sodium chloride concentrations were recognised as a symptom of CF and the diagnosis moved to a fixed criterion; an elevation of greater than 60m/L and a sibling or first cousin with a diagnosis of CF. This was however overruled by significant lung or pancreatic disease. In practice the sweat test yielded difficult results and had to be carried out by allocated centres meeting national standards^{6 14}. Performed by experienced technicians it was common practice to repeat tests twice. A percentage of CF patients did have normal sweat chloride concentrations and for these patients other tests were available. Measuring NPD was first advised in 1989¹⁶.

Later in the same year, the gold standard for diagnosing CF stemmed from the identification of 2 CF mutations of 2 alleles¹⁹. By the 21st century there were over 1600 identified CF mutations though 93% of patients were identified when tested for just 86 of these²². Genetic screening fast became important and was now offered at the prenatal stage when parents were known carriers. This was carried out using amniocentesis and chronic villous sampling²³.

The introduction of newborn screening in the United Kingdom (UK) in 2004 led to an increase of cases being diagnosed post-natally²⁴. All newborn babies in the UK were screened for phenylketonuria, congenital hypothyroidism, sickle cell disorders, medium chain acyl-CoA dehydrogenase deficiency and CF. This was known as the Guthrie test. The level of Immunoreactive Trypsin (IRT) was measured in a blood spot collected at birth²⁴. IRT was raised in most CF patients. Unfortunately, the Guthrie test for CF yielded a high number of false positives (up to 5- times) and was followed by a repeat IRT level, sweat testing or genetic testing²⁵. In men, it was possible to check for sperm in the semen. Late manifestations included intestinal obstruction and liver and gall bladder function²⁵.

This text box is where the unabridged thesis included the following third party copyrighted material:

Discoveries relevant to diagnosis, clinical description, and therapy of CF.

Davis PB. Cystic Fibrosis Since 1938.
Am. J. Respir. Crit. Care Med. 2006;173(5):475-82.

Figure 1.1: Milestones in the development of diagnosing, managing and treating cystic fibrosis⁶.

1.3. The Significant Developments in Managing Cystic Fibrosis

Early treatment of CF centred mainly round palliation. It wasn't until the mid twentieth century that rudiment self assembled CF centres developed⁵. Knowledge of the disease remained primitive with little awareness of the true causes of CF. In 1954, Leroy Matthews in Cleveland centred his approach to treatment on 4 main concepts; respiratory relief, maintaining nutrition, antibiotic therapy and suppression of inflammation⁶. He addressed each of the issues individually with aggressive treatment. The results were a clear increase in survival rate. The Cystic Fibrosis Foundation (CFF) was formed the following year. This was a true milestone providing an excellent base for international communication and distribution of research findings. It enabled resources and evidence-based research to be shared amongst centres further improving treatment. The improvement in the treatment, management and survival of CF patients continued⁵.

1.3.1. The Four Main Concepts in Managing Cystic Fibrosis

1.3.1.1. Respiratory Relief

Though presently recognised as an independent component of CF resulting from a genetic mutation, lung disease was initially believed to be the result of severe malnutrition^{5,26}. Post mortems revealed that the lungs of newborns carrying the CF genotype were normal unlike the digestive tract and pancreas which were affected at birth. The only abnormality the lungs portrayed prior to infection is that of widening of the mouths of the submucosal glands²⁶. Aims of early treatment were to clear the thick, mucous secretions that often plugged the airways. This involved humidification, postural drainage and clapping. Later, as novel methods were founded the focus moved to biological products; recombinant deoxyribonuclease (DNAse), polymerised actin and mucins in aid of improving pulmonary function and preventing pulmonary exacerbations⁵⁻⁶.

1.3.1.2. Maintaining Nutrition

Eighty-five percent of CF patients are pancreatic insufficient at birth and many follow the same path during their lifetime. Bicarbonate secretion was also impaired in CF resulting in hostile conditions in the digestive tract. The formation of enteric coated microspheres which resisted the effects of gastric acid in the 1980s was a development in the treatment of pancreatic insufficiency⁵. Fat malabsorption resulted in the deficiency of fat-soluble vitamins; A, D, E and K. Thus in 1989 the introduction of vitamin supplements was recommended²⁵. The inefficient circulation of bile and increased calorific demands due to chronic lung disease resulted in the necessity of nutritional supplementation²⁷.

1.3.1.3. Antibiotic Relief

Since the introduction of antibiotics in 1944 aggressive, careful antibiotic treatment became gold standard in the prevention and treatment of lung disease²⁸. The development of oral antibiotics further radicalised treatment, though severe exacerbations were still treated intravenously. The role of routine long term use of antibiotics to prevent or lessen pulmonary exacerbations is still under debate²⁹.

1.3.1.4. Suppression of Inflammation

Following the discovery of high levels of neutrophils and interleukin-8 in infants with CF compared to controls, the significance of suppression of inflammation was recognised and promoted⁶. This was followed by the introduction of regular steroids. However they were soon replaced by ibuprofen due to the resultant levels of side-effects. Ibuprofen was later discouraged due to increased risk of gastrointestinal haemorrhage²⁵.

1.3.2. The Psychological Impact of Cystic Fibrosis

By the late 20th century the significant role of the psychologist in the management of CF was recognised, particularly in paediatrics³⁰. The impact of CF on the QOL of the child was described as a significant focus of treatment and management. There was now a secure place for the psychologist in the Multidisciplinary Team (MDT)³¹. The need to measure QOL using Patient Reported Outcome Measures (PtROM) was recognised as a necessity encouraging research in this field³². This progression has continued with an aim to improve the overall QOL of the child, the caregiver and the family³³.

Cystic Fibrosis Today

2.1. Epidemiology of Cystic Fibrosis

CF is the most prevalent, autosomal recessively inherited, life shortening disorder in the Caucasian populations with the highest incidence in Ireland³⁴. There are over an estimated 10 million carriers of the gene worldwide and figures in 2004 showed that 50 000 people had been diagnosed with CF³⁵. There are over 7000 people diagnosed with CF in the UK and on average 1 in 2500 live births are affected²².

CF was previously known as a short, painful disease of the young. In 1938 the median survival age was less than 2 years^{6 36}. By 1989 the median survival age had risen to 26.5 years and the current prognosis for infants born in the 21st century is over 30 years (Figure 2)

22 37

This text box is where the unabridged thesis included the following third party copyrighted material:

Median survival age for patients with CF at various times since the first description of CF.

Davis PB. Cystic Fibrosis Since 1938.
Am. J. Respir. Crit. Care Med. 2006;173(5):475-82.

Figure 2.1: The changing median survival age since cystic fibrosis was first accurately described⁶.

The prevalence of CF is increasing in all populations. Accordingly, the number of CF centres and research interests are increasing too. Following recent medical advances it is estimated that the number of adults with CF is comparable to the number of children with CF³⁸. *Elborn et al* published up to date survival and population estimates in 1991 when 27% of patients in England and Wales were over the age of 16³⁹⁻⁴⁰. They correctly predicted that as medical advances came into practice this figure would increase. In 2003 *Blau et al* found that 30% of patients were above the age of 18 years and this proportion rose to 35% in 2005 (Table 1)^{6 36}.

This text box is where the unabridged thesis included the following third party copyrighted material:

Mid-2003 cystic fibrosis population by age and sex

Dodge JA, Lewis PA, Stanton M, Wilsher J. Cystic fibrosis mortality and survival in the UK: 1947-2003. *Eur Respir J* 2007;29(3):522-6.

Table 1: Mid-2003 cystic fibrosis population by age and sex

The size of the 0 – 9 years age group were estimated from population births and survival data. The size of the age groups of ≥ 10 years was based on data held by the UK Cystic Fibrosis Society³⁸.

2.2. The Aetiology of Cystic Fibrosis

The CF gene codes for a cAMP-regulated chlorine channel, CFTR. CFTR is a protein regulating transport of electrolytes across epithelial cell membranes⁴¹. Since the gene was isolated, over 1600 mutations have been described²². Mutations cause a dysfunction in the CFTR gene⁴². The most prevalent mutation is seen at codon 508; the deletion of phenylalanine (phe508del) also referred to as $\Delta 508$. This mutation accounts for over 70% of the CF chromosomes carried in the UK⁴².

CF is a multisystem disease extending into adulthood affecting the respiratory, digestive, liver, pancreas and reproductive systems. Most manifestations are attributed to the mutations in the CFTR gene however some symptoms remain elusive. The release of thick, viscous secretions affects the lungs, pancreatic ducts, intestines, biliary tree and vas deferens. The consequences include; irreversible lung pathology, bronchiectasis, chronic sinusitis, nasal polyposis, pancreatic insufficiency, intestinal obstruction, biliary cirrhosis and Congenital Bilateral Absence of the Vas Deferens (CBVAD) causing male subfertility⁴¹.

The spectrum of disease caused by the different mutations is wide. Respiratory, pancreatic and digestive functions all vary in severity with mutations carried (Table 2). Some functions remain normal. The mildest form of CF is found in males with no clinical CF yet CBAVD. Heterozygote carriers of CF are at an increased risk of pancreatitis, sinusitis and

Allergic Bronchopulmonary Aspergillosis (ABPA)⁴¹. Environment and therapeutic interventions can have an equal weight on disease outcome as genetic predisposition. Examples of environmental factors include exposure to tobacco smoke, poor socioeconomic status and lack of healthcare facilities^{25 43}.

Class	Effect on CFTR	Type of Mutation	CF Patients (Europe- %)	Potential Therapy
I	Defective synthesis of message causing absence of CFTR	Premature stop codon - either nonsense or frame shift (i.e. W1282X or G542X)	7	Aminoglycosides Gene transfer
II	Abnormal CFTR produced which fails to leave endoplasmic reticulum	Amino acid deletion or missense (i.e. ΔF508)	85	Butyrates Gene transfer
III	Abnormal CFTR causing disruption of activation and regulation at cell membrane	Missense mutation (i.e. G551D)	<3	Genistein Gene transfer
IV	Abnormal CFTR, reducing chloride conductance	Missense mutation (i.e. R117H or R347P)	<3	Milrinone Gene transfer
V	Reduced or absent synthesis of CFTR due to decreased splicing of normal CFTR	Missense mutation or splice site mutation (i.e. A445E or 5T)	<3	Gene transfer

Table 2: Classes of CFTR mutations adapted from *Peebles et al*²⁵.

2.3. Diagnosing and Screening Cystic Fibrosis

Early diagnosis and treatment are essential in CF. Aims of treatment are to prevent and reduce irreversible damage and prolong a good quality of life. Screening identifies most of the cases of CF however false negatives are apparent and cases may go undiagnosed. Due to variations in genotype and phenotype these patients may present with different manifestations to other specialities²⁷.

2.3.1 Indications

All newborn babies in the UK are screened for CF at birth. Those missed on screening are identified following presentation. Symptoms found at presentation include^{5 25 27};

1. Respiratory symptoms
 - a. Chronic or recurrent cough
 - b. Difficult asthma (20%)

- c. Purulent sputum
 - d. Chest infection
 - e. Nasal polyps
 - f. Chronic sinusitis
 - g. Pneumonia
 - h. Unexplained haemoptysis
2. Gastrointestinal symptoms
- a. Meconium ileus, meconium plug syndrome or echogenic bowel
 - b. Prolonged neonatal jaundice, neonatal hepatitis syndrome
 - c. Offensive diarrhoea and/or steatorrhoea (including Toddler's diarrhoea)
 - d. Failure to thrive
 - e. Rectal prolapse
 - f. Atypical gastro-oesophageal reflux
 - g. Biliary cirrhosis, portal hypertension
 - h. Hypoproteinaemia
 - i. Anaemia
 - j. Oedema
3. Other symptoms
- a. Bulging fontanelle and facial palsy (vitamin A deficiency)
 - b. Salty tasting sweat
 - c. Heat exhaustion
 - d. Rapid and prolonged skin wrinkling
 - e. Short stature
 - f. Delayed puberty
 - g. Clubbing
 - h. Appendicitis
 - i. Pseudo-Bartter's Syndrome
 - j. Male infertility (CBAVD)
 - k. International Normalising Ratio (INR) prolongation and abnormal bleeding caused by vitamin K deficiency

Presenting feature	Percentage
Gastrointestinal	30
Respiratory	25
Gastrointestinal and respiratory	15
Meconium ileus	15
Family history	10
Other	5

Table 3: Presenting features in unscreened Cystic Fibrosis²⁵.

2.3.2. Criteria

CF is diagnosed following the presence of;

1. Typical features as outlined above
and
2. Positive screening and/or a positive sweat test
and/or
3. The identification of 2 gene mutations

Whilst recognising typical CF the criteria also identifies patients with symptoms suggestive of CF who don't fit the criteria but still require treatment⁴¹.

2.3.3 Newborn Screening - Immune Reactive Trypsin

In 1979, a radioimmunoassay for IRT was developed²⁷. It allowed analysis of dried blood spots. This method was employed in 2001 when the Department of Health recommended the introduction of Newborn Screening (NBS) for CF. NBS tests for IRT levels in blood taken from newborn babies as part of the Guthrie Test²⁷. NBS came into practice in England in 2006⁴⁴. It reduces early hospitalisation and parental stress⁴⁵. Early accurate diagnosis through NBS also encourages a good relationship with the CF team^{24 46}.

In the first few weeks of life newborn babies with CF produce up to 5 times greater

concentrations of IRT than the normal population. Over the next few months this discrepancy is corrected. Unfortunately, NBS yields a significant number of false positives as there is a similar rise in the level of IRT in the first week of life in the normal population.

2.3.4 Sweat Testing

In the normal population, all sweat electrolytes are raised in the first 48 hours of life. This level falls over the next 7 days. In CF, sodium and chloride levels remain high¹⁴. The level of chloride is proportionally greater than the level of sodium and 98% of patients have a significantly higher level of chloride. Sweat testing assesses the level of chloride specifically⁴⁷. The process used is pilocarpine iontophoresis. Currently, two methods are employed; the Gibson and Cooke method and the Wescor Macroduct system⁴⁸.

Level of Chloride Detected	Diagnosis
< 40 mmol/L	Normal
40 - 60 mmol/L	Borderline
> 60 mmol/L	Cystic Fibrosis
> 150 mmol/L	Error

Table 4: Levels of Sweat Chloride and Diagnosis²⁵.

Sweat testing is unpopular in young children as obtaining the required amount of sweat can be difficult. Preterm babies may not sweat for a few weeks and young children produce lower quantities of sweat⁴⁸.

2.3.5 Genotyping

Genotyping is gold standard for diagnosis. Once Deoxyribonucleic Acid (DNA) is obtained from mouthwash samples, buccal scrapes or white blood cells²⁷ samples are tested for the most common CFTR mutations. The identification of 2 CFTR mutations supports a diagnosis of CF. If only 1 CFTR mutation is detected despite high suspicions rarer mutations are tested for²³. Due to the large and varied number of mutations, genotyping can lack sensitivity and may need repeating for rarer mutations. Genotyping is carried out in patients who have been diagnosed through other methods, for confirmation. It also provides

further information regarding their phenotype⁴¹. It is common practice to check the genotype of all siblings following the positive diagnosis of a child⁴⁹.

2.3.6. Nasal Potential Difference

Due to the electrolyte imbalances described above, patients with CF have a greater negative NPD. NPD across the respiratory epithelium is measured by inserting probes in both nasal cavities following the instillation of ionic solutions. Whilst the test is specific it requires expertise and is only carried out in specialist centres. It is also sensitive to slight changes caused by infections, surgery and allergic rhinitis, producing false positives⁴¹.

2.3.7. Other Methods

Other tests are available to confirm suspicions if routine tests are inconclusive. These include; sinus radiographs, bronchoalveolar lavage, tests for exocrine pancreatic function, tests for malabsorption and semen analysis^{23 41}. These tests also help recognise CF related disease including CBAVD⁵⁰.

In 2000 the World Health Organisation (WHO) released a new classification recognising CF related disease^{25 45 51}:

1. Classic CF (pancreatic insufficient)
2. Classic CF (pancreatic sufficient)
3. Atypical CF
4. CF (otherwise specified)
5. CF (not otherwise specified)
6. Isolated obstructive azoospermia (1 CFTR mutation)
7. Chronic pancreatitis (1 CFTR mutation)
8. Allergic bronchopulmonary aspergillosis(1 CFTR mutation)
9. Disseminated bronchiectasis(1 CFTR mutation)
10. Diffuse panbronchiolitis(1 CFTR mutation)
11. Sclerosing cholangitis(1 CFTR mutation)
12. Neonatal hypertrypsinogenemia

2.4. The Clinical Manifestations of Cystic Fibrosis

CF manifestations can be unique to individuals due to variations in phenotype. Symptoms can vary significantly between individuals and treatments are tailored to their specific needs⁵⁰.

2.4.1. The Respiratory System

Lung disease is the main cause of morbidity and mortality⁵². Ninety seven percent of CF patients die of respiratory causes. Twenty percent of patients have coexistent asthma and atopy whilst a further 10% develop ABPA^{27 53}.

Symptoms include an increased frequency and severity of coughing, an increase or change in the colour of sputum, increased shortness of breath, poor exercise capacity, haemoptysis, anorexia and weight loss⁵. These are detected clinically by a decrease in lung function, auscultation and changes on chest X-ray.

Patients with CF encounter the same number of infections as the normal population but they are more likely to be symptomatic. Bacterial infections are found in patients at an early age and aggressive antibiotic therapy is required³⁶. The lack of water in secretions and the viscosity of mucus in airways contribute to the risk of infection. It is the repeated cycle of infections which leads to the colonization of organisms in the lungs. This causes the clinical respiratory features of CF. *Staphylococcus aureus* and *Haemophilus influenza* are isolated early. *Pseudomonas aeruginosa* may be found at later stages in the disease²⁷. This is followed by a marked decrease in lung function. *Burkholderia cepacia*, is a pan-antibiotic resistant strain of bacteria with the potential to cause rapid pulmonary decline and can lead to death⁵⁴. Repeated infection results in a persistent neutrophilic inflammatory response and an increased volume of secretions. These eventually impact areas of the respiratory tract and destroy the walls^{25 55}. To avoid colonisation CF patients are regularly screened for respiratory infections.

2.4.2. The Gastrointestinal System

Maintaining a good nutritional status in patients is imperative to treatment. Eighty five percent of CF patients are pancreatic insufficient. And despite recent advances approximately 20% of children and 40% of adults are diagnosed with nutritional failure²⁷.

Thirty percent of patients not diagnosed at birth, present with gastrointestinal symptoms including meconium ileus or other forms of intestinal obstruction and recurrent rectal prolapse^{25 27 51}.

Symptoms commonly displayed are Gastroesophageal Reflux (GOR), abdominal pain, diarrhoea and constipation. It is not uncommon for the symptoms to be a result of other treatments. Poor adherence to medication must also be considered as a cause.

2.4.3. Diabetes Mellitus and Glucose Intolerance

Prevalence of Diabetes Mellitus (DM) and endocrine pancreatic insufficiency increases above the age of twelve³⁶. Pancreatic fibrosis leads to further decrease in insulin producing beta cells. A good history enquiring about symptoms of general malaise, polydipsia, polyuria and weight loss are necessary. It is good practice to monitor blood glucose, glucose tolerance, urinalysis and glycosylated haemoglobin annually.

2.4.4. Liver disease

Liver disease naturally develops in the first 2-10 years in 18-37% of patients. Five percent of these go on to develop cirrhosis and portal hypertension, a further 2.5% develop variceal bleeding and 2-3% require liver transplantation^{25 27}.

The abnormal CFTR is located on apical membranes of epithelial cells lining intrahepatic ducts leads to bile duct obstruction and destruction causing focal biliary cirrhosis. CF can also cause, fatty infiltration leading to secondary hepatomegaly, gallstones, liver cirrhosis, portal hypertension causing chronic liver disease⁵⁶. Patients present with acute variceal haemorrhage, haematemesis, aspiration and respiratory deterioration⁵.

2.5. Treating and Managing Cystic Fibrosis

Management of CF requires prompt MDT communication and involvement. The CF team consists of consultants, nurses, dieticians, physiotherapists, pharmacists, psychologists and most importantly the caregivers and family of the patient⁵⁷.

2.5.1. The Respiratory System

Respiratory failure due to lung damage is the main cause of premature death in CF⁶. Respiratory treatment aims to prevent long term irreversible lung damage. This requires careful prevention, early detection and eradication of organisms to delay chronic colonisation. It involves thorough, regular surveillance including, detailed histories, monitoring lung function including Forced Expiratory Volume in 1 minute (FEV₁) and Forced Vital Capacity (FVC), regular sputum culturing and taking chest X-rays. Symptoms are reviewed at 2 – 3 month intervals

Four areas of management include efficient airway clearance, administration of antibiotics, maintaining a good nutritional status and physical fitness. This involves careful delivery of antibiotics, anti-inflammatories, mucolytics and physiotherapy^{25 58}.

Whilst *Staphylococcus aureus* and *Haemophilus influenza* are the main source of primary infection, 80% of adults with CF are chronic carriers of *Pseudomonas aeruginosa*²⁷. Pathogen specific antibiotics are administered both orally and intravenously for a period of 10 to 14 days.

Treatment includes respiratory physiotherapy, inhaled nebulisers and steroids and oral and intravenous antibiotics. The progressive nature of CF results in the need for continual invasive treatment. This can impact negatively on the patient, their family and their caregiver's lives⁵⁷.

2.5.1.1. Physiotherapy

Patients are referred to the physiotherapy team at diagnosis for information regarding physiotherapy regimes and support. The physiotherapist is an important member of the CF team and must be included in the treatment of any inpatient.

Physiotherapy aids airway clearance, promotes exercise and maintains mobility and posture. Airway clearance reduces airway obstruction, improving ventilation and delaying disease progression. Exercise improves cardiovascular and respiratory fitness and strength, whilst ultimately improving lung function. Whilst stretching and strengthening exercises maintain and restore good posture^{25 59}.

Patients are reviewed at 2 – 3 month periods. This includes an assessment of their disease status, sputum collection, a review of airway clearing techniques, advice on general fitness and mobility and posture. All patients are seen annually as part of the annual assessment. This includes a complete demonstration of airway clearing techniques, exercise tolerance tests and inhaler and nebuliser technique review⁶⁰.

2.5.1.2. Lung transplantation

Lung transplantation offers a new lease of life for those with lung failure. The first lung transplantation on record was performed in 1983. At present more than 200 people received

new lungs each year. The current survival rate is 80% at one year, 64% at three years and less than 50% by year 4^{25 27}.

2.5.2. The Gastrointestinal System

The concept of prevention, detection, slowing progression and preventing complications also apply in the treatment and management of gastrointestinal manifestations. Regular surveillance includes monitoring height and weight, abdominal examination for organomegaly, abdominal ultrasound scans, liver function tests and monitoring faecal elastase levels⁴⁶.

Maintaining nutrition is vital. CF patients require up to 150% of the normal calorie intake due to increased respiratory efforts⁶¹. This is achieved through an increased intake of high calorific foods where possible and supplemented with calorie powders and glucose drinks where required. Due to fat malabsorption and maldigestion the fat soluble vitamins A, D, E and K are supplemented alongside minerals and sodium. Irregular bowel habits are managed through correcting dehydration, using oral lactulose, gastrograffin, laxatives and Pancreatic Enzyme Replacement Therapy (PERT). Symptoms of GOR are managed with antacids, and Proton Pump Inhibitors (PPI), prokinetics and changing bowel habits²⁵. Where conventional nutritional encouragement fails it may be necessary to introduce overnight feeding in the form of enteral nutrition; nasogastric or gastrostomy. This is usually introduced during pubertal years to maximise growth potential⁵².

2.5.3. Diabetes Mellitus and Glucose Intolerance

Twelve percent of patients over the age of 13 have Type I DM and the prevalence increases with age at a rate of 5% per year²⁵. Patients are encouraged not to conform to the normally recommended diabetic diet due to the increase calorific requirement though a reduction in sugar intake is advised. CF related DM responds well to insulin^{27 62}.

2.5.4. Liver Disease

Symptoms of liver disease are initially relieved by ursodeoxycholic acid. Hepatic steatosis and gallstones occur in 15% of patients and are treated with surgery. Liver transplantation provides permanent relief^{25 56}.

2.5.5. Complications

Complications of CF include nasal polyps, sinusitis, anosmia, musculoskeletal problems

which eventually affects 14% of patients, delayed puberty, subfertility problems and difficulties during surgical, anaesthesia and analgesia^{5 52}.

2.6. Organisation of Cystic Fibrosis Care

Most patients are managed at local specialist centres. Patients may be seen annually at major specialist centres which have a dedicated team, training and research specific to the management of CF. National resource centres specialise further and carry out transplantations. All patients are ideally seen every 2 months for routine investigations and prescription changes. Regular prescriptions may be repeated by general practitioners.

2.6.1. Shared Care of Cystic Fibrosis

Shared care brings together information about patients at a local and national level in order to alleviate travel and expense burden on the patient's family. Whilst patient care is based at one centre referrals may occur to other specialist centres. Joint clinics allow specialist staff from larger centres to attend peripheral clinics at a regular basis. Despite joint clinics patients should still be seen at specialist centres regularly.

2.6.2. The Annual Assessment Process

Annual assessments take place once a year and provide significant information relating to the treatment and management of the patient. They include height and weight measurements, full blood counts, liver function tests, urea and electrolytes, glucose tolerance tests in patients over the age of twelve, abdominal ultrasound, posterior-anterior chest X-ray, audiology tests, lung function tests, exercise tolerance testing, review of airway clearance techniques, review of inhaler and nebuliser techniques, physiotherapist assessment and assessment of nutritional status by a dietician. The assessment can last up to 4 hours and usually takes place at a national resource centre or major specialist centre. Much of this time is spent in waiting areas and the experience can be tedious and unwelcome by the child and the caregiver.

Feedback of the results occurs at the next outpatient appointment which is followed by a detailed letter outlining the results, any discussion that took place at the appointment and any change in treatment. Results and changes are compared to the outcome of the previous year's annual assessment. Any change in treatment is discussed at the MDT meeting.

Chapter Two

Literature Review

The literature search was conducted on OvidSP using the Ovid MEDLINE(R) (1948-2009) database. An advanced search was conducted to locate articles with the key words, “Cystic fibrosis,” and “Quality of life,” in their titles and abstracts. The Boolean operators, ‘AND,’ OR,’ and ‘NOT’ were used to ensure the inclusion and exclusion criterion was fulfilled. The inclusion criteria ensured that all studies; 1) discussed the topic of QOL in CF paediatric patients, 2) were on humans, 3) were available in English. Within this selection a sub category of articles which included the terms, “Questionnaire,” OR, “Outcome measure,” OR, “Patient reported,” OR, “Parent reported” was also included. A number of keywords were, ‘exploded,’ to allow for the international variation of terms used.

The information extracted from this section of the literature search was used to both signify the effect of CF on the QOL of the caregiver and to evaluate the need for a robust and accurate method of measuring QOL in patients and caregivers. The latter part of the literature review focussed on the properties of a good PROM and was used to inform the methodology of this study. The results of fifteen primary studies were mapped to compare outcome. Five studies were further appraised to assess the methods employed and the properties of the questionnaires developed or adapted. Studies were selected from the search results in accordance to the validity of the methods employed and the choice of the outcome measure. The pre-existence of a number of tools available to measure QOL in children with chronic disease and CF specifically was noted. The lack of such tools applicable to their caregivers was apparent. Specifically, a gap in studies looking at QOL in caregivers was identified.

The second half of this chapter focuses on the concept of SE, its role in caregivers of children with CF and the possible benefits of equating SE and mapping change in relation to disease management. SE is not a novel concept. But research relating SE to the role of caregivers is relatively recent. Research concentrating on caregivers of children with chronic disease is further finite. There is no current literature connecting SE in caregivers to the management of CF in children.

This literature search was less succinct due to the lack of specific literature available. The Ovid MEDLINE(R) (1948-2010) database was used to find articles containing the term, “Self efficacy,” in the title. The research was further refined by combining the search with the term, “caregiver” in the article body. To allow for international variance of terms the MeSH heading, “Caregiver” was exploded to include a further 14 terms. Articles were then

further subdivided to those including the term, “chronic disease” and “paediatric.” Articles located at each stage of this particular search thread were saved. The inclusion criteria included 1) the language English and 2) human subjects.

‘Full texts’ for all articles were located using Ovid MEDLINE(R) (1948-2010) and the University of Liverpool Electronic-Journal Database and transferred to Endnote. To assess for congruence identical searches were conducted on Scopus and ScienceDirect. All articles were further reviewed, analysed and critically appraised with regards to the validity of their results. The critical appraisal tool used is an adapted format of a tool proposed by *Guyatt et al*⁶³. This tool offers a simple yet robust method of analysis of studies whilst allowing easy comparison between methodology and conclusion.

As stated there is no current published data available assessing the role of SE in caregivers of children with CF and mapping the changes against disease management. Whilst the use of PROM has been exhausted in previous years their impact on caregivers and their ability to influence SE has not been studied to date. The literature review verified this hypothesised gap in recent studies.

Parent Reported Outcome Measures and Quality of Life

3.1. Defining Quality of Life

Caring for a child with CF can be expensive, exhaustive and requires a great deal of time and patience. Involvement of the MDT is imperative to this care.

Pain and discomfort are side effects of the disease and its treatment; headaches, chest pain, abdominal pain, back pain, limb pain, arthritis and neuropathic pain are all examples of types of pain that may be encountered. It is reasonable to assume that CF dramatically disturbs the QOL of the patient, the caregiver and the family unit including siblings.

Life expectancy of patients with CF is increasing as a result of recent medical advances. Measuring the impact of the chronic illness on patients is considered important⁶⁴. Over 3 decades ago the WHO published a definition for health,

“a state of complete physical and social well being, not just the absence of disease⁶⁵.”

Subsequently, time and resources have since been allocated to improve QOL in patients and over the past 2 decades numerous tools have been proposed to measure QOL.

Paediatric medicine aims to improve the patient's, their caregiver's and the family unit's global QOL where possible alongside stopping or slowing down disease progression. The disease and its treatment prevent the child from carrying out many Activities of Daily Living (ADL). Measuring QOL is a method of subjectively evaluating the impact of chronic disease on a patient's ADL and global well being. QOL is, *“a dynamic psychological construct, which describes the subjective health perceptions of the patients independently of objective health parameters,”*⁶⁶. It fluctuates over time and disease progression amongst other factors.

3.2. Measuring Health Related Quality of Life using Patient Reported Outcome

Measures

Over the past 25 years Patient Reported Outcomes (PtRO) have become increasingly important and greatly influence the treatment received. Considerable work has been carried out to develop tools to equate and evaluate PtRO. A (Patient Reported Outcome Measure) PtROM is,

“a measure of a patient's health status, elicited directly from the patient, that assesses how the patient feels or functions with respect to his or her health condition⁶⁷.”

Outcome measures range from items rating a single symptom to a multisystem analyses looking into the global Health Related Quality of Life (HRQOL) of the patient. HRQOL measurements in CF allow the inclusion of a patient's perspective.

HRQOL is a multidimensional construct. It includes the domains of physical functioning, social functioning and emotional and psychological functioning. They give an overall picture of the disease state incorporating otherwise untested for states. This includes anxiety and depression⁶⁸. At present, there are 3 types of PtROM commonly used to measure HRQOL⁶⁹.

3.2.1. The Utility Model

The utility model considers the economic decision theory, assessing the impact of disease and comparing the costs and value of available treatments. The Quality of Well-Being (QWB) scale is an example of this model⁶⁵. The outcome is a single value between 0 and 1 which represent overall health status. It allows cost-utility analyses to be performed and plays a major role in clinical trials⁷⁰⁻⁷¹. Utility models lack sensitivity and aren't suitable for use in paediatrics.

3.2.2. The Health Profiles Model

This represents a general health model of which the Child Health Questionnaire (CHQ) is an example. It offers reliable measurement in a wide range of disease states and has the advantage of ease. But it is non-specific and lacks sensitivity. Even with approved reliability and validity it has limited use to patients with CF.

3.2.3. The Disease Specific Model

Proceeding the discovery of the CF gene, the National Institute of Health Research stated the need for a disease specific measure of HRQOL for patients with CF⁶⁹. The resultant model would look specifically at the domains which affect CF patients and calculate their QOL accordingly. The Cystic Fibrosis Questionnaire (CFQ) is an example of this model.

From diagnosis, depending on disease stage and rate of progression, patients with CF are subject to numerous, invasive investigations and interventions⁷². Studies show that this fluctuating disease state and treatment has a direct causal influence on HRQOL^{54 73}. QOL correlates positively with lung function and improves significantly post lung

transplantation⁶⁶. There is a need to monitor HRQOL when assessing the physical health of any child with CF^{54 74}.

3.3. Review of Commonly used Patient Reported Outcome Measures

Fifteen studies were reviewed and a further five articles critically appraised to assess commonly used PtROM.

Measures reviewed included; the utility measure QWB scale, the generic measures; CHQ, Short Form-36 (SF-36) and the QOL scale and the disease-specific measures, CFQ, Cystic Fibrosis Questionnaire-Revised (CFQ-R) (for all age groups and parents), Cystic Fibrosis Quality of Life (CFQOL), Questions of Life Satisfaction-Cystic Fibrosis (FLMZ-CF) and Caregiver Quality of Life Cystic Fibrosis (CQOLCF) Scale used for assessing caregivers. The Beck Youth Inventories (BYI) and Hospital Anxiety and Depression Scale (HADS) measures looking at psychosocial aspects of living with CF were also included.

Three disease-specific models were studied in further detail; CFQ-R, CFQOL and the FLZM-CF. The CFQ-R was most user friendly and had both a child and parent version. Work has been done to develop a pictorial version. The latter two have been used on both adults and adolescents however not on children⁶⁷.

Both the methods employed in these studies and their findings are relevant to this research.

3.3.1. Summary of Studies

Table 5; Summary of 15 studies into the Use of Patient Reported Outcome Measures in Clinical Practice

Article	Objective	Setting and Sample Selection Method	Study Type, Analysis And Focus	Duration of Study	Participants Involved	Number Of Groups	Outcome
Linguistic validation of cystic fibrosis questionnaires ⁷⁵ – <i>Rozov et al</i>	To validate the Portuguese translations of 4 CF QOL questionnaires (CFQ); CFQ 6-11 years, CFQ 12-13 years, CFQ >14years and CFQ parents of children aged 6-13 years, respectively.	Multicentre sample from 3 CF centres with sample size estimation from past experience	Cross sectional study - comparative aspect checked for reproducibility	2 sessions over 15 days	150	4	The translation and cultural adaptation for Brazil resulted in 4 CFQ versions that are easy to understand and offer good reproducibility.
Differences between adolescents' and parents' reports of health-related quality of life in cystic fibrosis ⁷⁶ – <i>Britto et al</i>	To determine the magnitude and direction of differences between adolescents with CF and their parents' reports of the adolescents' HRQOL as measured by the adolescent and parent versions of the Child Health Questionnaire (CHQ)	Formal invitation prior to routine clinic visit at a CF centre	Comparative study – formed part of an extended cohort study.	1 session over 1 visit	124	2	Adolescents tended to rate their HRQOL better than their parents. They also rated themselves less susceptible to illness and worried less about their health. Optimal measurement of adolescent HRQOL will require determining both parent and adolescent perceptions of HRQOL.
Validation of a disease-specific measure of health-related quality of life for children with cystic fibrosis ⁷⁴ – <i>Modi et al</i>	To evaluate the psychometric properties of the CFQ -Child .	Recruited from 22 centres across the United States during routine clinic visits whilst waiting for consult	Cross sectional study with multi-trait analyses	1 session	164	1	Results demonstrated that the CFQ – Child is a reliable and valid measure of HRQOL for children with CF.
Associations between clinical variables and quality of life in adults with cystic fibrosis ⁵⁴ – <i>Gee et al</i>	To explore associations between clinical variables and HRQOL using the Cystic Fibrosis Quality of Life questionnaire (CFQOL)	All patients from 2 regional CF centres considered eligible	Cross sectional study – multiple regression analysis	6 months	223 adults	1	Whilst important associations were identified, much of the variance remains unexplained and merit further investigation. A longitudinal study is required to investigate further effects on QOL
Effect of disease-related pain on the health of children and adolescents with cystic fibrosis ⁷³ – <i>Palermo et al</i>	To describe the effect of recurrent pain symptoms on the HRQOL of children and adolescents with CF using the CFQ-Revised (CFQ-R)	Recruited on basis of availability from 3 university/community based paediatric CF outpatient clinics	Cross sectional study – with multivariate analysis of variance	3 months	46	2	Children with frequent CF-related pain experience broad decrements in HRQOL. Future research requires evaluation of the treatments used to reduce symptoms and improve QOL.
Can health-related quality of life predict survival in adults with cystic fibrosis? ⁷⁷ – <i>Abbott et al</i>	To evaluate whether patient-reported HRQOL could predict survival in CF using the CFQOL and SF-36 .	Approached consecutive patients from 2 adult CF clinics	Observational cohort study of – with death used as an endpoint	10 years	223	1	Aspects of patient reported QOL serve as prognostic measures of survival beyond a number of previously known factors in CF. This needs to be investigated further in a larger longitudinal study.
Health-related quality of life in adults with cystic fibrosis: the role of coping ⁷⁸ – <i>Abbott et al</i>	To examine the role of coping in explaining HRQOL in CF using CFQOL .	125 consecutive patients attending a Regional Adult CF Unit.	Cross sectional questionnaire design	2 sessions	125	1	Coping is an important factor in explaining some QOL domains but not others. This has important implications especially when employing HRQOL as an outcome measure in clinical trials.

Article	Objective	Setting and Sample Selection Method	Study Type, Analysis And Focus	Duration of Study	Participants Involved	Number Of Groups	Outcome
Psychological and family functioning and quality of life in adolescents with cystic fibrosis ⁷⁹ – <i>Szyndler et al</i>	This study examined the interrelationships between QOL, family functioning, individual psychopathology and optimism of adolescents with CF using CFQ and the Family Environment Scale .	All inpatient and outpatient adolescents approached over 8 months	Cross sectional multiple questionnaire study	1 session (time varied)	52	1	Adolescents with CF appear to be a psychologically well functioning and well-adjusted group. A more sophisticated model of well-being for adolescents with CF, which explores their views on QOL is required in a follow up study.
Living with cystic fibrosis; impact on global quality of life ⁸⁰ – <i>Wahl et al</i>	To examine the impact of living with CF from a global quality of life perspective using Norwegian version of the QOL Scale .	Control group drawn randomly; intervention group based on CF status	Cross sectional comparative design	16 months	1107	2	Results suggest the existence of a type of response shift in the CF group through changes in life standards and goals.
Development and validation of the cystic fibrosis questionnaire in the United States ⁸¹ – <i>Quittner et al</i>	This study evaluated the psychometric properties of the CFQ in a national study.	Recruited from 18 CF centres across the United States pending consent	Cross sectional comparative study – multi-trait analysis	10-14 days	212	2	Results demonstrate that the CFQ-teen/adult is a reliable and valid measure of HRQOL for individuals with CF. It may be utilised in clinical trials to assess the effects of new therapies and to document the progression of disease.
Quality of life in patients with cystic fibrosis: association with anxiety and depression ⁶⁸ – <i>Havermans et al</i>	To investigate whether CF patients with symptoms of anxiety or depression reported lower HRQOL scores using the Hospital Anxiety and Depression Scale (HADS) and the CFQ .	Recruited consecutively from an adult CF centre outpatient clinic	Cross sectional study – analyses of variance	1 session	57	1	Preliminary evidence was found on the role of anxiety and depression in different areas of QOL in CF, which may help in the development of appropriate medical and psychosocial treatment programs.
Quality of life of children with cystic fibrosis ⁶⁹ – <i>Koscik et al</i>	To review HRQOL and associated issues and to describe a study investigating CHQ scores in relationship to Newborn Screening (NBS) for CF and markers of disease severity.	Patients previously enrolled on a randomised prospective study	Cross sectional comparative study	1 session	36	2	Results did not demonstrate a benefit of CF NBS on QOL. This may be due to the lack of sensitivity of the CHQ in comparison to the disease specific CFQ, for example.
Psychosocial problems in children with cystic fibrosis ⁸² – <i>Bregnballe et al</i>	To compare the well being of children (7-14 years) with CF with the well being of healthy controls using the self-report questionnaire Beck Youth Inventories (BYI).	Invited for recruitment from a children's CF centre	Comparative study	8 months	1164	2	Children with CF did not differ from the norm when concerning depression, disruptive behaviour and self-concept. However there were significant differences in anxiety and levels of anger.
The caregiver QOL CF (CQOLCF) scale: modification and validation of an instrument to measure quality of life cystic fibrosis family caregivers ⁸³ – <i>Boling et al</i>	To modify the caregiver quality of life index – cancer scale as the CQOLCF scale, validate it with CF family caregivers and assess caregiver QOL with patient disease severity.	Recruited on the basis of CF family caregiver status from unknown location	Descriptive observational study	Time scale not detailed	100	1	The CQOLCF appears to be valid, reliable and internally consistent disease-specific scale with family caregivers. Future research recommendations include administering the CQOLCF to an increased study sample to explore item factor analysis.
Cross cultural differences in health related quality of life in adolescents with cystic fibrosis ⁸⁴ – <i>Abbott et al</i>	This study compared QOL between English and German adolescents with CF and their healthy peers using the SF-36 .	Consecutive outpatients attending 3 CF clinics in 3 locations were invited to join	Comparative cross sectional study	Time scale not detailed	208	4	Differences in quality of life between English and German adolescents with CF appear to be either culturally determined or due to idiosyncrasies in the translations of the SF-36, rather than a consequence of their disease or its management.

3.3.2. Critical Appraisal

Table 6; Study 1 –Validation of a disease-specific measure of health-related quality of life for children with cystic fibrosis⁷⁴ (Modi et al)

Criteria	Success in addressing the criteria
Screening questions	
Did the trial address a clearly focussed research question?	An objective was clearly outlined and followed throughout. A sound conclusion answering the initial research question was reached as a result of the study.
Did the authors use the right type of study?	An appropriate method was employed to analyse the results of the study. The CFQ was administered to the participants and a multi-trait analysis was applied to the results.
Detailed questions	
Was the assignment of patients randomised?	Patients were recruited from 22 specialist centres according to the inclusion criteria which included a strict outpatient status. There were no major modifiable discrepancies between participants although social statuses were diverse. Randomisation was not employed.
Were all the participants who entered the trial properly accounted for at its conclusion?	Eighty four child participants were accounted for throughout the study including the method, results and conclusion. The parent group had eighty participants.
Were participants, health workers, and study personnel 'blind' to treatment?	Research coordinators completed specialised training to standardise study protocol between sites during recruitment and screening. There was no mention regarding the input of these coordinators in neither assisting participants complete the CFQ nor the setting where they were completed. Blinding did not take place.
Were the groups similar at the start of the study?	There were two groups of participants, child patients with CF and their parents. There was no need for matching and adjustments.
Aside from the intervention were the groups treated equally?	There is little mentioned regarding the actual method employed in addressing participants.
Outcome	
Are the results of the study judging by the method employed valid?	Patients were not uniquely selected for this study. Only 168 patients were used. This being a relatively small sample results in a lower power and consequently less convergence of the results. Precaution was taken regarding the validity of the results. Research coordinators completed specialised training in order to standardise the study protocol across sites and the patients were matched for confounding factors. Gender and age differences were checked for.
Are the results of the study consistent and reliable?	Previous literature concurs with the results of the study. Effort was made to ensure that participants with results from the extremities were checked on. Multi-trait analysis was performed and paired correlation was calculated.
Can the results be applied to the local population?	The 84 participants and their parents were recruited from 22 centres across the United States and are a sound representation of the population. Patients were approached during routine visits whilst waiting for appointments, which was a realistic approach.
Were all clinically important outcomes considered?	The majority of the data yielded positive results though not all and no associations were found between pulmonary function and CFQ scores. However, the extremely ill were not included in recruitment and this cohort is most at risk of poor HRQOL. Young children also have difficulty expressing emotions to parents and on paper.
Will the results help locally?	
Are the benefits worth the harms and costs?	The purpose of this study was to recognise the reliability and validity of the CFQ-Child to aid health professionals treat their patients. This was done accurately and the model proposed no physical harm to patients in the general population. There was concern however, over the psychological impact of the questionnaire.

Table 7; Study 2 - Effect of disease-related pain on the health-related quality of life of children and adolescents with cystic fibrosis⁷³ (Palermo et al)

Criteria	Success in addressing the criteria
Screening questions	
Did the trial address a clearly focussed research question?	The objective was clearly outlined and justified with reference to the literature. The research question was answered adequately.
Did the authors use the right type of study?	46 children were subjects of a retrospective pain interview following the successful completion of the CFQ-R.
Detailed questions	
Was the assignment of patients randomised?	Participants were invited to participate via letters from 3 different paediatric outpatient CF clinics. All patients listed were invited. Letters were followed up at 2 and 4 weeks with phone calls. 36.8% of those could not be contacted again. Some chose not to participate, others couldn't schedule the time. 46 patients participated. Selection was opportunistic not randomised.
Were all the participants who entered the trial properly accounted for at its conclusion?	All participants who consented to participate in the trial were accounted for. However, 8 participants had been excluded from the start due to missing data in their files.
Were participants, health workers, and study personnel 'blind' to treatment?	There was no requirement for blinding in this study. All participants were given the same information. Questionnaires were not anonymous.
Were the groups similar at the start of the study?	The two groups were determined by age and had no influence on the results as this was not a group comparative study. Matching did not take place
Aside from the intervention were the groups treated equally?	There is no mention of how the groups were treated. The investigations were carried out on all patients and all participants were given a \$10 video rental gift.
Outcome	
Are the results of the study judging by the method employed valid?	The sample size was very small, predominantly Caucasian and drawn from only one centre; hence the results had a limited diversity and low power. Most patients experienced mild disease. patients with extreme symptoms were excluded. FEV ₁ % measurement took place up to 2 months after the questionnaire was completed; this may have skewed results. Patients were asked to recall any pain experienced 4 weeks prior of the date of the study, this may have been difficult particularly for children and introduced recall bias. The pain scale was an 8-point scale. Again these may have been difficult for the children to complete.
Are the results of the study consistent and reliable?	Previous literature results are consistent with these results.
Can the results be applied to the local population?	Pain is an important factor of CF and is an issue with most CF patients. This study showed that pain reduces HRQOL of patients. This is an important factor to consider when monitoring patients.
Were all clinically important outcomes considered?	This study used a single HRQOL measure; pain and this reduced the efficacy of the results substantially.
Will the results help locally?	
Are the benefits worth the harms and costs?	This study reports no harm and the benefits are substantial in the monitoring and treatment of patients.

Table 8; Study 3 – Quality of life in patients with cystic fibrosis: association with anxiety and depression⁶⁸ (Havermans *et al*)

Criteria	Success in addressing the criteria
Screening questions	
Did the trial address a clearly focussed research question?	This study had a succinct aim to address, one that has yet to be covered by previous literature covering HRQOL in CF patients.
Did the authors use the right type of study?	A cross-sectional study design was applied to compare participants' HRQOL scores related to their Hospital Anxiety and Depression Scale (HADS) scores, which assessed the level of anxiety and depressive symptoms experienced.
Detailed questions	
Was the assignment of patients randomised?	Patient selection was not randomised. Consecutive patients attending a CF clinics were invited to participate in the study.
Were all the participants who entered the trial properly accounted for at its conclusion?	All participants were accounted for at the start and end of the study. No participant left the study prematurely.
Were participants, health workers, and study personnel 'blind' to treatment?	This study did not require blinding to take place. The timing and order of the different measurements including lung function is not mentioned, however.
Were the groups similar at the start of the study?	There was only one principal group in this study. Only post-results were group devised.
Aside from the intervention were the groups treated equally?	It is not possible to deduce which way the results flowed; it is equally possible that the lower HRQOL came after the anxiety and depression. It is also equally possible that these results are also apparent in the non-CF population. HADS is not a diagnostic tool, it is commonly used for screening and the results must be followed up accordingly.
Outcome	
Are the results of the study judging by the method employed valid?	This study showed that the prevalence of anxiety and depression is no different in the CF population than the normal population. However, the sample size in this particular study was relatively small and patients at the far end of the disease scale were not included in the study.
Are the results of the study consistent and reliable?	Previous literature has shown that both anxiety and depression are more common in the chronically ill population and due to the limitations of this study these results may not be reliable.
Can the results be applied to the local population?	Poor pulmonary function may be associated with increased levels of anxiety and depression in patients with CF. These results may help identify these patients in clinical practice.
Were all clinically important outcomes considered?	This study focussed primarily on the prevalence of anxiety and depression in patients suffering with CF,
Will the results help locally?	
Are the benefits worth the harms and costs?	HADS is simple to use and takes little time to complete. It is viable and reliable as a screening tool. This study provides good preliminary evidence regarding the correlation between anxiety and depression and HRQOL in patients with CF, showing that screening is important in this group.

Table 9; Study 4 – Quality of life of children with cystic fibrosis⁶⁹ (Koscik et al)

Criteria	Success in addressing the criteria
Screening questions	
Did the trial address a clearly focussed research question?	This study aimed to answer a specific question. CHQ scores were compared in patients who entered the NBS programme for CF to a control group who did not.
Did the authors use the right type of study?	Patients involved in a previous Randomised Controlled Trial (RCT) involving screening in new born babies were enrolled on this randomised prospective study according to the diagnosis of CF. All patients were then treated equally.
Detailed questions	
Was the assignment of patients randomised?	Patients were borrowed from a previous randomised prospective study which was still in place. Only 2 CF centres were used. This limited variability and representation of the true population.
Were all the participants who entered the trial properly accounted for at its conclusion?	Only 36 of the 89 original patients enrolled in this study and all were accounted for throughout. The others were excluded due to age specifications.
Were participants, health workers, and study personnel 'blind' to treatment?	There was a control group but no blinding was in place. The control group was that of a previous study. For the purpose of this study all patients were treated equally.
Were the groups similar at the start of the study?	The independent variable was the study group which compared the results between the initial control and screened group from the previous trial. There were also 6 more people in the control group than in the normal group.
Aside from the intervention were the groups treated equally?	The groups had been treated differently in the previous study. One group was screened for CF at birth the other was not.
Outcome	
Are the results of the study judging by the method employed valid?	Although an interviewer recorded all results reducing recording error, patients were asked to recall information from the past 4 weeks leaving room for recall bias. All but 1 of the patients were of white origin. Due to sample size this study had low power. The results of the study were negative showing that NBS has little effect or no effect on HRQOL in later life. The validity of the results is questionable and it would be advisable for this study to be repeated.
Are the results of the study consistent and reliable?	No other studies of this nature are present in previous literature.
Can the results be applied to the local population?	NBS for CF is already in place in England. However, the importance of measuring HRQOL is outlined in the study.
Were all clinically important outcomes considered?	All clinically important outcomes were considered.
Will the results help locally?	
Are the benefits worth the harms and costs?	The study reports no harm to the patients who were not screened for CF and furthermore shows that their HRQOL remains unaffected. The questionnaire used is reported to take only 20 minutes to complete which will be of use in clinical practice.

Table 10; Study 5 – Psychosocial problems in children with cystic fibrosis³² (Bregnballe et al)

Criteria	Success in addressing the criteria
Screening questions	
Did the trial address a clearly focussed research question?	This trial addresses a clearly focussed research question looking in detail at the subject of psychosocial problems faced or experienced by patients with CF.
Did the authors use the right type of study?	A controlled cohort study compared results from a control group which were recycled from an earlier study to the results from a group of participants who had CF.
Detailed questions	
Was the assignment of patients randomised?	Over a period of 8 months all patients who met the inclusion criteria were invited to participate in this study. Socioeconomic status was accounted for.
Were all the participants who entered the trial properly accounted for at its conclusion?	1 patient declined to participate in the study. All other patients were accounted for throughout the study.
Were participants, health workers, and study personnel 'blind' to treatment?	There was no need for blinding in this study. All patients were treated the same. The same person instructed all participants on how to fill in the questionnaire.
Were the groups similar at the start of the study?	There were two groups in this study. A control group consisting of the population who did not have CF. This was of a good sample size; 1121. The intervention group consisted of only 43 participants.
Aside from the intervention were the groups treated equally?	Both groups were asked to fill in the Beck Youth Inventories (BYI). There is no mention of where and how the questionnaires were completed or over what time span.
Outcome	
Are the results of the study judging by the method employed valid?	Study sample size was small compared to the control sample. Consequently, the power was small. The age range used was 7-14 years of age. Children are not always well informed about their disease and they may have been too young to understand what was asked in the questionnaire.
Are the results of the study consistent and reliable?	The study showed psychosocially children with CF performed no differently in the BYI compared with the normal population, though some areas such as anxiety and anger differed. However, due to the differences in sample size this is not a reliable result.
Can the results be applied to the local population?	Both anxiety and depression are prevalent in the normal population and in the CF population. Previous studies have shown that both these states can affect behaviour and disease state in patients with CF. It may be important to use a screening tool such as the BYI in clinical practice.
Were all clinically important outcomes considered?	HRQOL was not taken into consideration in this study. This is an important concept which may have helped illustrate any existing correlation.
Will the results help locally?	
Are the benefits worth the harms and costs?	Although the study group did not differ from the norm it is still a valid acceptable test and important to perform in clinical practice. The authors did not comment on harms. Those patients who were screened positively benefitted.

Table 11 – Disease-specific patient reported outcome measures for health related quality of life in children with Cystic Fibrosis^{67 85-86}

Measure	Domains	Target Population	Time	Order Of Reliability		Validity
				Internal Consistency	Test-Retest	
CFQOL - 9 domains - 52 items	Physical / Social Treatment Issues Chest Symptoms Emotional Responses Concerns for Future Interpersonal Relationships Body Image Career	14 years +	Unknown	2	2	<ul style="list-style-type: none"> • Discriminates between disease severity • Sensitive to changes FEV₁ and BMI (IV antibiotics) • Correlates with SF-36 scores
CFQ-R Child - 8 domains - 35 items	Physical Emotional State Social Body Image Eating Treatment Burden Respiratory Digestion			5		<ul style="list-style-type: none"> • Discriminates between children with CF and healthy children • Discriminates between disease severity • Sensitive to changes FEV₁ and BMI (IV antibiotics) • Correlates with PedQL
CFQ-R Adolescent - 12 domains - 50 items	Physical / Social Emotional State Body Image Eating Treatment Burden Respiratory Digestion Role Vitality Health Perceptions Weight	6 years +	15 minutes	1	1	<ul style="list-style-type: none"> • Discriminates between disease severity • Sensitive to changes FEV₁ and BMI (IV antibiotics) • Correlated with FEV₁ and BMI • Correlates with SF-36
CFQ-R Parent - 11 domains - 44 items	Physical Emotional State Body Image Eating Treatment Burden Respiratory Digestion Vitality School Weight			3		<ul style="list-style-type: none"> • Discriminates between disease severity • Sensitive to changes FEV₁ and BMI (IV antibiotics) • Correlates with SF-36
FLZM-CF - 9 domains 9 items	Breathing Difficulties Coughing Abdominal Pain Digestive Trouble Eating Sleep Routine Therapy Adherence to Daily Therapy Significance for Others Understanding Free from Disadvantage	16 years +	5 minute	4	3	<ul style="list-style-type: none"> • Discriminates between disease severity • Correlates with FEV₁ and time spent on treatment • Responsive to change after inpatient rehabilitation

The table illustrates how all 3 measures are valid. On average the CFQ-R and CFQOL Scale are most reliable and comprehensive however only the CFQ-R has been validated for use with children. The FLZM-CF is brief and only worthy as a screening tool^{67 78}.

3.3.3. Analysis of Methods Employed to Measure Quality of Life

All of the studies analysed were descriptive in nature and most were cross-sectional. Cross-sectional studies are inexpensive and simple. They enable the study of many conditions at once and can be used to measure the needs of patients. This type of study is ethically safe hence ideal in paediatric patients with CF. They involve four simple procedures; observation, measurements, questioning and studying records. Studies explored a link between the variables of QOL and the change in disease progression. Unfortunately, this type of study is subject to recall bias and must have a high response rate or sample size to maintain power. It can be subject to bias and skewed outcomes. Causality is not assessed. This makes it difficult to show association. Cross-sectional studies show prevalence not incidence by collecting data from a snap shot in time. This is ideal as the results may help shape future management. Unfortunately, optimal, reliable results are formed when the samples are large and random. In most of the studies this was not the case. This could be due to a diagnosis of CF being a requirement^{68-69 75 79}.

Patients were outpatients in most studies hence the patients suffering from severe symptoms were not included. This is the group whose QOL is most affected⁶⁶. Patients who opted not to take part may have done so due to the severity of their disease^{73 87-88}. It was found that patients with greater insight into the disease lived a cautious lifestyle, sometimes in isolation in a bid to prevent infections and complications. Their expectations may have been lower hence subsequent QOL was subjectively higher⁷⁷.

Many factors correlate with changing QOL in patients with CF; disease state, hospital admissions, treatment, concurrent and past medical history, socio-demographic factors, gender, culture, Body Mass Index (BMI), schooling environment, family dynamics and psychopathology^{43 65-66 68 80 84 89}. No single study at all aspects.

The methodology of most studies consisted of assessing PtROM however little weight was put on where and when measures were taken. In some studies the study participation was pre-planned whilst others were approached in the waiting room prior to their appointment. Some participants may have prepared for the study⁷³⁻⁷⁴. The study setting was also subject to change. Some studies took up to 20 minutes to complete the PtROM other studies gave

participants a pack of questionnaires to take away. The order of questioning also varied. Where some patients were given lung function results others weren't⁷³. Such discrepancies may have skewed results dramatically and may have introduced substantial recall bias both between and within studies^{54 66 76}. Patients may have also withheld information due to embarrassment⁷⁹. HRQOL measuring tools are highly subjective and can be sensitive to small changes. Results can be unreliable depending on personal judgement and coping ability⁶⁶.

Where parents were included in studies it was found that they tended to rate QOL up to 12 points lower on average than their children^{76 90}. This may reflect on their personal QOL and the Burden of Care (BOC) they experience⁹⁰. As a primary caregiver the parent's QOL impacts on the management of the child's disease at home. There is a need for the assessment of primary caregivers together with the assessment of family functioning as a whole.

3.3.4. Analysis of commonly used Patient Reported Outcome Measures

CF is a complex and difficult to treat paediatric condition and an important health issue. The multisystem nature of CF requires regular MDT involvement, routine appointments and hospital admissions⁸⁹. Patients' perceptions of health are skewed compared to the normal population. This is referred to as a response shift. This results in an altered threshold for changes in HRQOL⁸⁰. It would explain how all studies involving parents deduced that the patients rated their QOL higher than their parents did⁷⁶. Individual coping mechanisms differ considerably between patients and caregivers and this is reflected in the PtROM results^{68 78}. *Gee et al* and *Quittner et al* showed that gender and cultural differences also exist with regards to coping and the subjective interpretation of the illness and its global effects^{65 84 87 91}. Females experience lower levels of anger than males and higher levels of anxiety. Male subfertility in adulthood worries many male patients too^{70 75}. Another example is the lower BMI which is often seen in patients; where females are happier with being lean the males interpret the symptom as embarrassing and take measures to avoid public display^{54 81}. Although emphasis must be put on educating patients⁶⁶ factors such as the expected shortened life span, male infertility and reduced QOL may cause undue stress⁸⁰. Certain risk factors; low birth-weight, poor pulmonary function, airway pathogens, anxiety and depression put patients at greater risk of a poor QOL^{72 77 86}. Measuring HRQOL in these patients may not be as valuable as their baseline HRQOL may be considerably lower^{73 78}.

Presently, the CFQ-R is the most widely used HRQOL measure in CF⁷². Recognised internationally and translated into 25 languages it has drastically changed CF care and

research⁹². Both the CFQ-Child and CFQ-Parent should be regularly incorporated into the annual assessment to maintain benefits⁷². It has a potential to increase responsiveness^{70 75} and could be computerised and used to formulate patient profiles⁹². These could be monitored closely by both the family and the MDT. With the addition of screening devices such as HADS and BYI a global image of HRQOL can be attained. Both the American Thoracic Society and European Respiratory Society have recommended that screening be in place and treatment given⁸⁶.

Screening tools are inexpensive and simple to administer. It is beneficial to incorporate screening into the annual assessment to check for easily missed disorders. It is acceptable that due to naïve reading levels and understanding and poor recall and attention in children such measures are difficult. Some of the symptoms of anxiety and depression like fatigue are commonly seen in CF and care must be taken to distinguish these. BYI enquires about sensitive issues such as suicide. It is however difficult to comprehend and is not ideal in children. As with all screening measures the subsequent outcome both positive and negative can have affects on all parties involved. A true positive result, whilst alerting the CF team, could also lead to further medicalisation of their life; a change that may not be welcomed by the individual. A false positive would lead to further unnecessary screening and possibly treatment. It could also disturb the relationship between the patient, the caregiver and the CF team.

It may be argued that the HADS and BYI measures are not significant as they do not measure QOL directly. The psychosocial aspects taken into consideration have been shown to have adverse effects on compliance, treatment and mood^{62 84}. Patients in this group tend to engage in smoking and drinking and consequently have a greater number of hospital admissions⁸⁶. Anxiety and depression reduce emotional arousal and can also lead to eating disturbances and low self esteem^{68 86}. Larger studies have shown that depression rates are as high as 50% in medical populations compared to 17.2% in the healthy population⁸⁶. It is widely accepted that anxiety and depression are also more prevalent in patients with chronic disease. Professional input from a psychologist or psychiatrist is vital in the screening of such disorders as compliance may diminish resulting in a reduced QOL^{68,86 93}.

The Hunter Opinions and Personal Expectations Scale (HOPES) showed that the majority of patients were hopeful with regards to their management of CF and their future⁷⁹. CF patients are psychologically well adjusted and live normal, happy successful lives. It is not the fatal childhood disease that it used to be. The main aim now is to monitor and optimise QOL.

An important issue raised is that of whom HRQOL measures should be aimed towards. Ultimately the QOL of the patient is empirical to the management however in the case of

paediatric CF where disease management is heavily weighted on the caregiver it may be of benefit to involve them too. The use of HRQOL measures on parents and caregivers is vital as they usually share the burden and are at times more anxious than the children due to their higher level of understanding^{76 90}. *Bregnballe et al* showed that parents worried about BMI more than children as children lacked understanding and in the case of adolescent female a lower BMI was often preferred⁸².

CF is a condition which affects the family as a whole influencing family dynamics and reshaping important decisions. CF influences the home environment and general mood. The state of the disease influences compliance with daily, complex, time consuming treatment⁹³. It is noted that there is a substantial maternal demand sometimes interpreted as burden by the family and caregiver. This can cause negative feelings and anger from all parties concerned. Increasing support is being offered to caregivers as the role can result in a lack of rest and sleep, fatigue, health problems, anxiety, fear, depression and financial difficulties⁸³. Recent studies have shown that depression is common in parents and caregivers, particularly following diagnosis⁸⁶. *Boling et al* found that the CQOLCF is a valid and reliable measure taking all aspects of the caregiver's well-being into account⁸³.

The generation of a flawless PtROM with the ability to take into consideration and be sensitive to every aspect of CF is a sophisticated science and will take time to be perfected. Measurement of HRQOL in CF has a lot to offer in future advances both to research and care⁷¹.

3.4. The Challenges of Living with Cystic Fibrosis Questionnaire – A Parent Reported Outcome Measure

The CLCF-Q is a 10 part, 62 item PROM measuring the burden of care on a parent/caregiver. It relies entirely on Parent Reported Outcomes (PRO) to equate the burden of living with the everyday challenges faced by the caregiver of a child with CF.

The development of the CLCF-Q began in 2005. It stemmed from the gap in literature identified by the Mental Health Unit and the Institute of Child Health at Alder Hey Children's NHS Foundation Trust; a need for a research tool focussing on the caregiver⁹⁴. *Glasscoe et al* aimed to,

“Capture the experience of bringing up a child with cystic fibrosis³.”

Much time, attention and revenue is focussed on measuring the QOL of the child and its changes in relation to health; HRQOL. Little attention has been given to the focal entity; the

caregiver of the child in question. In most areas of medicine and in particular paediatrics, the caregiver plays an essential role in the disease outcome having determined the treatment and management received by the child at home. Thus it is imperative that their health is also considered. The burden of caring for a child with a chronic disease and overcoming the challenges of living with CF can have detrimental effects on the caregiver⁶⁴. The QOL of the caregiver and the effect of the BOC on their QOL should be considered. No measure of caregiver challenge currently exists.

Using the avenues of selective consultation through in depth cognitive interviews, focus groups and active research involving parents and caregivers (n = 10) and the CF MDT the basic operative themes were constructed; demographic characteristics, family context, child's character, CF history, challenge to family life, CF routines, local medical service and supplies, hospital based care and pharmacy with careful consideration of time and effort exercised⁹⁴.

The present study forms part of a collection of studies looking into the development and validity of the CLCF-Q. The CLCF-Q was devised to take between 15 – 30 minutes to complete requiring minimal effort and employing the use of Likert scales. Pilot data (n = 39) supported face validity, acceptability and reliability establishing the CLCF-Q as a useful tool to employ⁹⁴.

The CLCF-Q aims to explore the themes of time and complexity of tasks and challenges estimating the demand on caregivers. It can be used to explore the effects of BOC on adherence and compliance. Most importantly it can help correlate changes in the BOC and consequently, QOL. Both the mental and physical health of the caregiver must be considered. Stress, depression and anxiety are all physiological states strongly associated with the BOC.

The CLCF-Q was recently used in the Home Intravenous Antibiotic Therapy (HIVAT) study as an outcome measure in a study estimating risk⁹⁵. *Dyer et al* also showed that the CLCF-Q was sensitive to changes in disease. Pulmonary exacerbations resulted in increased time demands and resultant BOC⁹⁶. This supported the face validity of the CLCF-Q in measuring BOC. Both studies examined the change in score of the CLCF-Q over two time point's relative to disease state.

The CLCF-Q is a multidimensional tool suitable as a research outcome in clinical trials, as a clinical tool in practice and perhaps most importantly as an indirect psychological intervention for caregivers by aiding feedback⁹⁷⁻⁹⁸. In a previous study caregivers expressed the value of the CLCF-Q as an essential piece in the annual assessment process⁹⁴. They

recognised its potential to detect changes in their child's condition and equate their coping by privileging their personal views. It helped to reflect on their experiences and recorded their coping abilities of complex challenges. The recollection also helped identify areas of concern. Diverting the focus from the child's HRQOL to the caregiver's challenges. In theory, allowing us a deeper understanding of caregiver challenge.

“It offers a framework for caregivers to raise any difficulties and has potential as a clinical tool at annual review as well as a research tool in clinical trials.³”

It has a strong potential to reveal where challenges lie allowing the identification of negatively impacting factors⁹⁹.

An issue arising from previous studies is the need for meanings to be associated with interventions. The basis of this present study were built on the concept of using the CLCF-Q to identify otherwise unrecognised issues encountered by caregivers when facing challenges. Through the process of careful feedback following the completion of the CLCF-Q a deeper understanding of the challenge would be gained by both parties.

Self Efficacy

4.1. The Concept of Self Efficacy

It is human nature to strive for control in any situation. Through control we are able to guarantee the desirable goals in life and avoid disappointments. It is reasonable to assume that our belief in our ability to attain an achievement determines our choice of activities and our general demeanour towards the situation. This includes effort expended, our sustainability, our resilience, our perseverance and essentially the level of anxiety experienced.

In 1977 Albert Bandura published his works on, 'Self Efficacy,' coining the phrase and paving the way for a new field of interest¹⁰⁰⁻¹⁰¹.

“Perceived self efficacy refers to people’s belief in their capabilities to organise and execute the courses of action required to deal with prospective situations¹⁰².”

- Bandura, 1977

Human behaviour develops through careful modelling techniques^{101 103}. Initially one may observe and follow others or instructions given by others. Later one will learn from others or one’s own mistakes allowing for self corrective adjustments until the technique is refined to a suitable standard. One’s Perceived Self Efficacy (PSE) dictates ones will to face challenges. This is due to the dependence on coping beliefs¹⁰⁴⁻¹⁰⁵. Without PSE or with a low PSE the task is seen as too great a challenge and may be avoided completely or performed with no intent to succeed; hence poorly¹⁰⁶.

PSE is a standalone concept and must not be confused with the other ideologies of global self esteem, locus of control and self confidence¹⁰⁷⁻¹⁰⁸. Expectations of personal SE ultimately determine whether or not an action is initiated¹⁰⁹. It holds significant promise and may help to explain the variability in a person’s ability to cope with the demands and challenges which arise from caring for a chronically ill patient.

Expectation is devised from two angles; efficacy expectation and outcome expectation. Whilst efficacy expectation refers to personal SE and belief in one’s ability to complete a task, outcome expectation refers is, “A person’s estimate that a given behaviour will lead to a certain outcome¹⁰¹.” The difference between efficacy expectation and outcome expectation is that the subject may believe in the outcome but they may doubt their personal ability. Expectations affect both initiation and persistence of coping behaviour and in order to maintain efficacy they must succeed, performing well consistently.

Psychological techniques can help create and strengthen SE. It is hypothesised that those who persistently face challenging situations will eventually overcome their inhibitions through corrective experience; learning from their mistakes¹⁰⁰. Thus overcoming barriers and facing challenging situations must be encouraged in order to promote coping behaviour¹¹⁰⁻¹¹¹. Unfortunately, it is also possible to adapt a defensive behavioural pattern which can be debilitating¹¹².

To date the SE model has been used in many areas of treatment and research. The initial focus of SE research was on phobias, chronic stress and coping. In health, it has now moved on to experiences of family caregivers of dementia patients and more recently chronically ill paediatric patients¹¹³⁻¹¹⁵. Whilst research has considered SE studies with CF patients the concept of measuring and influencing SE in caregivers of children with CF has not been looked into¹¹⁶.

4.2. Influencing Self Efficacy

‘An odd aspect of the perceptual control theory is the claim that, “People act to control the perceptions, and not actions”^{104 117}.’

- Vancouver et al

PSE is shaped through four main routes; performance accomplishments, physiological states, vicarious experience and verbal persuasion^{101-102 104}.

4.2.1. Performance Accomplishments

When a task is performed successfully the resulting sense of accomplishment and personal mastery increases the individual’s SE. The methods employed to confront the challenge are retained as information. This learned behaviour enables the individual to repeat the task with ease. Having completed the task previously the task faced no longer seems a challenge^{101 104}. Through this repetitive notion a greater number of challenges are faced successfully lessening the perception of burden.

4.2.2. Vicarious Experience

Vicarious experience refers to an individual modelling the actions of another on their own¹⁰³. Seeing another overcome a similar task successfully reinforces one’s belief in their personal ability to face that challenge. Furthermore when facing the task they mirror the actions

witnessed. Unfortunately, previous studies have shown that vicarious experience alone is not effective in noticeably improving SE^{100 107}.

4.2.3. Verbal Persuasion

The ease of use and ready availability of ideal situations promotes the use of verbal persuasion as an ideal source of information. Using appraisals and feedback individuals may be persuaded and encouraged to face overwhelming situations⁹⁸. Studies have shown that social persuasion in the appropriate environment and corrective performance are more likely to work than performance alone^{100 107}.

4.2.4. Physiological States

We rely heavily on emotional arousal when determining the appropriate reaction to a situation¹¹⁸. Situations causing a heightened sense of emotional arousal are deemed stressful and result in anxiety¹¹⁹. Individuals often surrender as a result of the associated anxiety¹¹⁴. As previously described for performance accomplishments this sensation is reinforced further when the task and associated anxiety are faced again^{100 107}.

4.3. Influencing Self Efficacy – In Practice

In order to ensure the maximal increase in SE all four sources of impact should be utilised, but this isn't always possible. The optimum method for maximising SE in any given situation would be to eliminate all sense of anxiety completely^{100 104 107 120}. But this isn't usually possible either.

SE regulates human behaviour via motivational, cognitive, affective and decisional processes^{109 121}. It can be used to influence actions both positively and negatively. Evidence from meta analyses have consistently illustrated that SE contributes significantly to motivation and performance^{104 109}. There is also evidence showing that negative persuasion manifests stronger than positive persuasion hence more effort is required to positively influence a situation than negatively¹⁰⁴. It is important to consider how each performance is unique and independent and whilst simultaneous situations may affect each other no situation can be imitated wholly. Furthermore the more difficult a challenge the greater the number of positive persuasions required to enforce positive SE and the greater the influences are taken into consideration; both positively and negatively¹⁰⁷.

Stimuli do not change in condition and cannot be influenced in any way. It is one's knowledge of the situation and the perceived environment of the challenge that can be influenced. Our reaction to the stimulus dictates this^{100-101 104}.

This study couples the concepts of performance accomplishments with verbal persuasion by exploring the use of a PROM in a clinical setting; namely the CF annual assessment. It studies the use of the tool the CLCF-Q to increase how able the primary caregiver feels in managing the everyday challenges they face as a consequence of caring for a child with CF. Unlike previous research in similar areas, this work does not aim to eradicate defensive behaviour. Instead it focuses on corrective behaviour and empowerment.

4.4. Assessing Self Efficacy

Assessing SE can be as complicated as influencing SE. The German version of the General Self Efficacy-Scale (GSE-S) was developed in 1979 by Matthias Jerusalem and Professor Ralf Schwarzer¹²². They discussed how PSE reflected on optimistic self belief and was required to facilitate goal setting and persistence in everyday activities¹²³. The scale was originally developed to help equate general PSE and allowed the assessor to predict the individual's ability to cope with daily challenges and recover following stressful life experiences¹²³. It has been translated in 26 languages and the measure has been used successfully for 3 decades. Originally designed for children and adults over the age of 12 it consists of 10 items graded on a 4-point Likert scale and yields a score between 10 and 40. The recommended completion time is up to 4 minutes. The GSE-S has previously been tested for reliability with Cronbach's alpha values of 0.75 – 0.91 (median = 0.80)^{122 124}. The GSE-S can be amended to include disease specific items. This study assesses the SE of caregivers in the field of CF. Four additional non-validated items specific to caring for a child with CF will be added to the GSE-S to cater for this.

4.5. The Role of Self Efficacy in Stressful Situations

When asked about the role of the caregiver the word most often used to describe their situation is, 'stressful.' But it is not the situation itself that causes discomfort, it is the concept of losing control of the situation^{104 107 125}. Correlation exists between low SE and increased anxiety. This poor belief in your ability to succeed in the situation is the main stressor. Stressful situations elicit emotional arousal debilitating performance further^{120 126}.

Experiments have shown that levels of stress are lowest when faced with an equally challenging situation to one which has been completed before in comparison to unfamiliar situations¹⁰¹. This is directly related to the higher level of SE maintained as a result of having completed the task before; previously described as performance accomplishment. When exposed to unfamiliar challenging situations which are perceived as difficult to tackle the resultant PSE is low. PSE mediates anxious behaviour. Improving an individual's PSE reduces the level of stress they experience¹⁰⁴.

Ideally, focussing on reducing emotional arousal and anxiety would allow caregivers to manage their physiological arousal and take control of situations but this is not possible within the constraints of outpatient hospital care¹²⁵.

There are two prominent theories which consider the concept of SE as pivotal in human response to stressors. The social learning theory recently referred to as the social cognitive theory, considers anxiety and defence mechanisms as coexistent factors which aren't causally linked. It takes the relationship between physiological arousal and performance as being inversely proportional. SE is seen as the focus with an increased SE being the cause of lower physiological arousal. The dual process theory states that eliminating anxiety will by definition eradicate defensive behaviour as they are directly linked¹²⁶⁻¹²⁷.

Early research into SE centred largely on reducing the affect of and eliminating phobias. The role of systemic desensitisation was explored and yielded promising results¹¹². To eliminate defensive behaviour we must ultimately reduce or eliminate anxiety¹²⁶. Systemic desensitisation to common stressors would be ideal but due to the constraints of the outpatient clinic this is not possible.

The social cognitive theory defines human self development as a product of constant adaptation to change¹⁰¹. Using this theory the possibility of influencing and improving an individual's reaction to a challenge is feasible.

Two main principles construct human behaviour; cognitive processes and performance-based procedures. Through modelling, we observe others, succeeding in our efforts and consequently forming new behaviour patterns. This is termed observational learning and leads to learning appropriate behaviour patterns through personal experience^{103 118}. Consequences of actions, inform performers which behaviours are appropriate and which aren't; which behaviours yield positive results and which lead to disappointment. This reasoned information is processed serving as an experience of mastery. Experiences of mastery improved PSE towards a specific challenge whilst also improving global SE^{105 120}.

This study looks at the possibility of assessing and influencing SE. The aim is to empower the caregiver and reduce the amount of stress experienced. The ideal result would improve the overall QOL of the caregiver and the family unit.

We aim to incorporate a recently developed and validated burden of care measure called the CLCF-Q into the annual assessment process. It details items concerning the caregiver's role which aren't regularly visited during clinic appointments. Issues will be identified from the CLCF-Q and simple feedback offered at a later appointment. Allocated time with specific members of the CF care team, to discuss any previously unidentified issues will follow. The identification and solution of challenges or problems using the theories of performance accomplishment and verbal persuasion may increase SE whilst decreasing anxiety and reducing defensive behaviour¹²⁶.

Positive behavioural change is proportional to change in PSE¹²⁷⁻¹²⁸. Behaviour is based on a background of thought and in order for SE to be affected subjects must recognise paired stimulation; cause and effect. They must understand that the outcome was the direct result of the action¹²⁹.

Through careful specific feedback and verbal persuasion the meaning of challenges and situations can be reevaluated significantly^{98 104}. It is worth noting that feedback information is synthesized and processed over relatively long periods of time so the effects are not normally expected to manifest instantly. The likely change in SE is gradual. This is similar to avoidance behaviour which persists for long periods after the situation and stressors have passed¹⁰⁶.

4.6. The Role of Self Efficacy in Promoting Quality of Life

PSE facilitates goal-setting, effort investment, persistence, and recovery. It is an operative construct relating to subsequent human behaviour and notably relevant to clinical practice¹⁰⁷. Human behaviour has equitable control over QOL. And SE dictates behaviour hence PSE has an indirect influence on QOL¹⁰⁵. To promote the QOL of the caregiver, SE must be monitored, reviewed, adjusted and maintained.

Exploring the SE levels of caregivers of children with CF would detail the scope and differences in SE between different caregivers and family units. It would allow the identification of cases with particularly poor levels of SE. It can also be used as an indicator of QOL at any locus. As a result it may be possible to influence and improve the SE of these caregivers.

Generally, positive habits and perseverance must be encouraged as accomplishments lead to satisfaction. Through repeated success the individual gains a sense of control. A global sense of personal control and mastery reduces depression and increases coping ability^{125 130}.

PSE also fosters the development of supportive relationships^{126 131}. It is this sense of a supportive environment which is imperative to a good QOL. Incorporating an intervention into routine care which aims to identify areas which the individual recognises as a challenge and following this with careful constructive feedback and support, will allow the healthcare team to influence SE, perceived control and inevitably QOL. Such an intervention would also improve perceptions of availability of social support¹²⁰.

4.7. The Role of Self Efficacy in Caregivers of Children with Cystic Fibrosis

CF is a unique disease specific to each individual. Similarly, the SE and coping ability of the caregiver are also unique to that individual¹¹¹. Different children with CF require differing levels of care depending on their disease state. This has an impact on the burden of care and the QOL of the primary caregiver.

The role of the caregiver is emotionally, mentally and physically challenging¹¹⁴. Caregivers often express difficulties confronting and regulating their worries about the future. Whilst the use of cognitive and behavioural therapy is usually aimed at reducing anxiety it may also be helpful in developing coping strategies¹²⁶. Coping efficacy reduces anxiety and depression¹³⁰.

Originally much of the research carried out on the concept of SE worked around the treatment of phobias. Recently this has moved to the role of caregivers caring for children with chronic disease, mainly cancer¹³¹⁻¹³⁶. Whilst SE contributes significantly to coping and anxiety levels it must not be confused with the concept of developing coping strategies and reducing anxiety. SE has a distinctive and significant role to play in health, particularly paediatric health. The care of a paediatric patient has more dimensions than the care of an adult. Caregivers of the paediatric patients play a pivotally influential role in the outcome of the disease. In the chronic, intensely demanding disease CF caregivers are expected to tackle a wide range of treatments and make decisions on a day to day basis. CF is now moving rapidly towards home based care. This dramatic shift has vast implications on the role of the caregiver and the family unit.

In the normal environment it is understandable that challenging situations are avoided¹⁰⁶. However, as the primary caregiver of a chronically ill child difficult situations must be faced on a daily basis and cannot be avoided. Persistently facing challenges reinforces a higher

level of SE¹⁰⁷. A greater level of PSE in any given situation is often associated with mastery and success¹⁰¹. By positively influencing SE the aim is to empower caregivers; though caregivers must believe that the outcome of any given action will be equally positive each time. Over time performance in similar situations is improved and a generalised increase in SE is noted. Enhanced SE can become generalised over time improving reactions to all situations faced. Contrarily, repeated failures within a short span of time and early on in the learning process result in dramatically lower general SE levels resulting in long lasting negative cognitions.

This study uses a PROM and feedback as tools to improve the SE of an individual. For this to be successful one must understand that feedback involves more than simply informing a patient of the findings. It is more than informing them that a treatment will work and a certain situation can be overcome. Carefully constructed and successful feedback is a two way process. It involves simultaneous interaction between the practitioner and the caregiver. Any interventions and changes to management must be illustrated and the path must be explained. The caregiver must believe that their views have shaped the discussion and are valued. Feedback should not only inform the caregiver it must also involve them. It is proposed that in the specific situation of caregivers of children with CF feedback must be followed by specifically allocated time with the healthcare team. This is to allow any concerns to be addressed^{104 107}.

This study proposes that handing over a proportion of control to the caregiver will improve their SE¹²⁵. More importantly helping caregivers understand how their control is exercised and giving them a direct avenue through which to seek help would further alleviate stress and anxiety. This avenue would be the inclusion of the CLCF-Q in the annual assessment process.

Psychological changes can be achieved through many different methods and modes of treatment. They all have the potential to alter SE¹⁰⁰. The impact of the information relayed to caregivers during this study will depend mostly on how it is cognitively appraised by the caregivers. Any given person may portray differing efficacy expectations when facing different challenges. There is no method of controlling this aspect and it is difficult to continuously monitor levels of SE. Likewise it is correct to expect intensity of action and persistence to vary with the strength of PSE. As different individuals have encountered differing levels of efficacy-influencing situations, any new source of information will impact subjects uniquely¹⁰⁰. For example, there are many levels of visual impairment. Not all are treated with the same pair of spectacles. However visually correcting spectacles will have *some* effect on *all* individuals. In a similar sense there are many levels of SE and whilst all

individuals cannot be treated by the same intervention, it will influence *all* individuals to *some* degree¹⁰⁰. The CLCF-Q and the feedback will work in the same way that training wheels do when a child is learning to ride a bike. It offers guidance and help teaching the caregiver how to face and overcome challenges until they no longer require the help. The healthcare team should serve as motivators and guides and the CLCF-Q allows for this¹⁰⁹. Using the CLCF-Q we should aim to reinforce the core belief that the caregiver has the power and control to produce the desired effects in any given situation. Caregivers need to receive honest feedback concerning their performance¹⁰⁴.

'No psychological factor or any other factor for that matter ever bears an invariant relation to human behaviour'¹⁰⁴.

Natural behaviour is adaptive and anticipates control and motivation. It is guided by goals and encouraged by the goal seeking process¹⁰⁹. Humans are proactive, aspiring towards targets and not just reactive. As actions are rewarded this behaviour promotes re-action and further attempts^{100 137}. Thus caregivers not only plan and fore think their actions; they self-regulate them. They adopt personal standards and monitor and regulate their actions to challenges through self reactive influence. However, self regulation of learning through performance and error correction is a prolonged drawn out process¹²¹.

As the demand on a primary caregiver increases the SE tends to automatically decrease. They tend to move their focus towards negative aspects of a challenge and personal deficiencies. Ideally, as the state of the patient's disease declines the persistence of the primary caregiver should increase and a favourable increase in SE would be welcomed. Beliefs regarding competency have varied but strong effects on the caregiver.

The two components of motivation are activation and persistence. Motivation requires a cognitive source of information consisting of goal setting and self evaluation. This is what the CLCF-Q aims to do^{107 109}. Self prescribed goals are more effective than goals set by others¹⁰⁹. To establish consistent optimal performance one must possess the necessary skills required and a high level of SE. The intervention we propose presents opportunities to develop both these skills and practice them.

Lastly, SE is an important construct in improving our understanding of normal reactions to challenging stressors. It is also worth noting that environmental factors are processed and transformed by caregivers and their worth depends on how the information is cognitively appraised hence they are other factors which need to be considered¹³¹.

Chapter Three

Does the introduction of a parent reported outcome measure influence the self efficacy of caregivers of children with cystic fibrosis?

Introduction

5.1. Introduction

CF is the most prevalent, autosomally recessive inherited, life shortening disorder in the Caucasian population^{40 75}. It is estimated that there are over 10 million carriers of the gene worldwide and 1 in 2500 live births are affected in the normal population^{54 74}.

As a result of recent advances in medical care the median age of survival has risen from 2 years of age in 1938 to over 50 years of age⁷⁴. However, the daily life prolonging treatment involved is expensive, intensive and time consuming. This can be a great burden on the caregiver.

Paediatric medicine aims to improve the patient and their caregiver's global QOL where possible alongside stopping or slowing down disease progression. Measuring the impact of the chronic illness on these patients is becoming increasingly important. Three decades ago the WHO released a definition for health,

“a state of complete physical and social well being, not just the absence of disease⁶⁵.”

Consequently, time and resources have been allocated to ensure a good standard of life is achieved and over the past 2 decades numerous tools have been proposed to measure QOL. Measuring QOL is a method of subjectively evaluating the impact of chronic disease on a patient's ADL and global well being. QOL is, *“a dynamic psychological construct, which describes the subjective health perceptions of the patients independently of objective health parameters⁶⁶”* and it fluctuates over time and disease progression.

Over the past 25 years PtROM have become increasingly important in influencing treatment. A PtROM is,

“A measure of a patient's health status, elicited directly from the patient, that assesses how the patient feels and functions with respect to his or her health condition⁶⁷.”

Measures range from an item rating a single symptom to a multi-system analyses looking into the global HRQOL of the patient. HRQOL measurement in CF allows the inclusion of a patient's perspective. It is a multidimensional construct comprised of the domains of physical functioning, social functioning and emotional and psychological functioning. Together they give an overall picture of the disease state incorporating otherwise untested for states such as anxiety and depression⁶⁸. Although previous studies have almost perfected the use of PtROM and HRQOL measurements, the exact use of this information and direct impact on the patient and their caregiver has had relatively fewer investments. The affect of

these interventions on the patient's further treatment or QOL remains unknown. Studies have identified a need to measure the caregivers QOL. Caregiver QOL directly influences the patient's disease management^{83 138}.

Working with parents a PROM called the CLCF-Q was developed. Its aim was to measure the burden of care on everyday living for caregivers of patients with CF. This study will explore the use of this measure in a clinical setting; the CF annual assessment process. The study will look at how this tool could be used to increase the caregiver's SE in relation to facing the challenges of living with CF. PSE is,

"the expectation of personal success in a given situation"¹⁰².

Bandura introduced his works on, 'Self Efficacy,' in 1977 paving the way for a new field of interest¹⁰⁰⁻¹⁰¹. The concept of measuring SE first came to light in 1979. Matthias Jerusalem and later Professor Ralf Schwarzer discussed how PSE reflected on optimistic self belief and is required to facilitate goal setting and persistence in everyday activities¹²². Human behaviour is generally developed through careful modelling techniques. Initially one may observe and follow others or follow instructions¹⁰³. Later one will learn from others or one's own mistakes allowing for self corrective adjustments until the technique is refined to a suitable standard¹⁰⁰. Without PSE or with a low PSE, the task is seen as too great a challenge and can sometimes be avoided or performed with no intent to succeed¹⁰². Four main sources of information alter PSE. These are; performance accomplishments, physiological states, vicarious experience and verbal persuasion¹⁰¹. Introducing the CLCF-Q with a feedback system, may influence the SE of caregivers by utilising the concepts of performance accomplishments and verbal persuasion.

Studies have shown that an increase in PSE could directly correlate with and improve performance and success rate¹⁰³. A person's PSE dictates their behaviour, choice of activities, effort expenditure, perseverance, coping efforts and will to face challenges¹⁰⁰. Caregivers may be more likely to take on challenging tasks with a belief of succeeding if they have a higher level of SE. They are also more likely to confidently make decisions about the treatment their child receives. This possible increase in SE may also correlate with their perception of control, improving the patient and their caregivers global QOL overall.

5.2. Objectives

5.2.1. Aims

The aim of this pilot study was to explore the feasibility and benefits of using the CLCF-Q measure in a clinical setting. The impact of using the CLCF-Q on the SE of caregivers was assessed. Semi-structured interviews were undertaken with a caregiver to inform the results qualitatively. Data from this study will be used in power and sample size calculations for a larger multicentre study.

5.2.2. Hypothesis

It is hypothesised that incorporating the CLCF-Q into the annual assessment process and offering subsequent feedback will have a positive influence on the SE of caregivers.

Methodology

5.3. Methodology

5.3.1. Administrative Organisation

This single centre study was carried out at the CF outpatients unit at Alder Hey Children's Hospital. All participants were approached, recruited and seen at the outpatient department. The study was affiliated with the Institute of Child Health, University of Liverpool. It was co-sponsored and funded by both parties. The study was also funded by the Alder Hey CF Trust Fund.

5.3.2. Ethical Approval

Ethical approval was granted in full from both the NHS Research and Development Committee and the NHS Research Ethics Committee. The study was also approved by the University of Liverpool Research Governance Officer. This study is part of a series of studies conducted to contribute to the validation of the CLCF-Q as a clinical tool.

5.3.3. Study population

Participants consisted of both genders with no restriction on age and background. They were selected on the basis of the inclusion criteria. In each caregiver/child dyad the primary caregiver was invited to participate in the study. It is not assumed that this would be the mother in the family; parents were asked to decide who is most suitable themselves.

5.3.3.1. Selection Procedure

The recruitment was headed by the research team. Prospective participants were determined using data available on their child's information sheet in the hospital database. They were matched according to the inclusion and exclusion criterion below to determine their suitability.

5.3.3.1.1. Principal Inclusion Criteria

The principal inclusion criterion was;

- 1 The caregiver must have a child diagnosed with CF.

- 2 The diagnosis of CF must have been made at least one year ago.
- 3 The child must be registered as a patient at Alder Hey Children's Hospital.
- 4 The child must be between 1 and 13 years of age as the CLCF-Q is only validated for use with children between this age range.
- 5 The caregiver must have a reasonable understanding of the study, understand what is involved of them and be competent enough to refuse participation.

5.3.3.1.2. Principal Exclusion Criteria

The principal exclusion criterion was;

- 1 Caregivers or children with physical or mental disabilities which prohibit them from participating in the study successfully.

5.3.3.2. Recruitment Procedure

Following verbal consent, prospective participants were approached by the researcher during an outpatient clinic appointment and invited to partake in the study. They were informed about the study and provided with an invitation letter and participant information sheet to take home and study at their own convenience. Participants were given the option and time to approach the research team or an independent party regarding any queries. There was no public advertisement of the study.

At the annual assessment appointment, caregivers were reminded of the study and given another opportunity to read the participant information sheet, as well as make any concerns known. Following voluntary, informed, written consent participants were recruited onto the study.

5.3.3.3. Randomisation procedures

On a randomised basis participants were allocated to 2 groups; a control and an intervention group. Participants were randomised in groups of 10 as recruitment rate was unclear. The participants were randomised immediately following consent and the Sealed Envelope[®] process was employed. To maintain objectivity they were allocated by an independent party.

5.3.4. Experimental Design

A randomised controlled non-blinded intervention pilot study supported by qualitative data was carried out between April 2010 and August 2010 at a single centre. All participants were asked to complete a selection of questionnaires at 2 time points during their child's annual assessment process. The outcome measure in the study was determined using the Cystic Fibrosis Self Efficacy-Questionnaire (CFSE-Q).

5.3.4.1. Narrative Interviews

A diagnosis of CF affects all areas of family dynamics⁶⁵. Whilst the value of science on the prognosis of a child's health and QOL cannot be undermined the value of congruent psychological input must be considered. Direct feedback from the child and their caregiver regarding a treatment is invaluable. Whilst the value of quantitative methods and results in research are clear the concepts put forward by qualitative data must not be undervalued. This study centres on two major concepts; improving SE using PROM and the effect of SE on QOL. Qualitative data are essential in these cases. In practice a change in SE can be mapped and identified statistically however if the participant does not feel that this change is a positive one then compliance is unlikely. Furthermore, if the intervention is disconcerting compliance is affected further. Thus, qualitative data are invaluable in our study. The mixed methodological approach is a real strength in this study and the quantitative and qualitative components support each other.

The aim of the qualitative component of this project was to add support to the quantitative data as well as forming standalone conclusions. The three forms of qualitative methods considered are; observations, interviews and focus groups. To extract individual caregiver opinions textually in a non-scrutinised and unbiased environment interviews were deemed most appropriate. The quantitative methods were employed to confirm the hypothesis and present statistically steady evidence. The qualitative data was initially presented to explore the role of the annual assessment. The anticipated scope was flexible. This was the primal attraction to qualitative data. This played a role in the type of interview carried out and stringent group interviews were discouraged.

Quota sampling which is similar to purposive sampling and involves identifying participants according to set criterion to fulfil set quotas was the most ideal method. The criterion

included; 1) early and late diagnosed children, 2) different family dynamics i.e. families with siblings and without and 3) both male and female caregivers where possible. This criterion was set as a guide. An equal number of participants from both the control and intervention groups will be sought. The interview process required the caregivers to consider the study in its entirety. Each participant had a unique study timeline projected along their pre-dated appointment times. This was designed to have minimal bearing on their already busy schedules. At this early stage in the study only 9 caregivers had completed both phases 1 and 2 ($n_{\text{control}} = 4$, $n_{\text{intervention}} = 5$). Of this subgroup only 1 caregiver was scheduled for a further appointment thus able to participate in an interview.

A semi-structured interview was carried out with 1 participant. For ease it was carried out in a designated interview room on hospital premises. The interview focussed on a challenging period the caregiver had faced over the last year, how this challenge was approached by the caregiver, family and the healthcare team and their views on how these challenges were managed as a whole. Opinions and thoughts were sought on the study intervention and the value of the CLCF-Q as a key data transfer tool between the caregiver and the CF team. The interview lasted 30 minutes.

Data collection involved full manual transcription of the audio taped interview and where possible included body language and demeanour. All correspondence between the research team and the participants also contained space for further feedback and queries. This data was also collated fully and maintained in the participants own words. The transcribed version was made available to the participant for approval of the contents. Only then were the contents analysed. The interview was interpreted through narrative analysis.

Narratives are not accurate reflections on past experiences. They are subjective accounts of what the storyteller perceives as significant details¹³⁹. The objective of the interview was to ascertain what the caregiver believed to be important in the approach to their child's treatment. The role, involvement and relationship with the CF team were of particular interest. Could a PROM aid this role? Approach to narrative data analysis was primarily thematic analysis however incorporated structural analysis. Emphasis was placed on the content of the narrative in an attempt to source out themes. Common themes will eventually be identified throughout all the narratives once data collection is completed. Structural analysis plays a role in interpreting where the storyteller places emphasis. This is important in identifying issues important to the caregiver.

Accounting for validity in qualitative methods is very difficult. Slight environmental changes including interviewer bias can restructure data entirely. The open nature of narratives is both its strength and a weakness. Whilst it allows the storyteller to lead the data it also makes it increasingly difficult to reproduce and can sometimes lead to a number of interpretations. Interviews will however, be semi structured with a clear direction of movement. The main limitation at present is the lack of participants at Phase 3 of the study. This will be addressed as the study progresses. All transcripts will be readdressed to the participant prior to analysis in the raw transcript format for permission. This also acts as respondent validation increasing the validity of any individual piece of data.

5.6.5. Study Intervention

All participants completed a CFSE-Q both at the start and at the end of the study. This captured their PSE at 2 time points before and after their child's annual assessment. Participants in the intervention group were also introduced to the CLCF-Q during their child's annual assessment appointment. At the next outpatient appointment all participants received the results of their child's annual assessment. The intervention group also received their copy of the CLCF-Q which was accompanied with feedback. This process involved the participant completing a self feedback form which asked them to predict their results of the CLCF-Q. This was followed with a formal feedback form which concluded with the accurate results of their CLCF-Q. Any issues which arose from the CLCF-Q were discussed with the researcher and advice was given regarding which member of the CF team could offer help. Participants were left to complete their appointment. All participants later received a letter outlining the results of the annual assessment. For participants in the intervention group this was followed by a summary of the results of the CLCF-Q. Following completion of the study all participants were given the opportunity to comment and feedback on the study. All parties involved with the study will also be offered a lay report.

5.3.6. Study Intervention Points

Table 12 - Study Intervention Points

Phase	Intervention	Time Point	Group	Duration
Phase 1	Invitation to participate	Outpatient clinic appointment 1	All participants	5 minutes
	Registration of interest and consent	Annual assessment appointment	All participants	10 minutes
	CFSE-Q (baseline)	Annual assessment appointment	All participants	4 minutes
	CLCF-Q	Annual assessment appointment	Intervention Group	30 minutes
Phase 2	Feedback from CLCF-Q	Annual assessment appointment	Intervention Group	30 minutes
	CFSE-Q (endpoint)	Outpatient clinic appointment 2	All participants	4 minutes
Phase 3	Semi structured interview	Outpatient clinic appointment 3	Selected participants	30 minutes

5.3.7. Interview Schedule

Meeting 1 - Last Routine Cystic Fibrosis Outpatient's Clinic, prior to the Annual Assessment

Prospective participants were informed about the study and invited to participate. They were given a copy of the, participant information sheet with a covering letter to take away and read in their own time. Details regarding researchers and an independent contact were included to answer any queries.

Meeting 2 - Annual Assessment Appointment

Prospective participants were given an opportunity to discuss the study and make any necessary queries about the study and their involvement. Informed voluntary consent was taken in writing by the research team. All participants were asked to complete the CFSE-Q taking no longer than 4 minutes. Following the successful completion of the CFSE-Q participants in the intervention group only were asked to complete the, CLCF-Q. Patients

and their caregiver were then left to continue with their annual assessment appointment. They were offered as much time as they required completing the CLCF-Q though the recommended time for completion was 30 minutes. The annual assessment appointments began between 08:30 and 09:30 and lasted up to 3½ hours. The completed CLCF-Q was collected at the end of the appointment.

Meeting 3 - Next Routine Cystic Fibrosis Outpatient's Clinic, post Annual Assessment

Participants from the intervention group were asked to complete the Self-feedback Form predicting how the CLCF-Q would score them on 17 specific areas. This was followed by a detailed, colour coded illustration of their actual results from the CLCF-Q measure. They were given the guidance, choice and opportunity to discuss their results with the researcher and different members of the CF team. Participants were then given feedback from the annual assessment. Following the clinic a summary of their annual assessment findings and any changes made were detailed in a letter and sent to all participants in the study; this is the norm. Participants in the intervention group were also sent copies of the Self-Feedback Form and an accurate Feedback Form from the CLCF-Q.

Meeting 4 – Second Routine Cystic Fibrosis Outpatient's Clinic, post Annual Assessment

Participants were asked to complete a second CFSE-Q.

Meeting 4/5 - Interviews

A further meeting was arranged with selected willing participants from across the study. This meeting took place after clinic visits and was in the form of individual semi-structured interviews. Participants were given an opportunity to discuss their thoughts and experiences and again address any concerns they may have had.

5.3.8. Scoring the Challenges of Living with Cystic Fibrosis-Questionnaire

The CLCF-Q consists of 10 domains and 62 items. Items to be equated numerically are scored on 4-point and 5-point Likert scales and yes-no format. A further 3 items require written input; these items.

Items may be positively or negatively marked. Answers are consolidated to sub-domains for feedback purposes. To illustrate the spread of opinions and feedback from caregivers in the intervention group items were equated and tabulated.

5.3.9. Scoring the CFSE-Q

Originally designed as a 10 item questionnaire where each item is marked on 4-point Likert scales, the GSE-S yields scores between 10 and 40 where 40 is the highest. The adapted model for this study, the CFSE-Q, includes the addition of 4 items specific to CF care. These follow the same trend in scoring and yield a final score of 56. For the purpose of this study scores were initially presented using just the validated items. The non-validated items are later introduced generating SE specific to caring for a child with CF.

5.3.10. Statistical Analysis

Due to time constraints, this study formed only part of the pilot study. Complete data was collected from a small number of participants (n=9). This study was not adequately powered to detect the difference between the groups, hence hypothesis testing was inappropriate. The results of this study are descriptive. The results of the CLCF-Q and CFSE-Q are tabulated as descriptive data. The baseline and endpoint spread and change in SE scores are also described as established from the CFSE-Q. Correlation between pre-annual assessment and post-assessment SE was evaluated in both the control and intervention groups. These initial results do suggest a positive trend in the intervention group. To assess the validity of these claims, the full sets of data from the entire study population (n=44) must be collated and analysed.

The reliability and validity of the questionnaires, the CLCF-Q and CFSE-Q have been shown in previous studies^{96 122}. As the validity of the questionnaires is already known it is assumed that the recorded changes in SE are valid. The reliability of the results of this study however, is difficult to anticipate at this early stage. Confounding factors such as interviewer bias and external changes may have contributed to the SE of the caregiver, i.e. issues at home. To determine the reliability of the full dataset the confounding factors will have to be accounted for.

The pilot study is due for completion in April 2011. When the full set of data has been

collected the results will be used to inform power calculations and establish the sample size required for a large multicentre study. A minimal clinical and statistical important difference in SE score will also be determined.

Chapter Four

Results

5.4. Results

The results described are preliminary results from the initial recruitment of the pilot study. The data are small in numbers and statistical analyses lack power and validity. Thus power and sample size calculations were omitted. The data displayed are descriptive in nature and depicts the data at a time point early in the study temporal. Further data will be collated as the study progresses and analysed accordingly.

The different elements of the study will be analysed separately. The data collated from the CFSE-Q is described and graphed descriptively. The qualitative element adapted from the CLCF-Q is illustrated and analysed further in the company of the written information and feedback from participants including the interview.

5.4.1. Population Data

Participants in the study were selected on the basis of their child; a patient with CF. At the time the study began 82 patients were registered at Alder Hey Children's Hospital on the CF register not including, patients registered to peripheral clinics. Of the 82 patients, 24 did not fulfil the inclusion criteria; 2 were too young and 22 were too old. A further 3 patients were excluded from the study due to social circumstances. Of the remaining, 2 more were excluded. In both cases there was more than one sibling in the caregivers/child dyad thus only one patient was included in the study.

Of the remaining 53 patients 9 are yet to be approached. Forty four families have been formally approached and presented with the participant information sheet. Due to the timeline outlined participants can only commence the study during their annual assessment appointment. The annual assessment takes place once a year at different times of the year for different patients. To date, of the 44 approached, 21 have had their annual assessment since April 2010. Three patients were lost at follow up due to appointment cancellations and changes. Eight patients were randomised to the control group and 10 to the intervention group.

There are 3 phases to the study. Phase 1, determines the caregiver's baseline SE score and takes place at the annual assessment appointment lasting the duration of the appointment. Phase 2 commences at the following outpatient clinic appointment where feedback from the annual assessment is given and continues to the next appointment where endpoint SE scores

are determined. Phase 3 involves a selection of participants from both groups for narrative based semi structured interviews.

The study is ongoing and recruitment and population records will alter accordingly.

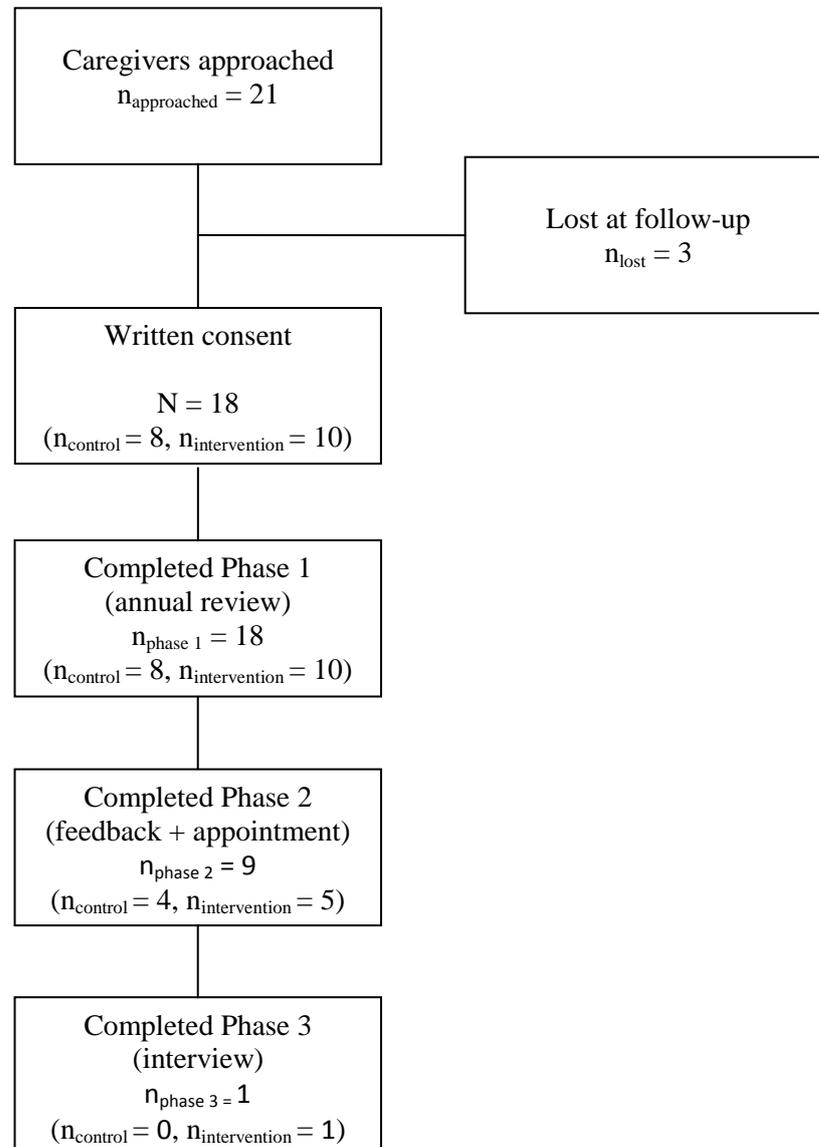


Figure 5.1: Study sample to date, August 2010

Participant characteristic data at baseline is detailed in the table below. Expectedly, the majority of caregivers were female. Only 5.9% were in receipt of help with childcare. The

ratio of female:male children was 7:11. Though patients were not stratified prior to randomisation, the age and sex distribution of the children was impartial.

Table 13 – Caregiver and Child Characteristics at Baseline

Characteristic	Control Group	Intervention Groups	Total
Relationship to Child			
Mother	7 (38.9%)	10 (55.6%)	17 (94.4%)
Father	1 (5.6%)	0 (0%)	1 (5.6%)
Child care support?			
Yes	0 (0%)	1 (5.6%)	1 (5.6%)
No	8 (44.4%)	9 (50.0%)	17 (94.4%)
Sex age of child			
Female	3 (16.7%)	4 (22.2%)	7 (38.9%)
Male	5 (27.8%)	6 (33.3%)	10 (55.6%)
Mean age of child			
1 - 4	0 (0%)	0 (0%)	0 (0%)
5 – 9	3 (16.7%)	4 (22.2%)	7 (38.9%)
9 - 13	5 (27.8%)	6 (33.3%)	10 (55.6%)

5.4.2. Quantitative Data

5.4.2.1. Cystic Fibrosis Self Efficacy-Questionnaire

5.4.2.1.1. Item Analysis

5.4.2.1.1.1. Analysis of Validated Items in the CFSE-Q

Table 14 - Baseline Scores of Previously Validated Items on the CFSE-Q

Item	Not at all true	Hardly true	Moderately true	Exactly true	Group
1 I can always manage to solve difficult problems if I try hard enough	-	-	6	2	Control
	-	-	5	5	Intervention
	0 (0%)	0 (0%)	11 (61.1%)	7 (38.9%)	Total
3 If someone opposes me, I can find the means and ways to get what I want	-	1	6	1	Control
	1	1	6	2	Intervention
	1 (5.6%)	2 (11.1%)	11 (61.1%)	3 (16.7%)	Total
4 It is easy for me to stick to my aims and accomplish my goals	-	-	4	4	Control
	-	1	7	2	Intervention
	0 (0%)	1 (5.6%)	11 (61.1%)	6 (33.3%)	Total
5 I am confident that I could deal efficiently with unexpected events	-	1	3	4	Control
	-	-	6	4	Intervention
	0 (0%)	1 (5.6%)	9 (50.0%)	8 (44.4%)	Total
6 Thanks to my resourcefulness, I know how to handle unforeseen situations	-	1	2	5	Control
	-	-	4	5	Intervention
	0 (0%)	1 (5.6%)	6 (33.3%)	10 (55.6%)	Total
8 I can solve most problems if I invest the necessary effort	-	-	3	5	Control
	-	-	7	3	Intervention
	0 (0%)	0 (0%)	10 (55.5%)	8 (44.4%)	Total
10 I can remain calm when facing difficulties because I can rely on my coping abilities	-	2	2	4	Control
	-	1	7	2	Intervention
	0 (0%)	3 (16.7%)	9 (50.0%)	6 (33.3%)	Total
11 When I am confronted with a problem, I can usually find several solutions	-	2	3	3	Control
	1	1	6	2	Intervention
	1 (5.6%)	3 (16.7%)	9 (50.0%)	5 (27.8%)	Total
12 If I am in trouble, I can usually think of a solution	-	-	5	3	Control
	-	-	7	3	Intervention
	0 (0%)	0 (0%)	12 (66.7%)	6 (33.3%)	Total
14 I can usually handle whatever comes my	-	-	3	5	Control
	-	-	7	3	Intervention

way	0 (0%)	0 (0%)	10 (55.6%)	8 (44.4%)	Total
-----	--------	--------	------------	-----------	-------

Table 15 - Endpoint Scores of Previously Validated Items on the CFSE-Q

Item	Not at all true	Hardly true	Moderately true	Exactly true	Group
1 I can always manage to solve difficult problems if I try hard enough	-	-	2	2	Control
	-	-	2	3	Intervention
	0 (0%)	0 (0%)	4 (44.4%)	5 (55.6)	Total
3 If someone opposes me, I can find the means and ways to get what I want	-	1	1	2	Control
	1	2	2	-	Intervention
	1 (11.1%)	3 (33.3%)	3 (33.3%)	2 (22.2%)	Total
4 It is easy for me to stick to my aims and accomplish my goals	-	-	3	1	Control
	-	1	3	1	Intervention
	0 (0%)	1 (11.1%)	6 (66.7%)	2 (22.2%)	Total
5 I am confident that I could deal efficiently with unexpected events	-	-	2	2	Control
	-	-	1	4	Intervention
	0 (0%)	0 (0%)	3 (33.3%)	6 (66.7%)	Total
6 Thanks to my resourcefulness, I know how to handle unforeseen situations	-	1	-	3	Control
	-	-	3	2	Intervention
	0 (0%)	1 (11.1%)	3 (33.3%)	5 (55.6)	Total
8 I can solve most problems if I invest the necessary effort	-	-	3	1	Control
	-	-	1	4	Intervention
	0 (0%)	0 (0%)	4 (44.4%)	5 (55.6)	Total
10 I can remain calm when facing difficulties because I can rely on my coping abilities	-	1	1	2	Control
	-	-	3	2	Intervention
	0 (0%)	1 (11.1%)	4 (44.4%)	4 (44.4%)	Total
11 When I am confronted with a problem, I can usually find several solutions	-	1	1	2	Control
	-	1	2	2	Intervention
	0 (0%)	2 (22.2%)	3 (33.3%)	4 (44.4%)	Total
12 If I am in trouble, I can usually think of a solution	-	-	2	2	Control
	-	-	2	3	Intervention
	0 (0%)	0 (0%)	4 (44.4%)	5 (55.6)	Total
14 I can usually handle whatever comes my	-	-	2	2	Control
	-	-	2	3	Intervention

way	0 (0%)	0 (0%)	4 (44.4%)	5 (55.6)	Total
-----	--------	--------	-----------	----------	-------

Figure5.2: Self Efficacy Score at Baseline – Intervention Group

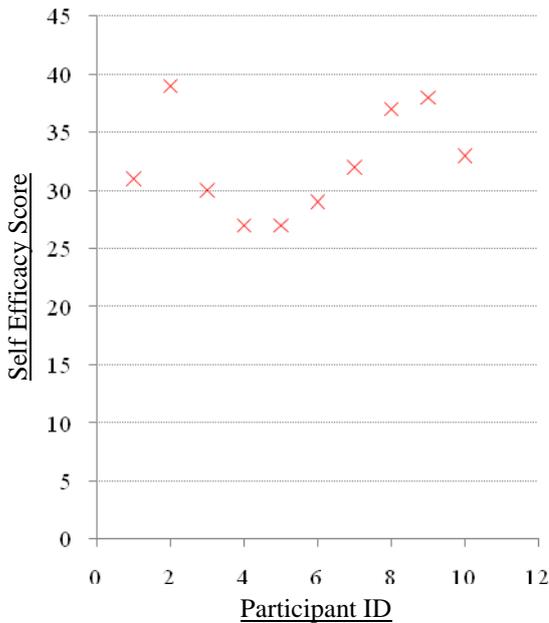


Figure5.3: Self Efficacy Score at Baseline – Control Group

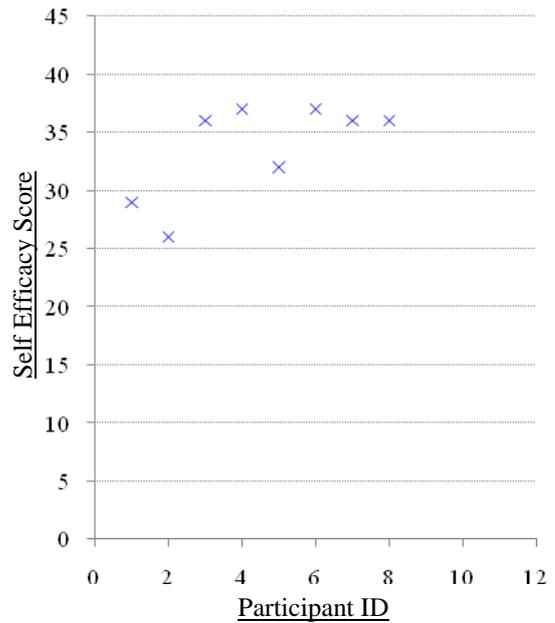
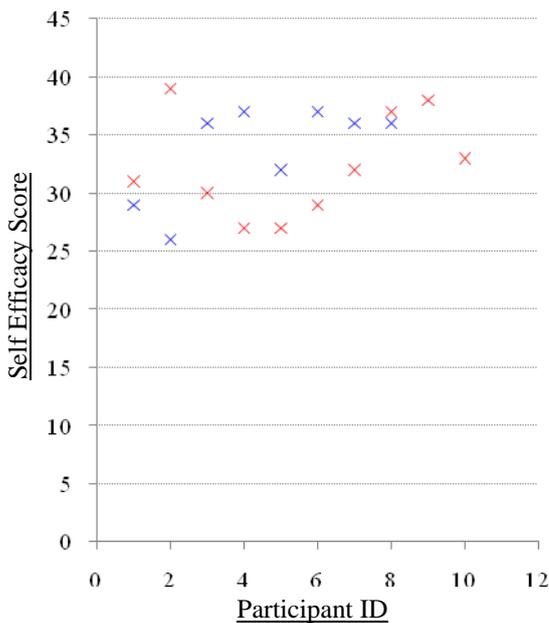


Figure5.4: Self Efficacy Score at Baseline – Comparison



Baseline SE across all participants who completed phase 1 only, ranged from 26 – 39 (mean 32.9). SE of participants in the control group who completed phase 1 only, ranged from 26 – 37 (mean 33.6) whilst ranging 27 – 39 (mean 32.3) in the intervention group. Baseline SE across all participants who completed phase 1 and phase 2 ranged from 26 – 39 (mean 30.8). SE of participants in the control group who completed phase 1 and phase 2 ranged from 26 – 36 (mean 30.8) whilst ranging 27 – 39 (mean 30.8) in the intervention group.

Figure5.5: Self Efficacy Score at Endpoint – Intervention Group

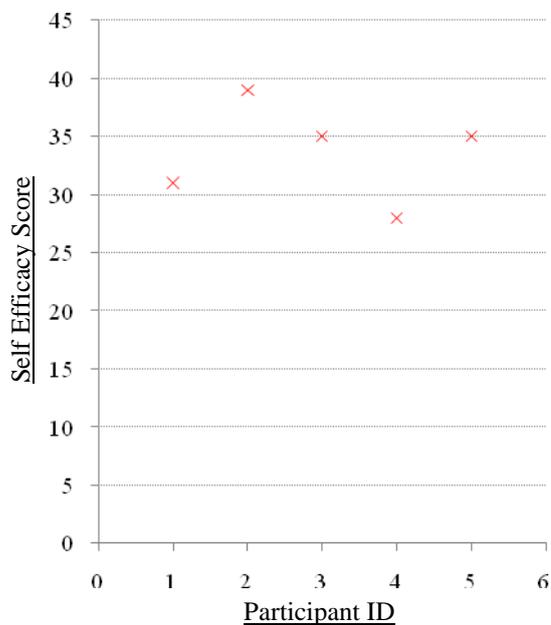


Figure5.6: Self Efficacy Score at Endpoint – Control Group

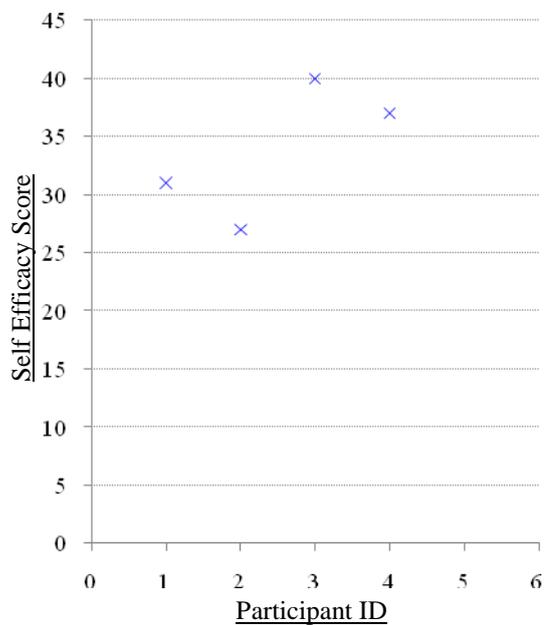
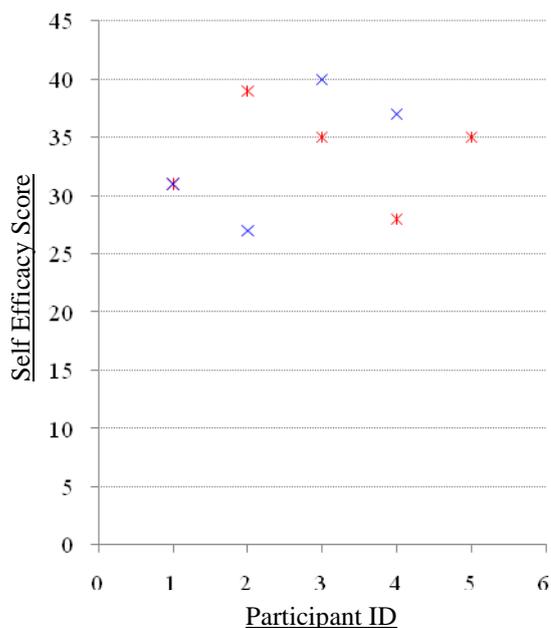


Figure5.7: Self Efficacy Score at Endpoint – Comparison



Endpoint SE across all participants ranged from 27 – 40 (mean 33.6). SE in control group ranged from 27 – 40 (mean 33.5) whilst ranging 28 – 39 (mean 33.6) in the intervention group. Non-validated items specific to CF care have not been included in final scores.

Figure5.8: Self Efficacy Score at Baseline and Endpoint – Intervention Group

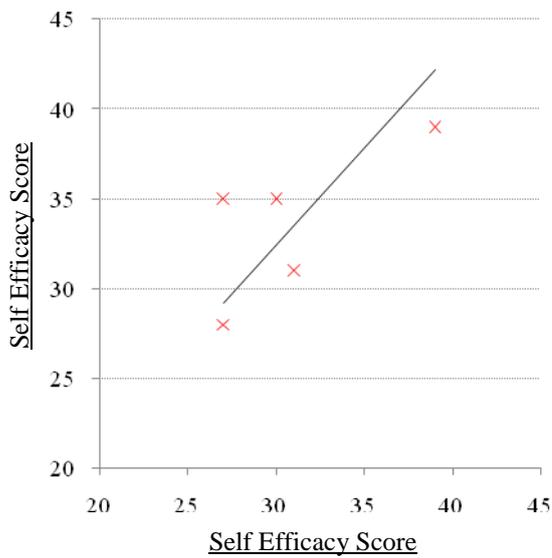


Figure5.9: Self Efficacy Score at Baseline and Endpoint – Control Group

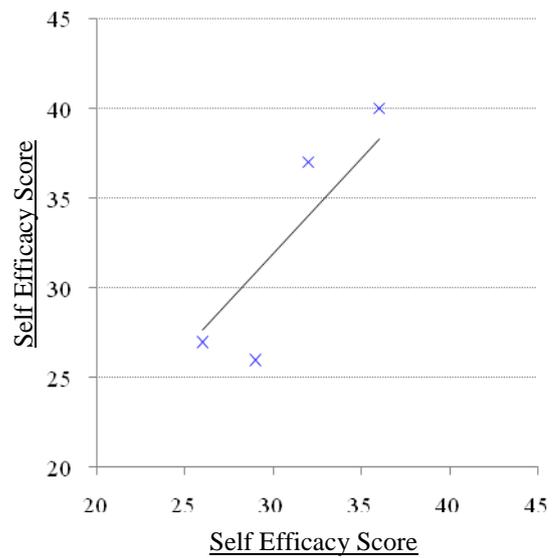
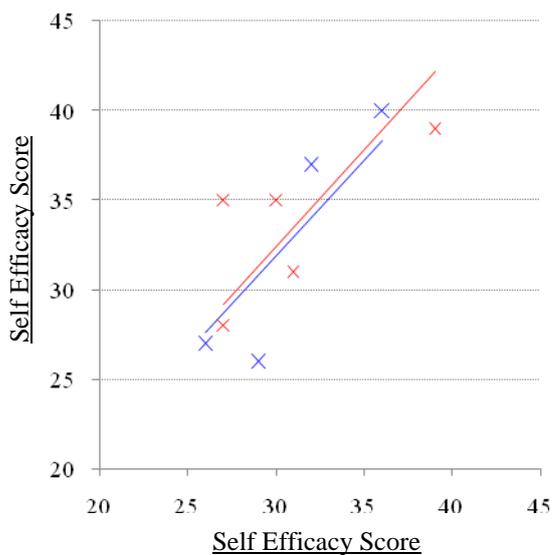


Figure 5.10: Self Efficacy Score at Baseline and Endpoint – Comparison



The correlation between baseline and endpoint SE is closely matched in both control and intervention groups. However, non-validated items specific to CF care have been excluded from analyses.

5.4.2.1.1.2. Analysis of Non-Validated Items on the CFSE-Q

Table16 - Baseline Scores of Non-Validated Items on the CFSE-Q

Item	Not at all true	Hardly true	Moderately true	Exactly true	Group
2 I face problems on a daily basis	-	2	4	2	Control
	-	5	4	1	Intervention
	0 (0%)	7 (38.9%)	8 (44.4%)	3 (16.7%)	Total
7 I do have the support I need to solve problems	-	1	2	5	Control
	-	2	3	5	Intervention
	0 (0%)	3 (16.7%)	5 (27.8%)	10 (55.6%)	Total
9 I can only solve a problem if I expected it to happen	2	4	2	-	Control
	4	3	-	3	Intervention
	6 (33.3%)	7 (38.9%)	2 (11.1%)	3 (16.7%)	Total
13 I never feel my views are fully appreciated	3	3	1	1	Control
	2	4	3	1	Intervention
	5 (27.8%)	7 (38.9%)	4 (22.2%)	2 (11.1%)	Total

Table 17 - Endpoint Scores of Non-Validated Items on the CFSE-Q

Item	Not at all true	Hardly true	Moderately true	Exactly true	Group
2 I face problems on a daily basis	-	-	1	3	Control
	-	3	2	-	Intervention
	0 (0%)	3 (33.3%)	3 (33.3%)	3 (33.3%)	Total
7 I do have the support I need to solve problems	-	1	1	2	Control
	-	1	-	4	Intervention
	0 (0%)	2 (22.2%)	1 (11.1%)	6 (66.7%)	Total
9 I can only solve a problem if I expected it to happen	1	1	2	-	Control
	3	2	-	-	Intervention
	4 (44.4%)	3 (33.3%)	2 (22.2%)	0 (0%)	Total
13	1	-	2	1	Control

I never feel my views are fully appreciated	2	2	1	-	Intervention
	3 (33.3%)	2 (22.2%)	3 (33.3%)	1 (11.1%)	Total

Figure 5.11: Self Efficacy Score at Baseline and Endpoint – Intervention Group

Figure 5.12: Self Efficacy Score at Baseline and Endpoint – Control Group

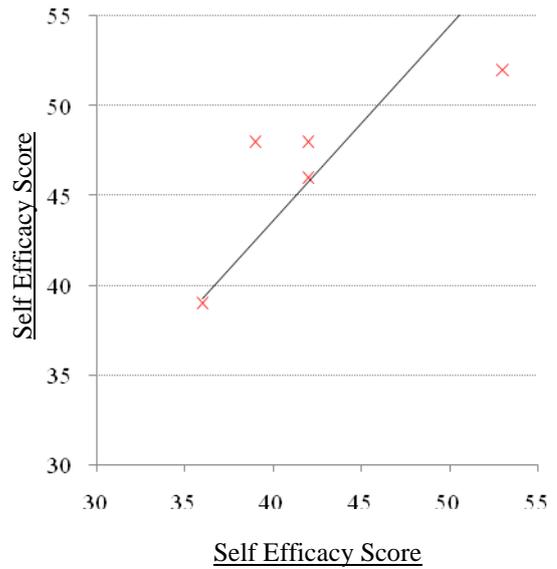
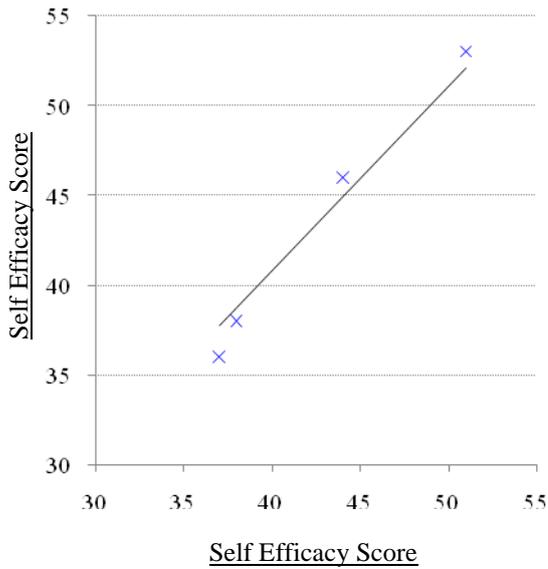
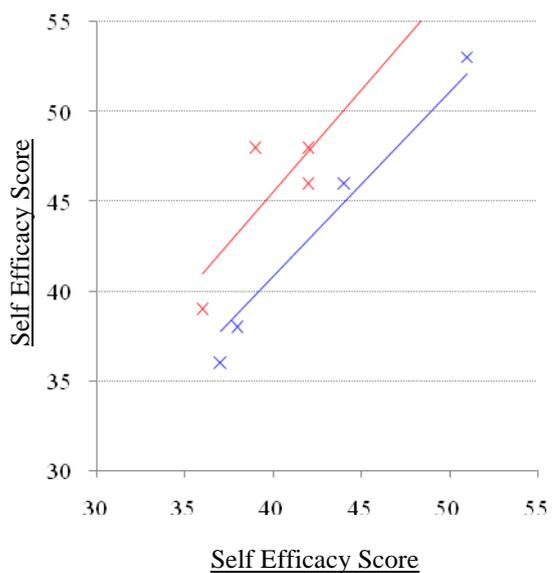
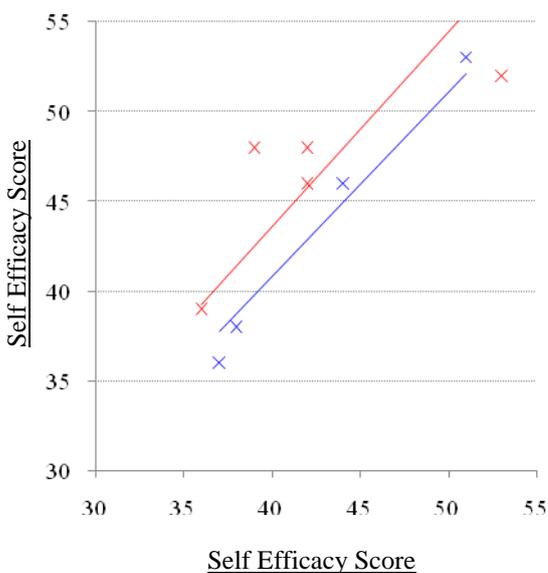


Figure 5.13: Self Efficacy Score at Baseline and Endpoint – Comparison

Figure 5.14: Self Efficacy Score at Baseline and Endpoint – Comparison



Baseline SE across all participants who completed phase 1 and phase 2 ranged from 36 – 53 (mean 42.4). SE of participants in the control group who completed phase 1 and phase 2 ranged from 37 – 51 (mean 42.5) whilst ranging 36 – 53 (mean 42.4) in the intervention group.

Endpoint SE across all participants ranged from 36 - 53 (mean 45.1). SE in the control group ranged from 36 - 53 (mean 43.3) whilst ranging 39 - 52 (mean 46.6) in the intervention group. Non-validated items specific to CF care have not been included in final scores.

Following the inclusion of the non-validated items specific to CF care there is a clear difference in correlation between baseline and endpoint SE between the control and intervention groups. The rate of change between baseline and endpoint SE is greater in the intervention group (Figure 5.13). Figure 5.14 illustrates the rate of change following the removal of a possible outlier in the data. The rate of change in Figure 5.14 is further apparent.

5.4.2.1.2. Analysis of all Items in the CFSE-Q

Table 18 : Comparison of Consistent Changes in Items on the Cystic Fibrosis Self Efficacy-Questionnaire

Item	Change in Control Group	Change in Intervention Group	Type of Change
1	✓	✓	Positive
I can always manage to solve difficult problems if I try hard enough			Negative
2			Positive
I face problems on a daily basis	✘		Negative
3	✓		Positive
If someone opposes me, I can find the means and ways to get what I want		✘	Negative
4			Positive
It is easy for me to stick to my aims and accomplish my goals			Negative
5		✓	Positive
I am confident that I could deal efficiently with unexpected events			Negative
6	✓		Positive
Thanks to my resourcefulness, I know how to handle unforeseen situations			Negative
7		✓	Positive
I do have the support I need to solve problems			Negative
8		✓	Positive
I can solve most problems if I invest the necessary effort			Negative
9			Positive
I can only solve a problem if I expected it to happen	✘		Negative
10			Positive
I can remain calm when facing difficulties because I can rely on my coping abilities			Negative
11	✓	✓	Positive
When I am confronted with a problem, I can usually find several solutions			Negative
12		✓	Positive
If I am in trouble, I can usually think of a solution			Negative
13		✓	Positive
I never feel my views are fully appreciated	✘		Negative
14		✓	Positive
I can usually handle whatever comes my way			Negative
Total	4	8	Positive

In the control group there were several consistent changes in the CFSE-Q that were mirrored between participants. These changes in SE occurred between the annual assessment appointment and the outpatient clinic appointment following feedback. Consistent changes occurred in items 1, 2, 3, 6, 9, 11 and 13. Item 1 increased in 50% of participants and related to their belief in their problem solving abilities.

Item 1 - 'I can always manage to solve difficult problems if I try hard enough.'

Item 3 which considered facing opposition, item 6 considering handling unforeseen circumstances and item 11 dealing with confrontational challenges all consistently increased in 50% of the participants.

Item 3 - 'If someone opposes me, I can find the means and ways to get what I want.'

Item 6 - 'Thanks to my resourcefulness, I know how to handle unforeseen situations.'

Item 11 - 'When I am confronted with a problem, I can usually find several solutions.'

Interestingly, despite these positive changes in SE participants also showed similar changes in items 2, 9 and 13. These items are 3 of the 4 non-validated items that were included in our version of the CFSE-Q. They were negatively marked on the 4-point Likert scale thus an increase in the scores would show a decrease in SE.

Item 2 - 'I face problems a daily basis.'

Item 9 - 'I can only solve a problem if I expected it to happen.'

Item 13 - 'I never feel my views are fully appreciated.'

Fifty percent of participants showed an increase in scores of items 2 and 9. Seventy five percent showed an increase in item 13. As these were negatively marked on the Likert scale an increase in the score translated to the statements being truer. Thus some participants in the control group felt, that they faced more problems, found it harder to solve problems and most felt that their views weren't fully appreciated following the feedback they received from the annual assessment compared to their baseline SE score.

The intervention group portrayed consistent exaggerated changes in SE scores. Item 1, 11, 12 and 14 were increased in 40% of participants. Items 5 and 8 were increased in 60% of participants.

Item 1 - 'I can always manage to solve difficult problems if I try hard enough.'

Item 11 - 'When I am confronted with a problem, I can usually find several solutions.'

Item 12 - 'If I am in trouble, I can usually think of a solution.'

Item 14 - 'I can usually handle whatever comes my way.'

Item 7, an additional non-validated item increased score in 60% of participants. Item 13, also a non-validated item decreased in 40% of participants. However it is negatively scored thus a decrease in score reflects an increase in SE. However, item 3 which related to facing opposition decreased in 40% of participants.

Item 7 - 'I do have the support I need to solve problems.'

Item 13 - 'I never feel my views are fully appreciated.'

Item 3 - 'If someone opposes me, I can find the means and ways to get what I want.'

SE scores between baseline and endpoint remained consistent or increased across all participants in both groups. There were no negative changes in overall SE score, determined using validated items, between time points. The inclusion of non-validated items in assessment reveals a significant and consistent negative change in 3 of the 4 items in the control group in comparison to only one item in the intervention group. This is further supported by the significant consistent increase in 8 of the 10 validated items in the intervention group compared to a consistent increase in only 4 items in the control group.

5.4.3. Qualitative Data

5.4.3.1. Challenges of Living with Cystic Fibrosis-Questionnaire

Table 19 – Challenges of Living with Cystic Fibrosis-Questionnaire

Item	Subscale	N	
Family Lifestyle			
Are you?	Lone caregiver	2 (20%)	
	With spouse	8 (80%)	
	With family	0 (0%)	
How many children do you care for in your family?	5	1 (10%)	
	3	1 (10%)	
	2	7 (70%)	
	1	1 (10%)	
How many children with CF do you have living with you?	2	2 (20%)	
	1	8 (80%)	
How does your family divide childcare relating to CF?	I do it all	4 (40%)	
	I receive some help	6 (60%)	
How would you describe your general family lifestyle?	Is it relaxed or stressed out?	Stressed out	1 (10%)
		Sometimes stressed	5 (50%)
		Relaxed	4 (40%)
	Is it busy or laid back?	Busy	6 (60%)
		Sometimes busy	3 (30%)
		Laid back	1 (10%)
	Do you work together or work as individuals?	Work as individuals	1 (10%)
		Sometime work alone	3 (30%)
		Work together	6 (60%)
	Is it disorganised or organised?	Disorganised	0 (0%)
		Sometimes disorganised	4 (40%)
		Organised	6 (60%)
	Do you have no fixed routines or fixed routines?	No fixed routines	0 (0%)
		Sometimes have routines	5 (50%)
		Fixed routines	5 (50%)
How well do you think you are juggling the demands of CF with the needs of your family?	Great difficulty	1(10%)	
	Marginal difficulty	4 (40%)	
	No difficulty	5 (50%)	
How well do you think your family as whole handles the challenges of CF?	Great difficulty	3 (30%)	
	Marginal difficulty	4 (40%)	
	No difficulty	3 (30%)	
CF Background			
*All children were pancreatic insufficient			
Over the last two weeks, how has your child been?	Unwell	0 (0%)	
	A mixture	2 (20%)	
	Mostly well	8 (80%)	
Has your child ever had a hospital admission?	Yes	10 (100%)	

	No	0 (0%)
--	----	--------

Child's Character			
Please grade the following statements	My child makes more demands on me than I expected	Agree	3 (30%)
		Sometimes	2 (20%)
		Disagree	5 (50%)
	My child goes to bed easily	Agree	7 (70%)
		Sometimes	2 (20%)
		Disagree	1 (10%)
	My child sleeps throughout the night	Agree	7 (70%)
		Sometimes	2 (20%)
		Disagree	1 (10%)
	It takes a long time for my child to settle with new routines	Agree	1 (10%)
		Sometimes	4 (40%)
		Disagree	5 (50%)
	My child is easily upset by things generally	Agree	3 (30%)
		Sometimes	4 (40%)
		Disagree	3 (30%)
	My child is very moody	Agree	1 (10%)
		Sometimes	4 (40%)
		Disagree	5 (50%)
	My child reacts very strongly when something happens that s/he doesn't like	Agree	6 (60%)
		Sometimes	4 (40%)
		Disagree	0 (0%)

Challenges to Family Life			
How supported do you feel by the following groups of people?	Family members	Not at all supported	3 (30%)
		Very supported	7 (70%)
	Friends	Not at all supported	2 (20%)
		Very supported	8 (80%)
	Another parent whose child has CF	Not at all supported	6 (60%)
		Very supported	4 (40%)
	CF team	Not at all supported	0 (0%)
		Very supported	10 (100%)
	GP	Not at all supported	2 (20%)
		Very supported	8 (80%)
	Pharmacy	Not at all supported	1 (10%)
		Very supported	9 (90%)
Caring for a child with CF can involve extra expense. How difficult is it for you to manage this?	Very difficult	1 (10%)	
	Moderately	3 (30%)	
	Not at all difficult	6 (60%)	
To what extent do you think CF has changed your work pattern?	A great deal	8 (80%)	
	Not at all	2 (20%)	
How often have you had a disturbed night's sleep in the past 2 weeks?	Frequent	1 (10%)	
	Some	1 (10%)	
	Few	8 (80%)	

Hopes and Worries			
Some say that living with CF is like a balance of hope and worry: What hope do you have for your child?	S/he will adjust well to secondary school	Not hopeful	1 (10%)
		Hopeful	9 (90%)
	S/he will go on to higher education	Not hopeful	1 (10%)
		Hopeful	9 (90%)
	S/he will have a job	Not hopeful	2 (20%)
		Hopeful	8 (80%)
	S/he will have a family of his/her own?	Not hopeful	4 (40%)
		Hopeful	6 (60%)
	S/he will continue to be as well as s/he is now?	Not hopeful	3 (30%)
		Hopeful	7 (70%)
	There will be an advance in science that will help my child?	Not hopeful	1 (10%)
		Hopeful	9 (90%)
It is difficult to predict what the future holds in relation to CF. To what extent does this uncertainty affect your family's approach to life?	A great deal	2 (20%)	
	Moderately	4 (40%)	
	Not at all	4 (40%)	
How much does the responsibility of looking after a child with CF affect you?	A great deal	7 (70%)	
	Moderately	1 (10%)	
	Not at all	2 (20%)	
How much is your child's height a worry for you?	A great deal	2 (20%)	
	Moderately	5 (50%)	
	Not at all	3 (30%)	
How much is your child's weight a worry for you?	A great deal	2 (20%)	
	Moderately	3 (30%)	
	Not at all	5 (50%)	
How worried are you about a change in your child's lung function?	A great deal	6 (60%)	
	Moderately	2 (20%)	
	Not at all	2 (20%)	

CF Routines			
How easy was it to establish the CF care routine after our child was diagnosed?		Not at all easy	4 (40%)
		Moderately easy	4 (40%)
		Very easy	2 (20%)
How much of a problem is it to manage the daily routine of CF now?	Mealtimes	A constant problem	4 (40%)
		Sometimes a problem	2 (20%)
		No problem	4 (40%)
	Digestion	A constant problem	5 (50%)
		Sometimes a problem	3 (30%)
		No problem	1 (10%)
	Taking enzymes	A constant problem	7 (70%)
		Sometimes a problem	3 (30%)
		No problem	0 (0%)
	Taking vitamins/oral antibiotics	A constant problem	8 (80%)
		Sometimes a problem	2 (20%)
		No problem	0 (0%)
	Doing physiotherapy	A constant problem	5 (50%)
		Sometimes a problem	2 (20%)
		No problem	3 (30%)
	Doing nebulised medications *n/a for 2 participants	A constant problem	4 (50%)
		Sometimes a problem	2 (25%)
		No problem	2 (25%)
With all the things that need to be done, it may be overwhelming at times. How true has this been for you over the last 2 weeks?		Very true	4 (40%)
		Neutral	4 (40%)
		Not at all true	2 (20%)
Community Support			
What quality of relationship do you have with your local GP/surgery?		Not at all good	2 (20%)
		Moderately good	3 (30%)
		Very good	5 (50%)
How helpful is your pharmacist?		Not at all helpful	0 (0%)
		Moderately helpful	1 (10%)
		Very helpful	9 (90%)
What sort of relationship do you have with your child's minder/nursery/school?		Not at all good	1 (10%)
		Moderately good	1 (10%)
		Very good	8 (80%)
CF Clinic & Pharmacy Visits			
How long on average do you spend in the clinic at each appointment?		4 hours	1 (10%)
		3 hours	7 (70%)
		2 hours	2 (20%)
Please think about your last visit to the pharmacy. How acceptable was the wait for medicine?		Very unacceptable	3 (30%)
		Neutral	5 (50%)
		Very acceptable	2 (20%)
How much information would you like to have from the CF team about your child's condition or treatments?		More information	3 (30%)
		The same as now	7 (70%)
During hospital admissions patients and caregivers experience stress regarding staying in overnight, disruptions to family life, getting good care in hospital, getting the intravenous line in, child's loneliness and communication with healthcare professionals.			

Collective data from the CLCF-Q revealed 20% of participants were lone caregivers and 90% of participants had between 2 and 5 children to care for. In 20% of cases there were 2 children in the caregiver/child dyad with CF furthermore 40% of primary caregivers admitted to doing all the work at home. Resultantly, 10% acknowledged that their family lifestyle was stressed and a further 50% agreed that it was sometimes stressful.

Understandably lifestyle was described as busy by 60% though 60% did also work together juggling the demands of CF as family. Ten percent managed this with great difficulty; a further 40% managing with marginal difficulty. Focussing on the family, 40% were coping with marginal difficulty whilst 30% of families handled challenges with great difficulty.

All children in the intervention had experienced an inpatient stay at some point since diagnosis. Clinically, 80% of children were described as mostly well over the fortnight prior to the annual assessment appointment. As a result, the caregiver's SE is expected to be high due to the lower levels of stress experienced when a child is well. In general, when asked about their child's character 30% were described as making more demands than expected and getting upset by things easily, a further 60% of children react very strongly when something happens that they don't like. Settling into routines, mood and sleeping were issues in a minority of cases but were generally well managed.

Family and friends play a pivotal role in supporting caregivers in 70% and 80% of cases respectively. Caregivers are not supported by other parents and families who are living with CF and a number of participants expressed concern regarding this. Though ill advised due to cross infection parents would like to have contact with other parents in a similar situation. One caregiver stated that this lack of contact, "affected her a great deal." All participants felt particularly supported by the CF team and praised their efforts. A small minority expressed further concern regarding their relationship with the GP and pharmacist.

Caring for a child with a chronic disease can add to expenses. Transport, parking, particular diet requirements, "cost of meals for parents during hospital admissions," loss of income and adaptations made to the home all contribute to this. Added expenses were an issue with 40% of caregivers and 80% admitted that they had sacrificed previous paid employment to care for their child. As well as expense, time is another burden on the caregiver and family. On average participants spent 2 hours 54 minutes in clinic every 10 weeks.

Hopes and worries play a major role in managing CF. Whilst most were hopeful regarding short term commitments including secondary school, higher education and finding

employment less were hopeful of long term prospects including their child raising a family of their own and continuing to be as well as they are now. Ninety percent were also hopeful of an advance in scientific research dramatically changing their lives and all participants expressed the benefits of taking part in research. This uncertainty regarding their future affected the families approach to life in 60% of cases and 70% of primary caregivers stated that the responsibility of looking after a child with chronic disease affected them greatly. Height, weight and lung function were amongst the main concerns, with the latter being a concern in 60% of cases.

A diagnosis of CF results in substantial changes to family life. Establishing a routine can be difficult and 40% described it as a difficult challenge. Amid the concerns, were establishing mealtimes (40%) and promoting eating habits in particular taking enzymes (70%), vitamins and oral antibiotics (80%). Doing physiotherapy and administering nebulised medications were constant difficulties for the participants. During hospital admissions patients and caregivers experience stress regarding staying in overnight, disruptions to family life, getting good care in hospital, getting the intravenous line in, the child's loneliness and communication with healthcare professionals. Whilst all participants were very pleased with the service received from the CF team, 30% of caregivers expressed a need for further information about their child's CF.

The CLCF-Q not only allows caregivers to express concerns on certain areas it also enables them to voice their opinions on matters which concern them specifically.

Question 28 enquired about the degree of change in work pattern attributed to being a primary caregiver. One parent stated, "All my attention and energy goes on my child." Caring for a child with CF is a full time commitment leaving little time for other ADL particularly employment. Caregivers expressed feeling guilt and felt that they had to be at home for their child's, "early years," for several reasons. A number of caregivers had, "changed to part time employment from full time" however this wasn't always ideal. One caregiver said, "Part time, annual leave used for appointments and illness. Employers not helpful or colleagues when off with son." She went on to explain that her employers were not sympathetic to the needs of her child and requesting time off work to care for him was an awkward and unwelcomed gesture.

During the multiple appointments the caregiver attends regarding their child's CF, they are rarely asked how they are feeling and coping. Parents seldom experience respite and so it is important that they recognise their own limits. When asked how they detect their limits, a trend was apparent. Many talked about feeling, more, "stressed and tired," than usual.

References to mood and losing their, “temper easily” were also evident. One caregiver wrote, “I may feel stressed and tearful for nothing at all” another added, “When we lose sight of ourselves and are overwhelmed.” In the fortnight preceding the annual assessment appointment, 40% of the caregivers had felt frequently overwhelmed and a further 40% felt overwhelmed sometimes.

Amongst other things caregivers balance their worries and hopes. This affects both ADL and their approach to the future. Worrying about how their child’s, “health will be in the future” and “about life expectancy,” was a common concern. Caregivers expressed its impact on planning for the future and their outlook. It is important to consider that co-morbidities do exist within the CF population. One caregiver stated “His CF/autism combined means we have to plan for the future more than other families.”

For parents lung function results provide a good indication of how well their child is coping with CF. Particularly at the annual assessment, caregivers felt that their main concern was the lung function result. When asked about their main worry comments consistently followed a similar theme stating, “the chest,” “lung function decreasing slightly,” “lung damage that’s irreversible” and “that it will get worse and affect her breathing preventing her from doing her activities.” General worries were described as the child not, “staying well,” “the unknown” and the need for routine and non routine admissions for interventions like, “needing a new port.”

Coupled with the worries were similar hopes shared by caregivers. These included a strong sense of hope for the future with the possibility of, “a cure,” hopes of, “treatments getting better,” and innovative “research.” For the children who had been well, caregivers were subsequently hopeful that they would stay this well. The care they received by the CF team also contributed to their hopes and happiness, I hope, “that she is happy and well looked after by the CF Team.” Interestingly, even though the CLCF-Q enquired about the caregiver’s thoughts and abilities, one caregiver commented, “I am hopeful about my child’s ability to cope with CF.”

5.4.3.2. Interview Analysis

The aim of this study was to explore the practicality of measuring the SE of caregivers of children with CF., with an aim to improve SE using a simple feedback intervention employing the use of the CLCF-Q. It was perceived that the variations in SE would be apparent.

In order to improve SE however, one must understand the determining factors. It is important to understand the caregiver's point of view. How they recognise a challenge, their familial approach to the challenge and the meaning they associate with the CF team's involvement are all key principles that need to be understood in order to improve a caregiver's SE.

During a semi-structured interview with a caregiver randomised to the intervention group 2 challenges recently faced by the family were discussed. The caregiver Mandy (pseudonyms have been applied throughout the script) was mother to 2 children with CF; a boy aged 12 (Kevin) and a girl (Sarah) aged 10. The father (James) remained in full time employment hence the participant was the primary caregiver at home. When asked to think about a recent challenge Mandy began with,

'Change'

“Kevin started seniors and that was a bit of a challenge...Because at junior school, he was always given the right dose and we'd ask the staff to get them which all his friends new about anyway. So that wasn't a problem but in seniors I didn't want to... separate him from his friends for him to be going off somewhere. I didn't know whether he might not want to tell his friends that he's got CF because it was only him going from juniors so he didn't know anybody. So I thought it would be up to him to tell them if he wanted to. So he had them in his bag... and he often forgot, which led to him having a lot of pain and also when he needs to go to the toilet he's got to go and, and they're not allowed to go out in class so he has a toilet pass. So really all the really it was going from the comfort of his junior school where everyone knew him to like a whole new um environment where he didn't know anyone. That was quite daunting.”

The first challenge described is a uniformly experienced by all caregivers – It is a period of change. A number of specific issues are addressed here; changing school is the primary challenge but within that are the issues of establishing relationships, medications, establishing routine and the disease management itself.

Mandy talked about, Kevin establishing new relationships with his peers and placed emphasis on the effect of CF on their relationship. She mentions the concepts of separation from friends and the difficulties of explaining CF. The effect this challenge has on Kevin's disease status is also discussed.

'Independence'

"I didn't know whether he might not want to tell his friends that he's got CF because it was only him going from juniors so he didn't know anybody. So I thought it would be up to him to tell them if he wanted to."

The change in power over administering medication from school staff to Kevin and the option to tell his friends being left to him could be interpreted as a step towards establishing a degree of independence.

'Support'

The interview moved from the actual challenge itself to the management of the challenge and the first port of called was discussed.

"Jenny! I just phoned her! Cos Jenny came to the Junior School when... because we moved when he started in Year 1. He wasn't in reception year. We were living somewhere else. So uh Jenny came to that school when he started juniors and Jenny went to the senior school and talked to the teachers he was going to have and the head teacher... and that was a big help. Jenny just takes... Jenny just takes the number of the school and arranges everything so I don't even know when she's going she just arranges it with the school. So that just – that takes all weight."

Mandy seemed very pleased with the service and relieved that it was something less for her to worry about. The interview progressed to a discussion about the study she had recently completed and her views on its worth as a permanent intervention in the annual assessment process.

'I don't know any different'

The different components of the study were discussed and the value of each part of the intervention was considered.

"Yes I think its... helpful, it's helpful to pick up on anyone who's having problems. You know, you sort of learn how to deal with the problems. It would detect the different problems people are having. Helpful to me as well because of the feedback you get from it and also it just makes you realise, because it's something I've always done. Especially with me having two children with CF, I

don't know any different. I need stopping and actually think about how I am coping. It didn't take much time at all and I think any studies and research is a good thing.

I think all of the study was important. But I think the bit for me was... sort of how you're dealing with things how you're... especially the bit where, it says do you feel overwhelmed. And I think the first time I did it, I said no, and then I think the second time I said yes because that was how I felt at the time, because, they both had symptoms and I go for so long with them being well and when they're both not well at the same time it does overwhelm me a bit."

Mandy talked about the potential of the CLCF-Q to pick up on problems the caregiver is experiencing.

'I don't normally get asked if I'm overwhelmed'

Her particular interest was in the feedback. Seldom do caregivers get asked how they feel and yet it is their health which plays a pivotal role in that of their child's. Her reference to coping and dealing with the challenges, including the sense of feeling overwhelmed was characteristic of how the CLCF-Q portrayed the sample of caregivers in the intervention group. They all expressed a concern over their ability to cope with challenges and felt that more emphasis on their health would be of benefit.

"I don't normally get asked if I'm overwhelmed. Normally the focus is on the children not really us. So it's all about how they are. I feel fine about it though... it would be helpful. It would. But I also think that if I do have problems and I felt I couldn't cope I do always ask for help. I'd ask (laughter) Jenny and Elinor... again. There is always someone there. There always has been."

'We'

The next challenge reviewed concerns about Sarah. She had recently been admitted to hospital. Mandy's role in the decision making process and her views on how the situation was handled were considered.

"That came about when I went for the results of the annual review. There were changes on the X-ray and Dr Southern sent her for a CT scan which showed that she had inflamed airways and then we decided to send her for a bronch....

Bronchoscopy. ...which we think we still hadn't had the result. But he seemed to think that it all pointed towards her growing pseudomonas. Okay so now she's in and having IVs three times a day and so hopefully I am going to learn how to do those. So we can come home. That's my main worry, he's not well at the moment and so I want to be there for him as well and my husband has got his own business so he has got to – he can't have days off. You know he's trying to do the best he can in between work. So I'm eager to get home and I can do the physio, she's got a new physio regime to do. So she has to do the physio and not just the acapello. And nebuliser and inhaler. So I've got to do all that as well as the IVs. But I'd rather do that and be at home. At least we're there then – we're at home. We're having a good sleep – so that's what I'm hoping. So she can come out on Friday. So that within itself is quite a big challenge.”

In contrast to the previous challenge this challenge was clinically orientated and focussed more on the topic of communication. Emphasis was placed on the interaction between the CF team, specifically the consultant and Mandy as the primary caregiver. This theme is apparent throughout the account. X-rays are performed routinely at the annual assessment unless otherwise specified. So this challenge arose as a consequence of the annual assessment. References to other tests recommended by the consultant were made, however this was followed by a reference to, “we.” The decision for the more intrusive test, the bronchoscopy was made by the consultant and Mandy. This is followed on by the term, “he seemed to think,” which suggests that Mandy is not convinced. Mandy returns to this comment later on in the interview. The dialogue now focuses on the actual implications of the decisions made; the necessity of an admission, the need for intravenous antibiotics, the change in the physiotherapy routine and the addition of further treatment. These are all added pressures for Mandy, “*I've got to do all that.*” And this aspect is described as, “quite a big challenge.” Whilst reference is made to James, her role as primary caregiver and his role as the main breadwinner are implied. Throughout this section repeated references are made to, “home,” coupled with, “hoping.” A positive outcome may be interpreted as being able to go home. Evidence of the need to balance the care of both children is also apparent.

‘treat you like a human being’

“Dr Southern showed me the CT scan and the biopsy as well. He showed me that. I feel as though he does treat you like a human being and tells you exactly how it is you know. This is really important to me – to be involved with the care.”

Previously, Mandy had stated, “But he seemed to think that it all pointed towards her growing pseudomonas,” suggesting that the diagnosis was still unclear in her mind. The dialogue above illustrates how the consultant improved her understanding further involving her in the decision making. The significant phrase here is, “treat you like a human being and tells you exactly how it is.” Caregivers want to be educated and need to be involved. As shown by the results of CLCF-Q they welcome information and feedback thus keeping caregivers informed and involved must be a priority.

“I do see the future positively now that it has been explained to me. And I’ve seen the bronchoscopy and I’ve seen the... when they put the camera down and the secretions what she’s got to get up and the physio regime and and I am confident that we’ll get rid of the pseudomonas and keep her well.”

Involving the caregiver in the decision making process helps empower them. It enables them to gain a locus of control further supporting their confidence. This is the aim of the CLCF-Q in this study.

“I think when you understand a bit more. When everything is explained to you and you understand it in your mind you can deal with things better. It’s the unknown isn’t it that you that you worry about.”

Essentially, a deeper understanding of the situation enables the caregiver to plan ahead when challenges manifest, further improving their coping abilities and SE.

The conclusion will bring together the quantitative results described and the qualitative data from the CLCF-Q, the interview and the vignettes made on feedback sections. Whilst qualitative data is listed in its original form the researcher’s interpretation adds further depth to the understanding.

Chapter Five

Discussion

5.5. Discussion

This study builds on the results of a number of studies exploring the use of the CLCF-Q in clinical practice. The CLCF-Q was initially developed to equate the burden of care on the caregiver. This pilot study aimed to explore the feasibility of integrating the CLCF-Q into the annual assessment process in CF care. The effect on the SE of caregiver's of children with CF was of particular interest. To determine the impact of the PROM on the SE of caregivers, a SE-measure specific to CF care was introduced; the CFSE-Q. Qualitative methods were also applied to support the results and gain an understanding of challenge management; how the caregiver deals with the everyday challenges of living with a child with CF brings to the family¹⁴⁰⁻¹⁴¹. Data collected to date forms part of the information required in this pilot study to allow for power and sample size calculations. The remainder of the pilot study is due for completion in April 2011.

The role of the primary caregiver is complex and multidimensional. It is subject to change in response to the child's disease state and treatment demand^{138 142}. Caregivers face complex challenges on a daily basis and adapt to normalise these situations¹¹¹. Challenges are faced in a particular way and it is important to understand which aspects of management are important to the caregiver. Medical and social supports play major roles in the coping behaviour of caregivers. Interaction with the healthcare team in particular, is welcomed. Caregivers want to be informed and involved in treatment decisions. And their involvement in the decision making process can affect adherence to treatment¹¹⁹. Feedback using the CLCF-Q was welcomed by all caregivers¹⁴³. The feedback not only identified issues concerning the caregiver but also served as an educational tool. The educational needs of caregivers should be considered particularly when considering changes in treatment. *Kettler et al* describes how knowledge of the disease and its management affects adherence to treatment^{144 145}. The process of completing the questionnaire as a standalone intervention, also served as a good *self* feedback tool.

The process of caring for a child with CF is both physically and mentally demanding and can be overwhelming, particularly following diagnosis¹¹¹. The BOC and the responsibility placed on the primary caregiver can cause varying amounts of stress and anxiety and can lead to depression¹¹⁹. This responsibility of caring for a child with CF and the uncertainty of disease progression can affect the outlook of the caregiver and the family unit⁶⁴. Coping behaviours play an important role in the management of challenges and in mediating stress levels¹¹¹. Whilst improving coping behaviour is beyond the scope of hospital intervention,

SE has gained recognition as an important component of coping behaviour¹⁰². SE is sensitive to the introduction of minor interventions and can improve coping abilities¹⁴⁶. Influencing SE from an outpatient setting can be a useful intervention for the caregiver and the family. Studies reveal that coping behaviour is most positively influenced following interventions which enable individuals to understand challenging situations¹¹¹. Interventions should focus on informing participants how to manage stressful situations. Providing feedback on current behaviour and administering a PROM is an ideal example of this. It is a form of both performance accomplishments and verbal persuasion¹⁰².

Data in this study support the administration of the PROM, the CLCF-Q during the annual assessment. It is feasible and of benefit to routinely integrate the CLCF-Q into the annual assessment process. Furthermore, the initial data support the theory that this integration may improve the SE of caregivers. This improvement in SE could lead to an increased QOL.

Baseline SE ranged from 26/40 – 39/40 using only validated items and 36/56 – 53/56 including non-validated items. Average change in SE was -0.1 in the control group and +1.3 in the intervention group. With the inclusion of the non-validated items average increase in SE was +0.8 in the control group and +4.2 in the intervention group. The control group showed consistent improvement in 4 items and decline in 3 items. In comparison the intervention group showed consistent improvement in 8 items and a decline in only 1 item. The CFSE-Q equates changes in personal confidence, efforts employed, confrontation strength and support available. Firm changes in personal confidence, perceived support, efforts employed, problem solving, feeling appreciated and facing challenges were evident in the intervention group.

As suggested, in this study, 94.4% of caregivers were female and 94.4% did not receive support with care⁸³. Ninety percent of caregivers cared for 2 or more children at home. Childhood chronic diseases impact on the wider family including siblings and 30% of families handled the challenges of living with CF with great difficulty. Forty percent of these families admitted to struggling financially¹⁴⁷. Family and friends offer vital support networks to caregivers in addition to the CF team. Nevertheless, most responsibility remains with the primary caregiver and this affects 70% of caregivers greatly. There is a characteristic balance between hopes and worries in caregivers. Short term hopes remains high and unaffected whilst long term worries increase with disease progression.

Feedback data from all caregivers in the intervention group and the interview shows that caregivers uniformly welcomed the study as an intervention to help improve their SE. The qualitative data supported this and established that whilst at home as the primary caregiver

they are expected to uphold the burden, they do also require considerable attention themselves. The role of caring for a child is both emotionally and mentally disconcerting. Caregivers may also have co-morbidities themselves. Whilst they normalise their ADL to include any additional demands when asked to break down their capabilities and self beliefs they are able to do so.

It is important to recognise that the interpretation of what defines a challenge varies greatly both for the caregiver themselves and between caregivers, thus supporting the need for qualitative data in this study. Broad approaches to challenges can be mapped. Important aspects of this approach include family support, CF team support and accessibility, caregiver involvement in management decisions and rapport between the caregiver and the clinician. Support from the CF team is of particular importance and increases caregiver involvement in the decision making process. The current system in place for communication revolves around set clinic appointments. Whilst the telephone service is available around the clock, it relies heavily on the caregiver recognising a weakness and asking for help. The CLCF-Q identifies issues which may not necessarily be recognised as challenges and may otherwise be left unidentified. The annual assessment process is an essential element in monitoring disease states and consequent shaping of management. By improving caregiver-clinician communication the CLCF-Q builds on the results available to inform future decisions. It helps emphasise the importance of, “*we,*” in the decision making process.

CF care has undergone a rapid change whereby the optimum environment of care is now the home. The benefits of being cared for at home are evident and its value to the caregivers is clear¹³⁸. To maintain a good level of care, communication between the family and the CF team must be maintained. This involves three key principles; increasing caregiver involvement, listening to caregivers and giving regular feedback. The CLCF-Q works on all three principles.

The basic design of the study was informed following a 3 month period of shadowing annual assessments and outpatient clinics to ensure the careful and efficient integration of the CLCF-Q. Study phases were timed to match appointment schedules. The study commitments required from participants were limited to waiting periods in appointments and as a consequence all questionnaires and feedback were completed and delivered whilst waiting to see members of the healthcare team. This non-demanding study design contributed to the high response rate. The response rate was initially 100% but later fell to 85.7% due to cancellations in appointments. Non-response bias was deemed low as the drop in uptake was due to cancellation and not the nature of the study.

Despite the successful response rate the total numbers are low due to the restrictions of the study design. Phase 1 of the study centres around the patient's annual assessment appointment, which is individual to the patient. Phase 2 relies on the individuals successive appointments at the outpatient clinic. These are also flexible. Thus the duration and timing of each phase is unique to the patient concerned. The relatively few results of the study at this early stage, though favourable to the hypothesis, are insignificant for statistical analyses. All results have been reported descriptively. Following the collection of the remainder of the data for the pilot phase all results will be graphed to determine distribution type and analysed statistically for both minimal clinical and statistical difference. Power calculations will also be carried out to inform sample size for the larger study.

Descriptively all results except one outlier support the hypothesis showing an increase in SE post intervention. This outlier was only apparent on addition of the non-validated items in the CFSE-Q. The baseline CFSE-Q score was 53/56 and 52/56 post-intervention. The baseline score including validated items only was 39/40 and there was no change in phase 2. This study has illustrated that SE is subject to sensitive change. It is reasonable to assume that individuals who hold particularly high levels of SE benefit little from such interventions. The high baseline SE score in this case may be the cause of the outlier. Further data are required to verify the merit of this outlier.

Limitations

The staggered timeline of the study was shaped around each participants pre-dated appointments schedule. This affected both quantitative and qualitative aspects of the study making it difficult to collate results at any one time point.

Thus an obvious limitation of this dataset is the modest sample size. Despite an 85.7% final recruitment rate the complete dataset only encompasses results from 9 caregivers; though partial datasets are available for 18 caregivers. This thesis does not mark the end of the preliminary data collection, however. The complete set of preliminary data will not only allow sample size predictions for a larger study, it will also inform study design.

Whilst the unique study timeline aided recruitment and compliance it may also adversely affect the results. The time lapse between the intervention and contact with the researcher, and the measurements of endpoint SE was sometimes relatively broad, differing from case to case; the possibility of interviewer effect at the endpoint must therefore be considered. As demonstrated, SE can be sensitive to slight interventions including interactions. To maintain consistency this bias cannot be removed from the study design. Further results are required

to judge the merit of the change in SE. Qualitative data will aid this. If an interviewer effect is evident the method of administering the CLCF-Q may need to be adjusted.

This staggered timeline also relied on the flexibility of the researchers as appointments were often rescheduled, cancelled or postponed at short notice. The drop in final response rate was solely due to appointments being rescheduled. The difficulties of performing such a flexible study are currently outweighed by the benefits of compliance but will require reviewing when the large scale multicentre study is performed.

Though the study yielded high recruitment, due to the lack of commitment required outside of hospital appointments, one participant raised the issue of privacy.

“Completing forms in clinic is fine, one problem is my son curious about the answers. I am very open and honest with him about his treatment. However, I don’t like him to see if I have any worries, as he has enough to deal with. I could tell him not to read what I am filling in, but this would increase his anxieties and I don’t like to tell him to go away.”

Separating the caregiver from their child in a clinic environment is difficult particularly in the waiting areas, which is where the majority of questionnaires were completed. A possible solution would be to allow caregivers protected time outside of clinic time though; this may increase the commitment required, affecting response rate. This was attempted by *Dyer et al* but led to poor recruitment and compliance⁹⁶.

Several other issues have arisen from the pilot phase. Baseline SE varies greatly amongst caregivers. External influences are most effective when baseline SE is low¹⁰². A possible direction in the study could involve introducing a requirement for low baseline SE in the inclusion criteria. As previously mentioned, those with a high baseline SE are most difficult to motivate and show the least improvement following intervention. This study may not help this group of participants at all. Furthermore, the concept of overconfidence must also be considered. High levels of SE can have negative effects on behaviour. The second phase of the study, can last up to 3 months as it relies solely on the child’s appointment schedule. This lengthy period of time may necessitate a greater number of time points between SE measurements to improve understanding of external influences on SE.

Narrative analysis is a slow process making it unsuitable for large amounts of data. This is an indirect limitation on the quantity of qualitative data collectable. Although the criterion for quota sampling only serves as a guide it may further add unnecessary restriction. Reproducibility is also difficult with qualitative methods^{141 148}. Hence, the qualitative data

regardless of its validity in the pilot study may not be reflected in the larger study¹⁴⁸. This shouldn't affect the methodology, however. To increase face value of the data and increase representation the larger study should include a larger qualitative sample.

All of these limitations may have influenced our ability to detect associations between the administration of the CLCF-Q and changes in caregiver SE. They will influence the study design and employment of the larger study.

These findings will add to the growing literature emphasising the role of assessing SE in caregivers. Findings also illustrate a need for assessment and further support for caregivers of children with CF. This is not evident in current research.

Research in the field of CF has transformed significantly from purely quantitative to qualitative and now mixed methodology approaches. Continuity of work in the latter nature is welcomed by caregivers and patients. It substantiates quantitative data.

“I look forward to taking part in the research as all research with regards to cystic fibrosis is extremely welcome! Thank you.”- (taken from participant feedback)

5.9. Conclusion

Despite the relatively small sample size, the results of the study to date have shown that SE can be assessed and influenced. Through careful feedback mechanisms aiming to involve and empower caregivers in the decision making process regarding the treatment of their child, SE can be influenced. The simple intervention employed in this study was integrated into the normal routine of appointments without discord.

Whilst the results of this study may be used to inform future decisions in relation to caregiver-clinician interaction, the ideal result would be for caregivers to utilise self directed mastery themselves. The inclusion of the CLCF-Q into the annual assessment though achievable would only be a stepping stone in this process. SE is subject to sensitive change and must be monitored regularly and maintained. Maintaining SE in all caregivers at the CF centre would require considerable commitment. Ideally, influences on SE must be regulated by the caregivers themselves. Measuring and assessing SE has a place in both research and practice where SE measures could reliably show the effect of the intervention on the participant^{111 146}.

The SE theory needs to be explored further with the family of the child, in particular the siblings. Living with chronic disease can have specific emotional and psychological effects on siblings living in the same household. Paediatric medicine aims for a holistic approach to disease management and this includes the family.

As the child with CF reaches adolescence the role of the primary caregiver diminishes and the burden of care lessens. The burden on the patient however inversely increases as they are expected to take responsibility of their own care. At this transitional stage the SE of the patient is important to understand. Improving SE at this level would be beneficial. *Turner et al* in Australia working under eHealth Services Research Group are currently aiming to do this in a similar manner using information technology as a tool. There is no published data available to date. Promoting self-management and self-efficacy amongst adolescents with CF would be a natural progression from this study¹¹⁶.

References

1. Skipper HE. Improvement of the Model Systems. *Cancer Res* 1969;29(12):2329-33.
2. Carpenter DR, Narsavage GL. One breath at a time: Living with cystic fibrosis. *J Pediatr Nurs* 2004;19(1):25-32.
3. Glasscoe C, Southern KW, Hope H, Burrows E, Heaf L, Brownlee K, et al. Challenge of Living with Cystic Fibrosis (CLCF): psychometric validation. *Journal of Cystic Fibrosis* 2008;7(Supplement 2):S108-S08.
4. Welsh MJ, Smith AE. Cystic fibrosis. *Scientific American* 1995;273(6):52-9.
5. Hodson ME. *Cystic fibrosis*. London: Hodder Arnold, 2007.
6. Davis PB. Cystic Fibrosis Since 1938. *Am. J. Respir. Crit. Care Med.* 2006;173(5):475-82.
7. Dicke W, Weijers H, van de Kamer J. Coeliac disease: the presence in wheat of a factor having a deleterious effect in cases of coeliac disease. *Acta Paediatric Scand* 1953;42:344-99.
8. Gee S. On the coeliac affection. *St Bartholomew's Hospital* 1888;24:17-20.
9. Harper M. Two cases of congenital pancreatic steatorrhoea with infantilism. *Med J Aust* 1930;2:663.
10. Harper M. Congenital steatorrhoea due to pancreatic defect. *Arch Dis Child* 1938;13:45-56.
11. Sommer A, West KP. *Vitamin A deficiency : health, survival, and vision*. New York: Oxford University Press, 1996.
12. Andersen DH. Cystic fibrosis of the pancreas and its relation to celiac disease: a clinical and pathologic study. *Am J Dis Child* 1938;56(2):344-99.
13. Farber S. Pancreatic insufficiency and the celiac syndrome. *NEJM* 1943;229:653-357.
14. Di Sant'Agnese PA, Darling R, Perera G, Shea A. Abnormal electrolyte composition of sweat in cystic fibrosis of the pancreas. Clinical significance and relationship to the disease. *Pediatrics* 1953;12:549-63.
15. Quinton P. Chloride impermeability in cystic fibrosis. *Nature* 1983;301:421-22.
16. Knowles M, Strutts M, Spock A, Fischer N, Gatzky J, Boucher R. Abnormal ion permeation through cystic fibrosis respiratory epithelium. *Science* 1983;221(1067-1070).
17. Boucher R, Stutts M, Knowles M, Cantley L, Gatzky J. Na⁺ transport in cystic fibrosis respiratory epithelia: abnormal basal rate and response to adenylate cyclase activation,. *J Clin Invest* 1986;78:1245-52.
18. Rommens J, Iannuzzi M, Kerem B-S, al e. Identification of the cystic fibrosis gene: chromosome walking and jumping. *Science* 1989;245:1059-65.
19. Riordan J, Rommens J, Kerem B-S, al e. Identification of the cystic fibrosis gene: cloning and characterisation of the complimentary DNA. *Science* 1989;245:1066-73.

20. Kerem B-S, Rommens J, Buchanan J, et al. Identification of the cystic fibrosis gene: genetic analysis. *Science* 1989;245:1073-80.
21. Romeo G, Devoto M, LJV G. Why is the cystic fibrosis gene so frequent. *Hum Genet* 1989;84:1-5.
22. Davies JC, Alton EFWF, Bush A. Cystic fibrosis. *BMJ* 2007;335(7632):1255-59.
23. Rosenstein BJ, Cutting G. The diagnosis of cystic fibrosis: A consensus statement. *J Pediatr* 1998;132(4):589-95.
24. Sims EJ, McCormick J, Mehta G, Mehta A. Neonatal screening for cystic fibrosis is beneficial even in the context of modern treatment. *J Pediatr* 2005;147(3 Suppl):S42-6.
25. Peebles A, Connett G, Maddison J, Gavin J. *Cystic Fibrosis Care: a Practical Guide*. London: Elsevier (Churchill Livingstone), 2005.
26. Sturgess J, Imrie J. Quantitative evaluation of the development of tracheal submucosal glands in infants with cystic fibrosis and control infants. *Am J Pathol* 1982;106(3):303-11.
27. Smyth R. Diagnosis and management of cystic fibrosis. *Arch Dis Child Educ Pract Ed* 2005;90:ep1-ep6.
28. Fleming A. The discovery of penicillin. *Br Med Bull* 1944;2(1):4-5.
29. Southern KW, Barker PM, Solis A. Macrolide antibiotics for cystic fibrosis. *Cochrane Database Syst Rev* 2004(2):CD002203.
30. Dushenko TW. Cystic fibrosis: a medical overview and critique of the psychological literature. *Soc Sci Med E* 1981;15(1):43-56.
31. Venters M. Familial coping with chronic and severe childhood illness: the case of cystic fibrosis. *Soc Sci Med A* 1981;15(3 Pt 1):289-97.
32. de Jong W, Kaptein AA, van der Schans CP, Mannes GP, van Aalderen WM, Grevink RG, et al. Quality of life in patients with cystic fibrosis. *Pediatr Pulmonol* 1997;23(2):95-100.
33. Abbott J. Health-related quality of life measurement in cystic fibrosis: advances and limitations. *Chron Respir Dis* 2009;6(1):31-41.
34. FitzSimmons SC. The changing epidemiology of cystic fibrosis. *The Journal of Pediatrics* 1993;122(1):1-9.
35. Griesenbach U, Geddes DM, Alton EFWF. Advances in cystic fibrosis gene therapy. *Curr Opin Pulm Med* 2004;10:542-46.
36. Blau H, Livne M, Mussaffi H. Cystic fibrosis in adults: a changing scene. *Isr Med Assoc J* 2003;5(7):491-5.
37. Orenstein DM, Nixon PA, Ross EA, Kaplan RM. The quality of well-being in cystic fibrosis. *Chest* 1989;95(2):344-47.
38. Dodge JA, Lewis PA, Stanton M, Wilsher J. Cystic fibrosis mortality and survival in the UK: 1947-2003. *Eur Respir J* 2007;29(3):522-6.

39. Dodge JA, Morison S, Lewis PA, Coles EC, Geddes D, Russell G, et al. Incidence, population, and survival of cystic fibrosis in the UK, 1968-95. UK Cystic Fibrosis Survey Management Committee. *Arch Dis Child* 1997;77(6):493-6.
40. Elborn JS, Shale DJ, Britton JR. Cystic Fibrosis: current survival and population estimates to the year 2000. *Thorax* 1991;46:881-85.
41. Stern R. The diagnosis of cystic fibrosis. *NEJM* 1997;336(7):487-91.
42. Zielenski J. Genotype and phenotype in cystic fibrosis. *Respiration* 2000;67(2):117-33.
43. Quittner AL, Schechter MS, Rasouliyan L, Haselkorn T, Pasta DJ, Wagener JS. Impact of socioeconomic status, race, and ethnicity on quality of life in patients with cystic fibrosis in the United States. *Chest* 2010;137(3):642-50.
44. Southern KW. Newborn screening for cystic fibrosis: the practical implications. *J R Soc Med* 2004;97 Suppl 44:57-9.
45. Bobadilla JL, Macek M, Jr., Fine JP, Farrell PM. Cystic fibrosis: a worldwide analysis of CFTR mutations--correlation with incidence data and application to screening. *Hum Mutat* 2002;19(6):575-606.
46. Sermet-Gaudelus I, Mayell SJ, Southern KW. Guidelines on the early management of infants diagnosed with cystic fibrosis following newborn screening. *J Cyst Fibros* 2010.
47. LeGrys VA, Yankaskas JR, Quittell LM, Marshall BC, Mogayzel PJ, Jr. Diagnostic sweat testing: the Cystic Fibrosis Foundation guidelines. *J Pediatr* 2007;151(1):85-9.
48. Parad RB, Comeau AM, Dorkin HL, Dovey M, Gerstle R, Martin T, et al. Sweat testing infants detected by cystic fibrosis newborn screening. *J Pediatr* 2005;147(3 Suppl):S69-72.
49. Hedgecoe A. Expansion and uncertainty: Cystic fibrosis, classification and genetics. *Soc Heath Illness* 2003;25(1):50-70.
50. Tsui LC. The spectrum of cystic fibrosis mutations. *Trends Genet* 1992;8(11):392-8.
51. Mandrusiak A, MacDonald J, Watter P. The International Classification of Functioning, Disability and Health: an effective model for describing young people with cystic fibrosis. *Child Care Health Dev* 2009;35(1):2-4.
52. Penketh A, Wise A, Mearns M, Hodson M, Batten J. Cystic fibrosis in adolescents and adults. *Thorax* 1987;42:526-32.
53. Boucher RC. An overview of the pathogenesis of cystic fibrosis lung disease. *Advanced Drug Delivery Reviews* 2002;54:1359-71.
54. Gee L, Abbott J, Hart A, Conway SP, Etherington C, Webb AK. Associations between clinical variables and quality of life in adults with cystic fibrosis. *J Cyst Fibros* 2005;4(1):59-66.
55. Wine J. The genesis of cystic fibrosis lung disease. *J Clin Invest* 1999;103(3):309-13.
56. Taylor CJ, Connolly S. Hepatobiliary disease in cystic fibrosis. *Paediatrics and Child Health* 2010;20(1):20-25.

57. Ievers CE, Drotar D. Family and parental functioning in cystic fibrosis. *J Dev Behav Pediatr* 1996;17(1):48-55.
58. Davis PB, Sant'Agnese PAD. A review. Cystic Fibrosis at forty - Quo Vadis? *Pediat Res* 1980;14:83-87.
59. Stevens D, Oades PJ, Armstrong N, Williams CA. A survey of exercise testing and training in UK cystic fibrosis clinics. *Journal of Cystic Fibrosis*;In Press, Corrected Proof.
60. Tipping CJ, Scholes RL, Cox NS. A qualitative study of physiotherapy education for parents of toddlers with cystic fibrosis. *Journal of Cystic Fibrosis* 2010;9(3):205-11.
61. Wicks E. Cystic fibrosis. *BMJ* 2007;334(7606):1270-71.
62. Ziaian T, Sawyer M, Reynolds K, Carbone J, Clark J, Baghurst P, et al. Treatment burden and health-related quality of life of children with diabetes, cystic fibrosis and asthma. *J Paediatr Child Health* 2006;42:596-600.
63. Guyatt G, Sackett D, Cook D. Users guide to the medical literature II: How to use an article about therapy on prevention. *JAMA* 1993;270:2598-607.
64. McClellan CB, Cohen LL. Family functioning in children with chronic illness compared with healthy controls: a critical review. *J Pediatr* 2007;150(3):221-3, 23 e1-2.
65. Quittner AL. Measurement of quality of life in cystic fibrosis. *Curr Opin Pulm Med* 1998;4(6):326-31.
66. Goldbeck L, Zerrer S, Schmitz TG. Monitoring quality of life in outpatients with cystic fibrosis: feasibility and longitudinal results. *J Cyst Fibros* 2007;6(3):171-8.
67. Quittner AL, Modi A, Cruz I. Systematic review of health-related quality of life measures for children with respiratory conditions. *Paediatr Respir Rev* 2008;9(3):220-32.
68. Havermans T, Colpaert K, Dupont L. Quality of life in patients with cystic fibrosis: Association with anxiety and depression. *J Cyst Fibros* 2008;7:581-84.
69. Kosciak RL, Douglas JA, Zaremba K, Rock MJ, Splaingard ML, Laxova A, et al. Quality of life of children with cystic fibrosis. *J Pediatr* 2005;147(3 Suppl):S64-8.
70. Tullis DE, Guyatt GH. Quality of life in cystic fibrosis. *Pharmacoeconomics* 1995;8(1):23-33.
71. Abbott J, Hart A. Measuring and reporting quality of life outcomes in clinical trials in cystic fibrosis: a critical review. *Health Qual Life Outcomes* 2005;3:19.
72. Quittner AL, Modi AC, Wainwright C, Otto K, Kirihaara J, Montgomery AB. Determination of the minimal clinically important difference scores for the Cystic Fibrosis Questionnaire-Revised respiratory symptom scale in two populations of patients with cystic fibrosis and chronic Pseudomonas aeruginosa airway infection. *Chest* 2009;135(6):1610-8.
73. Palermo TM, Harrison D, Koh JL. Effect of disease-related pain on the health-related quality of life of children and adolescents with cystic fibrosis. *Clin J Pain* 2006;22(6):532-7.
74. Modi AC, Quittner AL. Validation of a disease-specific measure of health-related quality of life for children with cystic fibrosis. *J Pediatr Psychol* 2003;28(8):535-45.

75. Rozov T, Cunha MT, Nascimento O, Quittner AL, Jardim JR. Linguistic validation of cystic fibrosis quality of life questionnaires. *J Pediatr (Rio J)* 2006;82(2):151-6.
76. Britto MT, Kotagal UR, Chenier T, Tsevat J, Atherton HD, Wilmott RW. Differences between adolescents' and parents' reports of health-related quality of life in cystic fibrosis. *Pediatr Pulmonol* 2004;37(2):165-71.
77. Abbott J, Hart A, Morton AM, Dey P, Conway SP, Webb AK. Can health-related quality of life predict survival in adults with cystic fibrosis? *Am J Respir Crit Care Med* 2009;179(1):54-8.
78. Abbott J, Hart A, Morton A, Gee L, Conway S. Health-related quality of life in adults with cystic fibrosis: the role of coping. *J Psychosom Res* 2008;64(2):149-57.
79. Szyndler JE, Towns SJ, van Asperen PP, McKay KO. Psychological and family functioning and quality of life in adolescents with cystic fibrosis. *J Cyst Fibros* 2005;4(2):135-44.
80. Wahl AK, Rustoen T, Hanestad BR, Gjengedal E, Moum T. Living with cystic fibrosis: impact on global quality of life. *Heart Lung* 2005;34(5):324-31.
81. Quittner AL, Buu A, Messer MA, Modi AC, Watrous M. Development and validation of The Cystic Fibrosis Questionnaire in the United States: a health-related quality-of-life measure for cystic fibrosis. *Chest* 2005;128(4):2347-54.
82. Bregnballe V, Thastum M, Schiøtz P. Psychosocial problems in children with cystic fibrosis. *Acta Paediatrica* 2007;96:58-61.
83. Boling W, Macrina DM, Clancy JP. The Caregiver Quality of Life Cystic Fibrosis (CQOLCF) scale: modification and validation of an instrument to measure quality of life in cystic fibrosis family caregivers. *Qual Life Res* 2003;12(8):1119-26.
84. Abbott J, Baumann U, Conways S, Etherington C, Gee L, Von Der Schulenburg J, et al. Cross cultural differences in health related quality of life in adolescents with cystic fibrosis. *Disab Rehab* 2001;23(18):837-44.
85. Goss CH, Quittner AL. Patient-reported outcomes in cystic fibrosis. *Proc Am Thorac Soc* 2007;4(4):378-86.
86. Quittner AL, Barker DH, Snell C, Grimley ME, Marciel K, Cruz I. Prevalence and impact of depression in cystic fibrosis. *Curr Opin Pulm Med* 2008;14(6):582-8.
87. Gee L, Abbott J, Conway SP, Etherington C, Webb AK. Quality of life in cystic fibrosis: the impact of gender, general health perceptions and disease severity. *J Cyst Fibros* 2003;2(4):206-13.
88. Britto MT, Kotagal UR, Hornung RW, Atherton HD, Tsevat J, Wilmott RW. Impact of recent pulmonary exacerbations on quality of life in patients with cystic fibrosis. *Chest* 2002;121(1):64-72.
89. Hegarty M, Macdonald J, Watter P, Wilson C. Quality of life in young people with cystic fibrosis: effects of hospitalization, age and gender, and differences in parent/child perceptions. *Child Care Health Dev* 2009;35(4):462-8.

90. Havermans T, Vreys M, Proesmans M, De Boeck C. Assessment of agreement between parents and children on health-related quality of life in children with cystic fibrosis. *Child Care Health Dev* 2006;32(1):1-7.
91. French DJ. Quality of life in cystic fibrosis. *Thorax* 1998;53(9):721-2.
92. Henry B, Aussage P, Grosskopf C, Goehrs JM. Development of the Cystic Fibrosis Questionnaire (CFQ) for assessing quality of life in pediatric and adult patients. *Qual Life Res* 2003;12(1):63-76.
93. Modi AC, Lim CS, Yu N, Geller D, Wagner MH, Quittner AL. A multi-method assessment of treatment adherence for children with cystic fibrosis. *Journal of Cystic Fibrosis* 2006;5(3):177-85.
94. Glasscoe C, Quittner AL, Smith JA, Burrows EF, Heaf L, Cottrell J, et al. 312 Living with Cystic Fibrosis Questionnaire (CFLC-Q): Development and preliminary validation of a new measure of the challenge experienced by caregivers. *Journal of Cystic Fibrosis* 2007;6(Supplement 1):S76-S76.
95. Glasscoe C, Southern KW, Lancaster GA, Quittner AL, Burrows EF, Heaf LJ, et al. Estimating risk associated with home-based intravenous antibiotic therapy (IVAT). *Journal of Cystic Fibrosis* 2009;8(Supplement 2):S95-S95.
96. Dyer KL, Glasscoe C, Hope H, Patel L, Southern KW. The impact of pulmonary exacerbation on the challenge of living with cystic fibrosis. *Journal of Cystic Fibrosis* 2010;9(Supplement 1):S100-S00.
97. Glasscoe CA, Quittner AL. Psychological interventions for people with cystic fibrosis and their families. *Cochrane Database Syst Rev* 2008(3):CD003148.
98. Schunk DH. Effects of effort attributional feedback on children's perceived self-efficacy and achievement. *Journal of Educational Psychology* 1982;74(4):548-56.
99. Glasscoe C, Quittner AL, Patel L, Dale C, Burrows EF, Hope HF, et al. Components of risk associated with home intravenous antibiotic therapy (H-IVAT) in the UK. *Journal of Cystic Fibrosis* 2010;9(Supplement 1):S105-S05.
100. Bandura A, Adams N. Analysis of self-efficacy theory of behavioral change. *Cog Theo Res* 1977;1(4):287-310.
101. Bandura A. Self-efficacy: toward a unifying theory of behavioral change. *Psychol Rev* 1977;84(2):191-215.
102. Bandura A. Self-efficacy and health behaviour. In: Baum A, editor. *Cambridge handbook of psychology, health, and medicine* Cambridge: Cambridge University Press, 1997:160-62.
103. Bandura A. *Psychological modelling: Conflictng theories*. Chicago: Aldine-Atherton, 1971.
104. Bandura A, Locke EA. Negative self-efficacy and goal effects revisited. *J Appl Psychol* 2003;88(1):87-99.
105. Hendrix CC, Abernethy A, Sloane R, Misuraca J, Moore J. A pilot study on the influence of an individualized and experiential training on cancer caregiver's self-efficacy in home care and symptom management. *Home Healthc Nurse* 2009;27(5):271-8.

106. Rescorla RS, RL. Two-processes learning theory: Relationships between Pavlovian conditioning and instrumental learning. *Psychol Rev* 1967;74:151-82.
107. Zimmerman BJ. Self-Efficacy: An Essential Motive to Learn. *Contemp Educ Psychol* 2000;25(1):82-91.
108. Steffen AM, McKibbin C, Zeiss AM, Gallagher-Thompson D, Bandura A. The revised scale for caregiving self-efficacy: reliability and validity studies. *J Gerontol B Psychol Sci Soc Sci* 2002;57(1):P74-86.
109. Weiner B. *Theories of motivation*. Chicago: Markham, 1972.
110. Eiser C. Helping the child with chronic disease: Themes and directions. *Clin Child Psychol Psychiatry* 1996;1:551-61.
111. Abbott J. Coping with cystic fibrosis. *J R Soc Med* 2003;96(43):42-50.
112. Wolpe J. *The practice of behaviour therapy*. New York: Pergamon, 1974.
113. Gignac M, Gottlieb B. Caregivers' appraisals of efficacy in coping with dementia. *Psychol Aging* 1996;11:214-25.
114. Schulz R, Visintainer PW, GM. Psychiatric and physical morbidity effects of caregiving. *J Gerontol: Psychol Sci* 1990;45:181-91.
115. Zeiss AM, Gallagher-Thompson D, Lovett S, Rose J, McKibbin C. Self-efficacy as a mediator of caregiving coping: Development and testing of an assessment model. *J Clin Geropsych* 1999;5:221-30.
116. Bartholomew LK, Parcel GS, Swank PR, Czyzewski DI. Measuring self-efficacy expectations for the self-management of cystic fibrosis. *Chest* 1993;103(5):1524-30.
117. Vancouver JB, Putka DJ. Analyzing Goal-Striving Processes and a Test of the Generalizability of Perceptual Control Theory. *Organ Behav Hum Decis Process* 2000;82(2):334-62.
118. Dulany D. Awareness, rules, and propositional control: A confrontation with S-R behavior theory. In: Dixon TH, DL., editor. *Verbal behavior and general behavior theory*,. Englewood Cliffs, NJ: Prentice-Hall, 1968.
119. Driscoll KA, Montag-Leifling K, Acton JD, Modi AC. Relations between depressive and anxious symptoms and quality of life in caregivers of children with cystic fibrosis. *Pediatr Pulmonol* 2009;44(8):784-92.
120. Williams HL, Cullen LA, Barlow JH. The psychological well-being and self-efficacy of carers of children with disabilities following attendance on a simple massage training and support programme: a 12-month comparison study of adherers and non-adherers. *Complement Ther Med* 2005;13(2):107-14.
121. Bandura A. *Self-efficacy: The exercise of control* New York: Freeman, 1997.
122. Schwarzer R, Jerusalem M. Generalized self-efficacy scale. In: Weinman J, Wright S, Johnston M, editors. *Measures in health psychology: A user's portfolio. Causal and control beliefs*. Windsor: UK: Nfer-Nelson, 1995:35-57.

123. Schwarzer R, Renner B. Social-cognitive predictors of health behavior: action self-efficacy and coping self-efficacy. *Health Psychol* 2000;19(5):487-95.
124. Scholz U, Doñab B, Sud S, Schwarzer R. Is General Self-Efficacy a Universal Construct? Psychometric Findings from 25 Countries. *Eur J Psychological Assess* 2002;18(3):242-51.
125. Averill J. Personal control over aversive stimuli and its relationship to stress. *Psychol Bul* 1973;80:286-303.
126. Williams S. Perceived self-efficacy and phobic disability. In: Schwarzer R, editor. *Self-efficacy: Though control of action*. Washington, DC: Hemisphere Publication Services, 1992:149-76.
127. Bandura A. *Social learning theory*. Englewood Cliffs, NJ.: Prentice-Hall, 1977.
128. Bandura A. *Psychological modelling: Conflicting theories*. Chicago: Aldine-Atherton, 1971.
129. Grings W. The role of consciousness and cognition in autonomic behavior change. In: McGuigan F, Schoonover R, editors. *The psychophysiology of thinking*. New York: Academic Press, 1973.
130. Aneshensel C, Pearlin L, Mullan J, Zarit S, Whitlatch C. *Profiles in caregiving: The unexpected career*. San Diego, CA: Academic Press, 1995.
131. Thomas J, Shuford D, Duke J, Cipriani D. Development and Psychometric Properties of the Self-Efficacy Scale for Transfers for Caregivers of Children Requiring Transfer Assistance. *Arch Phys Med Rehabil* 2007;88:481-88.
132. Schulz R, Quittner AL. Caregiving for children and adults with chronic conditions: introduction to the special issue. *Health Psychol* 1998;17(2):107-11.
133. Porter L, Keefe F, Scipio C, Garst J, Peterson B. (870): Patient, caregiver, and dyad self-efficacy for managing lung cancer symptoms: Associations with symptoms and psychological distress. *The Journal of Pain* 2006;7(4, Supplement 1):S68-S68.
134. Porter LS, Keefe FJ, McBride CM, Pollak K, Fish L, Garst J. Perceptions of patients' self-efficacy for managing pain and lung cancer symptoms: correspondence between patients and family caregivers. *Pain* 2002;98(1-2):169-78.
135. Porter LS, Keefe FJ, Garst J, McBride CM, Baucom D. Self-efficacy for managing pain, symptoms, and function in patients with lung cancer and their informal caregivers: Associations with symptoms and distress. *Pain* 2008;137(2):306-15.
136. Keefe FJ, Ahles TA, Porter LS, Sutton LM, McBride CM, Pope MS, et al. The self-efficacy of family caregivers for helping cancer patients manage pain at end-of-life. *Pain* 2003;103(1-2):157-62.
137. Estes W. Reinforcement in human behavior. *Am Sc* 1972;60:723-29.
138. Boling W. The health of chronically ill children: lessons learned from assessing family caregiver quality of life. *Fam Community Health* 2005;28(2):176-83.
139. Greenhalgh T, Hurwitz B. Narrative based medicine: why study narrative? *BMJ* 1999;318(7175):48-50.

140. Pope C, van Royen P, Baker R. Qualitative methods in research on healthcare quality. *Qual Saf Health Care* 2002;11(2):148-52.
141. Sofaer S. Qualitative research methods. *Int J Qual Health Care* 2002;14(4):329-36.
142. Makizako H, Abe T, Shimada H, Ohnuma T, Furuna T, Nakamura Y. Combined effect of factors associated with burdens on primary caregiver. *Geriatr Gerontol Int* 2009;9(2):183-9.
143. Goldbeck L, Babka C. Development and evaluation of a multi-family psychoeducational program for cystic fibrosis. *Patient Educ Couns* 2001;44(2):187-92.
144. Cruz I, Marciel KK, Quittner AL, Schechter MS. Anxiety and depression in cystic fibrosis. *Semin Respir Crit Care Med* 2009;30(5):569-78.
145. Kettler LJ, Sawyer SM, Winefield HR, Greville HW. Determinants of adherence in adults with cystic fibrosis. *Thorax* 2002;57(5):459-64.
146. Mitchell AE, Fraser JA. Parents' self-efficacy, outcome expectations, and self-reported task performance when managing atopic dermatitis in children: Instrument reliability and validity. *Int J Nurs Stud* 2010.
147. Havermans T, Colpaert K, Vanharen L, Dupont LJ. Health related quality of life in cystic fibrosis: To work or not to work? *J Cyst Fibros* 2009;8(3):218-23.
148. Mattingly C. In search of the good: narrative reasoning in clinical practice. *Med Anthropol Q* 1998;12(3):273-97.

Appendix

7.1. Study Information

7.1.1. Protocol

Protocol Title

Does administering a parent reported outcome measure during the annual review improve the self efficacy of the carer of a child who has cystic fibrosis?

Protocol Version

Version 7

Last Amended

27th January 2009

Principal Investigator

Dr Kevin Southern

Co-investigators

Miss Latifa Patel
Dr Claire Glasscoe
Dr Clare Dixon

Study siteAddress

Institute of Child Health
University of Liverpool
Alder Hey Children's Hospital
Eaton Road
West Derby
Liverpool

Post Code

L12 2AP

Contact

Email - kwsouth@liverpool.ac.uk
Telephone - 0151 228 4811 Ext. 3536
Mobile - 07515 519 574
Fax - 0151 228 0328

Duration

Proposed Start Date - 1 March 2010
Proposed End Date - 1 June 2010

Abstract

Background

Cystic Fibrosis (CF) is the most prevalent inherited, life shortening disorder in the Caucasian populations. It is estimated that there are over 10 million carriers of the gene worldwide and 1 in 2000-3000 live births are affected in the normal population.

As a result of recent advances in medical care the median age of survival has risen from 2 years of age in 1938 to over 40 years of age. Consequently, time and resources have been allocated to ensure a good standard of life and over the past decade numerous tools have been proposed to measure Quality of Life (QoL).

Working with parents a Parent Reported Outcome (PRO) measure called the "Challenge of Living with CF (CLCF)," was developed. Its aim was to measure the burden of care on everyday living for parents/carers of patients with CF.

This study will explore the use of this measure in a clinical setting; namely at the CF annual review. In particular the study will look at whether the use of this tool increases how able the parents/carers feel they are to manage the everyday challenges they face; this is termed their Perceived Self Efficacy (PSE).

Aims

We aim to explore the feasibility of and the effect of using the CLCF measure during the annual review. The adapted self-efficacy scale specific to CF will be used to analyse and evaluate the results.

Design

This study incorporates both qualitative and quantitative research methods.

In each carer/child dyad the main carer will be invited to participate in the study. It is usually assumed that this will be the mother in the average family however the parents/carers will be asked to decide who is most suitable amongst themselves.

Southern has developed an adapted version of the General Self Efficacy Questionnaire. The CF Self Efficacy-Questionnaire (CFSE-Q) has specific references to the tasks involved in the everyday care of a child with CF.

On a randomised basis participants will be allocated to two groups; control and intervention group. Both groups will fill in a CFSE-Q both at the start and at the end of the study. The intervention group however, will be asked to complete the CLCF-Questionnaire (CLCF-Q) during their annual review appointment and will be given feedback on the outcomes of the CLCF-Q accordingly.

Changes in SE will be noted, if any and statistically analysed.

Qualitative analysis will be performed out on interviews carried out with a select number of patients (n=4).

Background and Significance/Preliminary Studies

CF is the most prevalent autosomally recessive inherited, life shortening disorder in the Caucasian populations. It is estimated that there are over 10 million carriers of the gene worldwide and 1 in 2000-3000 live births are affected in the normal population.

CF affects the respiratory, digestive, liver, pancreas and reproductive systems resulting in the following complications; bronchiectasis, chronic sinusitis, nasal polyposis, pancreatic insufficiency, cirrhosis, bowel obstruction and male infertility.

As a result of recent advances in medical care the median age of survival has risen from 2 years of age in 1938 to over 40 years of age. However the daily life prolonging treatment involved is expensive, intensive and time consuming requiring multi-disciplinary team involvement. This can be a great burden on all parties involved.

Paediatric medicine aims to improve the patient and their carer's global quality of life where possible alongside stopping or slowing down disease progression. Hence measuring the impact of the chronic illness on these patients is becoming increasingly important. Three decades ago the World Health Organisation (WHO) released a definition for health,

"a state of complete physical and social well being, not just the absence of disease."

Consequently, time and resources have been allocated to ensure a good standard of life is achieved and over the past decade numerous tools have been proposed to QoL.

Measuring QoL is a method of subjectively evaluating the impact of chronic disease on a patient's activities of daily living and global well being. QoL is, *"a dynamic psychological construct, which describes the subjective health perceptions of the patients independently of objective health parameters,"* and it fluctuates over time and disease progression amongst other factors.

Over the past 25 years PRO measures have become increasingly important and greatly influences treatment. A PRO is,

"A measure of a patient's health status, elicited directly from the patient, that assesses how the patient feels and functions with respect to his or her health condition."

It ranges from a single symptom rating to a multi-system analyses looking into the global Health Related QoL (HRQoL) of the patient. HRQoL measurement in CF allows the inclusion of a patient's perspective elicited directly from the patient.

HRQoL is a multidimensional construct comprised of the domains of physical functioning, social functioning and emotional and psychological functioning. Together they give an overall picture of the disease state incorporating otherwise untested for states such as anxiety.

Although previous studies have almost perfected the use of PRO measures and HRQoL measurements, the exact use of this information and direct impact on the patient and their parent/carer has had relatively fewer investments. Whether these interventions have had any improvement on the patient's further treatment or life in general is unknown.

Working with parents a Parent Reported Outcome (PRO) measure called the "Challenge of Living with CF (CLCF)," was developed. Its aim was to measure the burden of care on everyday living for parents/carers of patients with CF.

This study will explore the use of this measure in a clinical setting; namely at the CF annual review. In particular the study will look at whether the use of this tool increases how able the parents/carers feel they are to manage the everyday challenges they face; this is termed their Perceived Self Efficacy (PSE).

PSE is

"the expectation of personal success in a given situation."

The concept of measuring general self efficacy first came to light in 1979 in Germany. Matthias Jerusalem and later Professor Ralf Schwarzer discussed how PSE reflected on optimistic self belief and is required to facilitate goal setting and persistence in everyday activities.

Human behaviour is generally developed through careful modelling techniques. Initially one may observe and follow others or follow instructions. Later one will learn from others or ones own mistakes allowing for self corrective adjustments until the technique is refined to a suitable standard.

Without PSE or with a low PSE the task is seen as too great a challenge and can sometimes be avoided or performed with no intent to succeed; hence poorly.

Four main sources of information which alter PSE are known; performance accomplishments, physiological states, vicarious experience and verbal persuasion.

Using this model the PSE of parents/carers may be increased by incorporating the CLCF-Questionnaire (CLCF-Q) into the annual review process.

If the PSE is increased it has been shown in studies that this increase could directly correlate with an improved performance and success rate. Thus parents/carers are more likely to take on challenging tasks with a belief of succeeding. They are also more likely to confidently make decisions about the treatment their child receives. This possible increase in PSE may also correlate with their perception of control, overall improving the patient and their parents/carers global QoL.

Study Aims

Aims of Research

1. To explore the feasibility of using the CLCF measure in a clinical setting
2. To adapt the established perceived self-efficacy scale to be specific to CF parents
3. To investigate the impact of using a PRO on PSE.
4. To conduct a qualitative investigation of parents/carers accounts of their experience at annual review with semi-structured interviews.
5. To conduct a pilot study that will allow for the power and sample size calculations for a larger multi centre randomized controlled trial to be carried out.

Objectives

1. Does administering a parent reported outcome measure during the annual review improve the self efficacy of the carer of a child who has cystic fibrosis?
2. Is it feasible to introduce the recently validated CLCF measure into the annual review and routine CF Clinics at Alder Hey Children's Hospital?

Hypothesis

"I hypothesise that incorporating the CLCF questionnaire into the annual review process will have a positive influence on the PSE of parents/carers, thus resulting in an improved global quality of life."

Administrative Organization

This study is a single centre study and will be carried out at the respiratory unit in Alder Hey Children's Hospital (Alder Hey Children's NHS Foundation Trust).

The data management centre is also located on site in a University of Liverpool building.

Study Design

Experimental design of the study

This is a randomised non-blinded study which incorporates both qualitative and quantitative research methods.

Study population general description

Participants will consist of both genders with no restriction on age range and varying backgrounds. They will be selected on the basis of the inclusion criteria.

In each carer/child dyad the main carer will be invited to participate in the study. It is usually assumed that this will be the mother in the average family however the parents/carers will be asked to decide who is most suitable amongst themselves.

Sample size determination and power analyses

As the study is a pilot study to be carried out at a single centre the number of patients available determined the sample size. Thus all prospective carers of all patients who are currently registered and fulfil the inclusion criteria will be invited to join the study.

Data from this pilot study will be used to calculate the sample size and power calculations for a subsequent larger study.

Study outcomes/endpoints

Southern has developed an adapted version of the General Self Efficacy Questionnaire. The CF Self Efficacy-Questionnaire (CFSE-Q) specifically details the tasks involved in the everyday care of a child with CF.

The study will evaluate and analyse the change in the score of the CFSE-Q between the start and the end of the study.

The study will conclude when all participants have second the last CFSE-Q and selected participants have been interviewed.

Study Procedures

Subject selection procedures

Principal Inclusion Criteria

The principal inclusion criterion is;

- 1) The carer must have a child diagnosed with cystic fibrosis.
- 2) The diagnosis of cystic fibrosis must have been made at least one year ago.
- 3) The child must be registered as a patient at Alder Hey Children's Hospital.
- 4) The child must be between 1 and 13 years of age.
- 5) The parent/carer must have a reasonable understanding of the study and what is involved and be competent enough to refuse participation.

Principal Exclusion Criteria

The principal exclusion criterion is;

- 1) Carers or children with physical or mental disabilities which prohibit them from participating in the study successfully.

Recruitment procedures

The recruitment will be headed by Patel.

Prospective participants will be identified from the hospital database and will be matched according to the inclusion and exclusion criterion.

Recruitment will commence when the ethical approval is finalised and will involve formally inviting carers to participate in the study. On the penultimate outpatient appointment at the CF clinic before their annual review carers will be informed of the study and invited to participate. They will be given a patient information sheet to take home and study at their own convenience.

At the annual review appointment carers will be reminded of the study and be given another opportunity to study the patient information sheet as well as make any concerns and queries known. Following this fully informed voluntary consent will be sought on a written document by Patel.

There will be no public advertisement of the study.

Screening procedures

Apart from the inclusion and exclusion criterion this study will not involve any other screening.

Randomization procedures

On a randomised basis participants will be allocated to two groups; a control and an intervention group. The participants will be randomised immediately following consent. The sealed envelope process will be employed and to maintain objectivity they will be allocated and signed for from an independent party.

Study Intervention

Both groups will fill in a CFSE-Q both at the start and at the end of the study. The intervention group however, will be asked to complete the CLCF-Q during their annual review appointment. This will involve them completing a 10 page document consisting of 10 domains and 60 items. The CLCF-Q is accompanied with a feedback process involving the formal feedback of all results at a subsequent outpatient appointment where help will also be offered by the CF team on issues raised in the CLCF-Q. All participants will receive a letter outlining the results of the annual review. However for participants in the intervention group this will also include a summary of the results of the CLCF-Q.

Study Assessments and Activities

Non-Clinical Interventions	Routine	Duration	Researcher
Seeking consent	NO	30 minutes	Miss Latifa Patel
Self Efficacy Questionnaire (1 st)	NO	10 minutes	Miss Latifa Patel
Challenges of Living with Cystic Fibrosis Questionnaire	NO (Group A Participants)	30 minutes	Miss Latifa Patel
Self Efficacy Questionnaire (2 nd)	NO	10 minutes	Miss Latifa Patel
Focussed Interview	NO	30 minutes	Miss Latifa Patel

Interview Schedule

Meeting 1 - Last Routine Cystic Fibrosis Outpatient's Clinic, prior to the Annual Review Appointment

Prospective participants will be informed about the PROMISE Study and invited to participate. Patients will be given a copy of the, 'Information for Participants Sheet' with a covering letter to take away and read.

Meeting 2 - Annual Review Appointment

Prospective participants will be given an opportunity to discuss the study and make any necessary queries about the study and their involvement. Informed voluntary consent will be taken by Miss Latifa Patel in writing.

Participants will be asked to complete a, 'Self Efficacy Questionnaire.' This should take no longer than 10 minutes.

Patients and their parent(s)/carer(s) will then begin their Annual Review.

Following the successful completion of the Self Efficacy Questionnaire Group 1 participants will be asked to complete the, 'Challenges of Living with Cystic Fibrosis Questionnaire (CLCF-Q).' They can take as much or as little time to complete the Questionnaire. Annual Reviews usually begin between 08:30 and 09:30 and can last up to 3½ hours. Patients/parents/carers are known to welcome distraction during the waiting times between appointments.

Group 2 participants will not be asked to complete the, 'Challenges of Living with Cystic Fibrosis Questionnaire.'

Meeting 3 - Next Routine Cystic Fibrosis Outpatient's Clinic, post Annual Review Appointment (feedback of Annual Review Results)

Participants from Group 1 will be asked to complete a, "Self-feedback form," on how they feel the CLCF-Q will score them on specific areas.

Participants will then be given feedback from the Annual Review Assessment.

Each participant from Group 1 will then be given a detailed, colour coded illustration of their actual results from the CLC-Q measure. They will then be given the guidance, choice and opportunity to discuss their results with different members of the CF Team.

Following the clinic a summary of their Annual Review findings and any changes made will be detailed in a letter and sent to all participants in the study; this is the norm.

Meeting 4 – 2nd Routine Cystic Fibrosis Outpatient's Clinic, post Annual Review Appointment

Participants will be asked to complete a second, 'Self Efficacy Questionnaire.'

Meeting 4/5 - Interviews

A further meeting will be arranged with selected willing participants from across the study. This meeting will take place ideally, during or after a clinic visit and will be in the form of individual interviews. As well as the researcher and participant, a moderator and scribe will also be present. Refreshments will be provided.

Participants will be given an opportunity to discuss their thoughts and experiences and again address any concerns they may have and wish to discuss.

Meeting 5 - Feedback

All participants will receive feedback at their next available appointment. There will also be ongoing opportunities for participants to voice any opinions, queries or concerns they may have regarding the study process and findings.

A lay report will be offered to all participants.

Confidentiality

Access to data from the study will only be given to Southern, Patel, Glasscoe and Dixon. No other parties will be distributed identifiable data.

Safety Monitoring Plan

There are no physical adverse or serious adverse events associated with this study.

Some participants may host some feelings of intrusion and embarrassment as some of the questions on both questionnaires are personal. To alleviate these feelings we have a child psychologist on the team who will be available to deal with such issues.

Analysis Plan

Any changes in the PSE as established from CFSE-Q will be statistically analysed.

As this is pilot study design with an aim to use the data in power and sample size calculations for a larger multi centre much of the analysis will be descriptive. This study will not be adequately

powered to detect the difference between the groups hence hypothesis testing will not be appropriate.

All data from this study will be plotted to assess distribution (i.e. normal versus skewed). If the data is normally distributed then the means and standard deviations at baseline and post intervention thus the change in the Cystic Fibrosis Self Efficacy-Questionnaire score will be determined. If the data is skewed the median and interquartile ranges will be calculated instead.

The results of this pilot study will determine the hypothesis for a larger study. They will also allow the clinical significant change in the Cystic Fibrosis Self Efficacy-Questionnaire score to be determined.

The quantitative data will be limited to proving/disproving our hypothesis. It will be unable to quantify the experience entirely. We are hoping to hold interviews with certain parents/carers (n=6), on a one to one basis with an aim to extract this data.

Qualitative methods will play a key role in this study.

"Our linguistic ability enables us to descend into the realm of our primary perceptual and emotional experience, to find there a reality susceptible to verbal understanding, and to bring forth a meaningful interpretation of this primary level of our existence. . . . By finding meaning in experience and then expressing this meaning in words, the speaker enables the community to think about experience and not just live."

- Narrative Analysis, Dr Catherine Kohler Riessman (Adapted from Polkinhorne, 1988, p29-30)

For the qualitative aspect of this pilot study Patel will carry out focussed interviews with 3 carers from each group (n=6).

The interview will focus on a challenging period the parent/carer may have faced over the last year, how this challenge was approached by the family and the healthcare team and their views on how these challenges were managed as a whole.

The aim of the interview will be to gain an insight into the parent's/carer's attitude with regards to the management and communication in order to understand what factors/events they classed as important during this period.

It would be useful to know how valuable they feel the annual review process is and in the case of the intervention group if the CLCF process adds any value.

Interviews will last no longer than 30 minutes to reduce the demand on the family. All interviews will be audio taped only and transcribed fully. The transcribed version will be made available to the participant for approval of the contents. Only then will the contents be analysed.

The interviews will be analysed using narrative analysis.

The, "Self efficacy questionnaire" and "Study feedback form," have space allocated for further feedback and any queries. On receiving the questionnaires from the parents/carers the researcher (Latifa Patel) will also ask if there were any concerns. This process will help us to further evaluate the parent's/carer's experience of filling out the questionnaires.

Literature Cited

1. Rozov T, Cunha MT, Nascimento O, Quittner AL, Jardim JR. Linguistic validation of cystic fibrosis quality of life questionnaires. *J Pediatr (Rio J)*, 2006;82(2):151-156.
2. Britto MT, Kotagal UR, Chenier T, Tsevat J, Atherton HD, Wilmott RW. Differences between adolescents' and parents' reports of health-related quality of life in cystic fibrosis. *Pediatr Pulmonol*, 2004;37:165-171.
3. Modi AC, Quittner AL. Validation of a disease-specific measure of health-related quality of life for children with cystic fibrosis. *J Pediatr Psychol*, 2003;28(7):535-546.
4. Gee L, Abbott J, Hart A, Conway SP, Etherington C, Webb AK. Associations between clinical variables and quality of life in adults with cystic fibrosis. *J Cys Fib*, 2005;4:59-66.
5. Palermo TM, Harrison D, Koh JL. Effect of disease-related pain on the health-related quality of life of children and adolescents with cystic fibrosis. *Clin J Pain*, 2006;22:532-537.
6. Abbott J, Hart A, Morton AM, Dey P, Conway SP, Webb AK. Can health-related quality of life predict survival in adults with cystic fibrosis? *Am J Respir Crit Care Med*, 2009;179:54-58.
7. Blau H, Livne M, Mussaffi H. Cystic fibrosis in adults; a changing scene. *IMAJ*, 2003;5:491-495.
8. Abbott J, Hart A, Morton A, Gee L, Conway S. Health-related quality of life in adults with cystic fibrosis: the role of coping. *J Psychoso Res*, 2008;64:149-157.
9. Quittner AL. Measurement of quality of life in cystic fibrosis. *Cur Op Pulmonol Med*, 1998;4:326-331.
10. Szyndler JE, Towns SJ, van Asperen PP, McKay KO. Psychological and family functioning and quality of life in adolescents with cystic fibrosis. *J Cys Fib*, 2005;4:135-144.
11. Abbott J, Webb K, Dodd M. Quality of life in cystic fibrosis. *J R Soc Med*, 1997;90(31):37-42.
12. Goldbeck L, Zerrer S, Schmitz TG. Monitoring quality of life in outpatients with cystic fibrosis: feasibility and longitudinal results. *J Cys Fib*, 2007;6:171-178.
13. Wahl AK, Rustøen T, Hanestad BR, Gjengedal E, Moum T. Living with cystic fibrosis: impact on global quality of life. *Heart lung*, 2005;34:324-331.
14. Quittner AL, Modi A, Cruz I. Systematic review of health-related quality of life measures for children with respiratory conditions. *Paediatr Respira Rev*, 2008;9:220-232.
15. Abbott J. Health-related quality of life measurement in cystic fibrosis: advances and limitations. *Chron Respir Dis*, 2009;6:31-41.
16. Quittner AL, Buu A, Messer MA, Modi AC, Watrous M. Development and validation of the cystic fibrosis questionnaire in the United States. *CHEST*, 2005;128:2347-2354.
17. Goss CH, Quittner AL. Patient-reported outcomes in cystic fibrosis. *Proc Am Thorac Soc*, 2007;4:378-386.

18. Havermans T, Colpaert K, Dupont LJ. Quality of life in patients with cystic fibrosis: association with anxiety and depression. *J Cys Fib*, 2008;7:581-584.
19. Tullis DE, Guyatt GH. Quality of life in cystic fibrosis. *Pharmaco*, 1995;8(1): 23-33.
20. Kosciak RL, Douglas JA, Zaremba K, Rock MJ, Splaingard ML, Laxova A et al. Quality of life of children with cystic fibrosis. *J Pediatr*, 2005;147:64-68.
21. Bregnballe V, Thastum M, Schiøtz PO. Psychosocial problems in children with cystic fibrosis. *Acta Paediatrica*, 2007;96:58-61.
22. Boling W, Macrina DM, Clancy JP. The caregiver quality of life cystic fibrosis (CQOLCF) scale: modification and validation of an instrument to measure quality of life in cystic fibrosis family caregivers. *Qual Life Res*, 2003;12:1119-1126.
23. Bandura A. Self-efficacy: Toward a unifying theory of behavioural change. *Psychol Rev*, 1977;84(2):191-215.
24. The general self efficacy scale
<http://userpage.fu-berlin.de/~health/engscal.htm>
25. Lancaster GA, Dodd S, Williamson PE. Design and analysis of pilot studies: recommendations for good practice. *J Eval Clin Prac*, 2002;10(2):307-312.

7.1.2. Summary and Flow Diagram of Study

Summary of Study

Working with parents we have developed a parent reported outcome measure called the "Challenge of living with cystic fibrosis". Its aim is to measure the burden of care on everyday living for parents/carers of children with CF.

In this study we aim to explore the use of this measure in a clinical setting; the cystic fibrosis annual review. In particular, we will be exploring whether the use of this tool increases how effective and able parents feel they are in relation to the clinical team with regards to decision making, treatment and management. A person's belief in their ability to succeed is known as perceived self efficacy.

If we are able to improve the parents/carers self efficacy we may be able to improve their performance in everyday challenging task as a person is more likely to take on a challenging task when they believe they can do it. They may also be more likely to make decisions with confidence about the treatment their child receives. This possible increase in self efficacy may also correlate with their perception of control, overall improving the patient and their parents/carers global quality of life.

This study will use both qualitative and quantitative research methods.

On a randomised basis participants will be allocated to two groups; control and intervention group. Both groups will fill in a SE questionnaire both at the start and at the end of the study. The intervention group however, will be asked to complete the challenges of living with cystic fibrosis questionnaire in between and will receive further feedback regarding their results.

We will look at the results for any changes in the parents/carers self efficacy.

Outcome data from this pilot study will be used for a larger multi-centre trial.

DIAGRAM

The first meeting will take place at the last routine outpatient appointment before the child's annual review appointment.

Parents/carers will be fully informed about the study and invited to participate. They will also be given a covering letter and the patient information sheet to take home and read in their own time.

The second meeting will be at the child's annual review appointment.

Parents/carers will have an opportunity to discuss the study and ask any questions about the study and their involvement. Informed voluntary consent will be taken by Patel in writing.

Parents/carers who do not consent will be able to carry on with their annual review appointment as normal.

Consenting parents/carers will be asked to complete a 'Self Efficacy Questionnaire.' This should take no longer than 10 minutes. Patients and their parent(s)/carer(s) will then begin their Annual Review.

Parents/carers in Group 1 will be asked to complete the 'Challenges of Living with Cystic Fibrosis Questionnaire.' They will be able to take as much or as little time to complete the questionnaire as they wish.

Annual Reviews usually begin between 08:30 and 09:30 and can last up to 3½ hours. Patients/parents/carers are known to welcome distraction during the waiting times between appointments.

Parents/carers in Group 2 will not be asked to complete the 'Challenges of Living with Cystic Fibrosis Questionnaire.'

The third meeting will take place at the child's next routine outpatient appointment. The results of the annual review are usually given at this appointment.

Parents/carers from Group 1 will be asked to complete a 'Self-feedback form,' on how they feel the CLCF-Q will score them on specific areas.

Parents/carers from Group 1 will then be given feedback from the Annual Review Assessment.

Each parent/carer from Group 1 will then be given a detailed, colour coded illustration of their actual results from the CLCF-Q measure. They will then be given the guidance, choice and opportunity to discuss their results with different members of their healthcare team.

After the clinic a summary of their annual review will be sent to all parents/carers. This is a normal procedure.

Parents/carers from Group 1 will also be sent a copy of their 'Self-feedback form,' the actual results of the CLCF-Q and any changes that have been made to their child's treatment because of the CLCF-Q.

The fourth meeting will take place at the child's next routine outpatient appointment.

All parents/carers in the study (Group 1 and 2) will be asked to complete a second, 'Self Efficacy Questionnaire.'

Another meeting will be arranged with selected parents/carers in the study. It will be either during the fourth or the fifth meeting point.

This meeting will take place during or after a clinic visit. It will be in the form of an interview.

As well as the researcher and participant, a moderator and scribe will also be present. Refreshments will be provided.

Parents/carers will be asked about their thoughts on the study and suggestions or questions they might have.

The last meeting will take place at another outpatient clinic.

Everyone in the study will be given the results of the study. There will be an opportunity for parents/carers to voice any opinions they may have regarding the study and findings.

A lay report will also be offered to all participants in the study.

7.1.3. Interview Schedule



Interview Schedule

Meeting 1 - Last Routine Cystic Fibrosis Outpatient's Clinic, prior to the Annual Review Appointment

Prospective participants will be informed about the PROMISE Study and invited to participate. Prospective participants will be given a copy of the, 'Information for Participants Sheet' with a covering letter to take away and read.

Meeting 2 - Annual Review Appointment

Prospective participants will be given an opportunity to discuss the study and make any necessary queries about the study and their involvement. Informed voluntary consent will be taken by Miss Latifa Patel in writing.

Participants will be asked to complete a, 'Self Efficacy Questionnaire.' This should take no longer than 10 minutes.

Patients and their parent(s)/carer(s) will then begin their Annual Review.

Following the successful completion of the Self Efficacy Questionnaire Group 1 participants will be asked to complete the, 'Challenges of Living with Cystic Fibrosis Questionnaire (CLCF-Q).' They can take as much or as little time to complete the Questionnaire. Annual Reviews usually begin between 08:30 and 09:30 and can last up to 3½ hours. Patients/parents/carers are known to welcome distraction during the waiting times between appointments.

Group 2 participants will not be asked to complete the, 'Challenges of Living with Cystic Fibrosis Questionnaire.'

Meeting 3 - Next Routine Cystic Fibrosis Outpatient's Clinic, post Annual Review Appointment (feedback of Annual Review Results)

Participants from Group 1 will be asked to complete a, "Self-feedback form," on how they feel the CLCF-Q will score them on specific areas.

Participants will then be given feedback from the Annual Review Assessment.

Each participant from Group 1 will then be given a detailed, colour coded illustration of their actual results from the CLC-Q measure. They will then be given the guidance, choice and opportunity to discuss their results with different members of the CF Team.

Following the clinic a summary of their Annual Review findings and any changes made will be detailed in a letter and sent to all participants in the study; this is the norm.

Meeting 4 – 2nd Routine Cystic Fibrosis Outpatient's Clinic, post Annual Review Appointment

Participants will be asked to complete a second, 'Self Efficacy Questionnaire.'

Meeting 4/5 - Focus Groups

A further meeting will be arranged with selected willing participants from across the study. This meeting will take place ideally, during or after a clinic visit and will be in the form of an interview.

Participants will be given an opportunity to discuss their thoughts and experiences and again address any concerns they may have and wish to discuss.

Meeting 5 - Feedback

All participants will receive feedback at their next available appointment. There will also be ongoing opportunities for participants to voice any opinions, queries or concerns they may have regarding the study process and findings.

A lay report will also be offered to all participants.

7.2. Information for Carers – Final Versions

7.2.1. Letter to Parents, Carers and General Practitioner



Alder Hey Children's 
NHS Foundation Trust

Alder Hey

Eaton Road
Liverpool
L12 2AP

Telephone: 0151 228 4811
www.alderhey.com

March 2010

Dear Parent or Guardian

RE: The PROMISE Study (Parent Reported Outcome Measures Improve Self Efficacy)

Last year, we validated a questionnaire which explores the time and effort that goes into caring for a child with Cystic Fibrosis. This questionnaire is called the Challenges of Living with Cystic Fibrosis (CLCF). It has been suggested that Patient/Parent Reported Outcome measures such as the CLCF may have positive effects on the Self Efficacy of participants. We would like to explore this theory in further detail and in order to do so we need your help.

You may have already been approached regarding your participation in the PROMISE Study. Consider this letter as a formal invitation to participate in the PROMISE Study.

Please find enclosed in this pack a, 'PARTICIPANT INFORMATION SHEET.' If you do wish to participate in this study please read the information sheet carefully. Please do not hesitate to contact me if you have any questions regarding this study and your participation.

You will be approached at your next Annual Review appointment to discuss your possible involvement and discuss any queries you may have. Only participants who understand the information provided and voluntarily consent will be included in the study.

I look forward to seeing you at your next appointment and I thank you in advance for your cooperation and time.

Yours sincerely,

Miss Latifa Patel

Medical Student/MPhil Student

E-mail: l.patel@liv.ac.uk

Telephone: 0151 228 4811 Ext 4532

27/01/2010 15:39:30

Version 3

7.2.2. Participant Information Sheet



Participant Information Sheet

1. Title

Does administering a parent reported outcome measure during the annual review improve the self efficacy of the carer of a child who has cystic fibrosis?

2. Investigators

Dr Kevin Southern PhD MBChB
Dr Claire Glasscoe PhD
Miss Latifa Patel (Medical/MPhil Student)
Dr Clare Dixon PhD

3. Version number and date

Version 3
Last amended 8th March 2010

4. Invitation

You are invited to participate in a research study. Before you make a decision, it is important for you to understand why the research is being done and what it will involve. Please read the following information carefully. You may wish to discuss this with your family and friends. I would like to stress that you do not have to accept this invitation and should only agree to take part if you want to. Thank you in advance for taking your time to read this information. If there is anything you do not understand or if you have any questions please do not hesitate to contact Elinor on the details below.

5. What is the purpose of the study?

Last year, we validated a questionnaire which explores the time and effort that goes into caring for a child with Cystic Fibrosis (CF). This questionnaire is called the Challenges of Living with Cystic Fibrosis Questionnaire (CLCF-Q). We believe that Patient/Parent Reported Outcomes such as the CLCF-Q may have positive effects on your Self Efficacy. Self efficacy is your belief in your own ability to succeed. We would like to explore this theory.

6. Why have I been chosen to take part?

All patients currently registered on the Cystic Fibrosis list have been considered for the study. As a parent/carer of a child with Cystic Fibrosis you are being invited to join the study.

7. Do I have to take part?

Participation is completely voluntary and you are free to withdraw at anytime. You do not need to give us a reason. If you are unsure of what is involved please contact Elinor on the details at the end of the document.

8. What will it involve for my family and me if I take part?

The principal investigator is Dr Kevin Southern.

If you choose to participate in the study Latifa Patel will take informed consent from you at your child's annual review appointment. The study will be explained to you in detail and there will be an opportunity for you to raise any questions you may have.

You will be randomly allocated into 2 groups. If you are in Group 1 you will be asked to complete 3 questionnaires and 1 feedback form. If you are in Group 2 you will be asked to complete 2 questionnaires and one feedback form.

The Self Efficacy Questionnaire should take no longer than 10 minutes to complete and will be issued to all participants. The CLCF Questionnaire may take up to 30 minutes to complete and will only be issued to you if you are allocated into Group 1. All questionnaires will be issued during clinic time and will be collected back in at the end of clinic time.

Throughout the study you will be given opportunities to voice your opinions and any concerns you may about the study. With your permission we may use these to support the results of this study. Your name will not appear in the study and all information your give will remain anonymous. You can withdraw any comments at any point during the study.

9. How time consuming is this going to be?

You will not be required to attend any extra sessions/appointments so you do not need to make any extra journeys to Alder Hey. However, you may be asked to participate in an interview at the end of the study. This again will be scheduled during a clinic session.

10. Expenses and/or payments

You will incur no expenses. The time used to complete the study will be taken from your currently scheduled clinic times.

11. Are there any benefits in taking part?

You may benefit from the feedback given in the clinic appointment after your Annual Review regarding your questionnaire answers. You may also find the results of the study of use.

12. Are there any risks in taking part?

You are under no risk or disadvantage.

13. What if I am unhappy or there is a problem?

If you are unhappy, or if there is a problem, please do let Elinor know. Her contact details are below and she will try to help you. If you remain unhappy or have a complaint which you feel you cannot come to Elinor with then you should contact the Research and Development Manager Dot Lambert on 0151 252 5673 or dot.lambert@ric.nhs.uk. When contacting the Research Governance Officer, please provide details of the name or description of the study (so that it can be identified), the researcher(s) involved, and the details of the complaint you wish to make.

14. Will my participation be covered by an insurance scheme?

You are taking part in a NHS Research Ethics Committee approved study and are fully covered.

15. Will my participation be kept confidential?

All data will be collected on the paper questionnaires and then transferred anonymously onto a secure computer program. It is completely anonymous and confidential and individual data will never be discussed. It will only be used in connection with the above named study. Only the principal researcher will have access to the data.

Once the study has been evaluated the data will be used to come to a conclusion about the participant population as a whole and never individually.

16. How will my personal data be used?

Relevant sections of your child's medical notes and data collected during the study may be looked at by individuals from regulatory authorities or from the NHS Trust, where it is relevant to you taking part in this research.

During the study it may also be necessary for the researchers to look at your child's medical records and access personal data. This will be to aid your participation in the study.

Personal data will not be removed from the hospital premises and will not be mentioned anywhere in the study. Your child's personal data will be kept confidential at all times.

17. What will happen to the results of the study?

The results will be written up and you will be able to access the grouped data but not the individual anonymous data.

This study may be published at a later date and you will be informed and acknowledged for all your help and support. Copies of the study will be forwarded to you at your request.

18. What will happen if I want to stop taking part?

You can withdraw at anytime, without explanation. Results up to the period of withdrawal may be used, if you are happy for this to be done. Otherwise you may request that they are destroyed and no further use is made of them. Your routine treatment will not be affected in anyway.

19. Who can I contact if I have further questions?

ELINOR F BURROWS
RESPIRATORY DEPARTMENT
ALDER HEY CHILDREN'S NHS FOUNDATION TRUST
EATON ROAD
LIVERPOOL
MERSEYSIDE
L12 2AP

E-mail: elinor.burrows@alderhey.nhs.uk
Tel: 0151 252 5297

THANK YOU

7.2.3. Participant Consent Form



Consent Form

To be completed by the parent or legal guardian

Title of Study:

Does administering a parent reported outcome measure during the annual review improve the self efficacy of the carer of a child who has cystic fibrosis?

Name of Investigators:

Dr Kevin Southern
Dr Claire Glasscoe
Miss Latifa Patel
Dr Claire Dixon

I agree to take part in the above study and for the relevant information about my child to be used.

Child's Name.....(please print in CAPITAL letters)

- Please tick**
- I confirm that the above study has been fully explained to me
 - I confirm that I was given opportunity to ask questions
 - I confirm that I have received a copy of the, 'Participant Information Sheet'
 - I confirm that I have received information on how to gain access to the findings of this study when available

'I understand that relevant sections of my child's medical notes and data collected during the study, may be looked at by individuals from regulatory authorities or from the NHS Trust, where it is relevant to my taking part in this research. I give permission for these individuals to have access to my child's records'

Participation in this study is entirely voluntary and I have a right to withdraw from the study without giving a reason and in the knowledge that this will not affect my child's treatment in any way.

Name of parent/legal guardian.....(please print in CAPITAL letters)

Signature of parent/legal guardian.....

Signed in the presence of:

Name of witness.....(please print in CAPITAL letters)

Signature of witness.....

Date.....

Address.....

.....Postcode.....

08/03/2010 11:41:07

Version 5

7.2.4. Participant Information Sheet – Interview Only



Participant Information Sheet - Interview

1. Title

Does administering a parent reported outcome measure during the annual review improve the self efficacy of the carer of a child who has cystic fibrosis?

(INTERVIEWEES ONLY)

2. Investigators

Dr Kevin Southern	PhD MBChB
Dr Claire Glasscoe	PhD
Miss Latifa Patel	Medical/MPhil Student
Dr Clare Dixon	PhD

3. Version number and date

Version 1
Last amended 8th March 2010

4. Invitation

You previously consented to taking part in the above study.

Now that the above study is coming to an end you are being invited to participate in an interview.

Before you make a decision, it is important for you to understand why the research is being done and what it will involve. Please read the following information carefully. You may wish to discuss this with your family and friends. I would like to stress that you do not have to accept this invitation and should only agree to take part if you want to. Thank you in advance for taking your time to read this information. If there is anything you do not understand or if you have any questions please do not hesitate to contact Elinor on the details below.

5. What is the purpose of the interview?

As part of the study Latifa Patel will be conducting interviews. These interviews will enable us to gather important data which cannot be found elsewhere. We hope to learn about the true opinions of the participants with regards to the everyday challenges faced in caring for a child with cystic fibrosis.

This will also be an opportunity for the participant to voice their opinions about the care provided by the respiratory team and the impact of the study on the management of their child's treatment.

6. Why have I been chosen to take part?

By participating in the above study you are suitable for the interviews. Three participants from the 2 original groups were chosen on the basis of suitability.

7. Do I have to take part?

Participation is completely voluntary and you are free to withdraw at anytime. You do not need to give us a reason. If you are unsure of what is involved please contact Elinor on the details at the end of the document.

8. What will it involve for my family and me if I take part?

During or after one of your clinic appointments you will be asked to participate in an interview. You will be the only participant at the interview. Latifa Patel will be the interviewer.

The interview will focus on a challenging period that you, your child with cystic fibrosis and your family may have faced over the last year and how this challenge was approached by your family and the healthcare team. We want to know your views on how these challenges were managed as a whole. That is the aim of the interview.

Additionally, we would also like to discuss the role of this study in the care of your child and whether you feel that you have benefitted from the study.

A transcriber will also be present at the interview. This will be an independent person who is there only to note down what is happening and being said.

An independent observer (moderator) will also be present to simply observe the interview.

The interview itself will be taped. Once the interview is finished the taped interview will be transcribed (written down) and the tape will be destroyed.

Your details will be completely anonymous and will not be linked to the interview transcript.

9. How time consuming is this going to be?

You will not be required to attend any extra sessions/appointments so you do not need to make any extra journeys to Alder Hey.

10. Expenses and/or payments

You will incur no expenses. The time used to complete the study will be taken from your currently scheduled clinic times.

11. Are there any benefits in taking part?

You may benefit from the interview itself and you may also find the results of the study of use.

12. Are there any risks in taking part?

You are under no risk or disadvantage.

13. What if I am unhappy or there is a problem?

If you are unhappy, or if there is a problem, please do let Elinor know. Her contact details are below and she will try to help you. If you remain unhappy or have a complaint which you feel you cannot come to Elinor with then you should contact the Research and Development Manager Dot Lambert on 0151 252 5673 or dot.lambert@rlc.nhs.uk. When contacting the Research Governance Officer, please provide details of the name or description of the study (so that it can be identified), the researcher(s) involved, and the details of the complaint you wish to make.

14. Will my participation be covered by an insurance scheme?

You are taking part in a NHS Research Ethics Committee approved study and are fully covered.

15. Will my participation be kept confidential?

As mentioned previously all the data you provide will remain anonymous.

Once all the data has been collected you will be forwarded a copy of the interview transcript. If you approve it we will use it to support our data in the study. If you wish to retract certain comments made you will be given the opportunity to do so.

All data will be kept completely anonymous and confidential and individual data will never be discussed. It will only be used in connection with the above named study. Only the principal researcher will have access to the data.

Once the study has been evaluated the data will be used to come to a conclusion about the participant population as a whole and never individually.

16. What will happen to the results of the study?

The results will be written up and you will be able to access the grouped data but not the individual anonymous data.

This study may be published at a later date and you will be informed and acknowledged for all your help and support. Copies of the study will be forwarded to you at your request.

17. What will happen if I want to stop taking part?

You can withdraw at anytime, without explanation. Results up to the period of withdrawal may be used, if you are happy for this to be done. Otherwise you may request that they are destroyed and no further use is made of them. Your routine treatment will not be affected in anyway.

18. Who can I contact if I have further questions?

ELINOR F BURROWS
RESPIRATORY DEPARTMENT
ALDER HEY CHILDREN'S NHS FOUNDATION TRUST
EATON ROAD
LIVERPOOL
MERSEYSIDE
L12 2AP

E-mail: elinor.burrows@alderhey.nhs.uk
Tel: 0151 252 5297

THANK YOU

7.2.5. Participant Consent Form – Interview Only



Consent Form - Interview

Title of Study:

Does administering a parent reported outcome measure during the annual review improve the self efficacy of the carer of a child who has cystic fibrosis?

You have been invited to participate in an interview about the above study and the challenges you face.

Name of Investigator:

Miss Latifa Patel

I agree to take part in an interview about the above study and for the relevant information I provide in the interview to be used in the study.

Child's Name.....(please print in CAPITAL letters)

The researcher named above will interview you. There will also be a moderator (an independent observer) and a transcriber (an independent observer making notes) present and the interview will be recorded using a dictaphone. Refreshments will be provided.

Following the interview you will be given a copy of the information we will be using from your interview. You have the right to ask for certain information to be omitted and all information you do provide will remain anonymous.

Participation in this study is entirely voluntary and I have a right to withdraw from the study without giving a reason and in the knowledge that this will not affect my child's treatment in any way.

Name of parent/legal guardian.....(please print in CAPITAL letters)

Signature of parent/legal guardian.....

Signed in the presence of:

Name of witness.....(please print in CAPITAL letters)

Signature of witness.....

Date.....

Address.....

.....Postcode.....

08/03/2010 11:32:20

Version 2

7.3. Study Questionnaire – Final Versions

7.3.1. Cystic Fibrosis Self Efficacy-Questionnaire



Self Efficacy Questionnaire for Carers **Instructions**

The following questions are designed to assess your, 'self efficacy;' your belief in your own ability to succeed.

Please answer the questions below by ticking the box that most closely represents how you feel about the statement on the left. Only tick one box per statement.

1 = Not at all true

2 = Hardly true

3 = Moderately true

4 = Exactly true

The answers should be your own and should reflect how you feel, no one else. Do not spend too long thinking about the answers.

Self Efficacy Questionnaire for Carers

	Statement	Your Response			
		1 Not at all True	2 Hardly true	3 Moderately true	4 Exactly true
1	I can always manage to solve difficult problems if I try hard enough				
2	I face problems on a daily basis				
3	If someone opposes me, I can find the means and ways to get what I want				
4	It is easy for me to stick to my aims and accomplish my goals				
5	I am confident that I could deal efficiently with unexpected events				
6	Thanks to my resourcefulness, I know how to handle unforeseen situations				
7	I do have the support I need to solve problems				
8	I can solve most problems if I invest the necessary effort				
9	I can only solve a problem if I expected it to happen				
10	I can remain calm when facing difficulties because I can rely on my coping abilities				
11	When I am confronted with a problem, I can usually find several solutions				
12	If I am in trouble, I can usually think of a solution				
13	I never feel my views are fully appreciated				
14	I can usually handle whatever comes my way				

Self Efficacy Questionnaire for Carers
Feedback

Thank you for taking the time to complete this questionnaire.

Do you have any queries or comments regarding this questionnaire or this study?

If you would like to discuss your queries or concerns please contact Miss Latifa Patel on;

Institute of Child Health
Alder Hey
Eaton Road
Liverpool
L12 2AP

E-mail: l.patel@liv.ac.uk
Tel: 0151 228 4811 Fxt 3536
Mobile XXXXXXXXXX

7.3.2. The Challenges of Living with Cystic Fibrosis-Questionnaire

ID Number:

Challenges of Living with Cystic Fibrosis (CLCF)

A questionnaire for caregivers of children one year after diagnosis up to 13 years of age

validation 300
final draft

Name of person completing form	Relationship to child with CF
Name of child with CF	Date of Birth of Child with CF ____/____/____ Day / Month / Year
Boy/girl <i>Please circle</i>	
When was your child diagnosed with CF? ____/____ Month / Year	Does your child have a minder or baby sitter for part of the day? What nursery/school year/grade is your child in?
Today's Date ____/____/____ Day / Month / Year	

ID Number:

In answering the questions on this and the next page, please consider your responses over the past two weeks

Family Lifestyle

1. Are you: A lone caregiver Living with spouse or partner A lone caregiver living with family

2. How many children do you care for in your family?

3. How many children with CF do you have living with you?

4. How does your family divide childcare relating to CF?

I do it all Equal shares My partner/others do it all

5. How would you describe your general family lifestyle? (please circle one number on each of the scales below)

1 2 3 4 5
Relaxed Stressed out

1 2 3 4 5
Busy Laid back

1 2 3 4 5
Chatty Quiet

1 2 3 4 5
Work together Work as individuals

1 2 3 4 5
Disorganised Organised

1 2 3 4 5
No fixed routines Fixed routines

1 2 3 4 5
Sporty/active Not sporty/active

6. How well do you think you are juggling the demands of CF with the needs of your family?

1 2 3 4 5
No difficulty A little Marginal Definite Great difficulty

7) How well do you think your family as a whole handles the challenges of CF ?

1 2 3 4 5
Very easily Easily Marginally easily Some difficulty Great difficulty

CF Background

8) Does your child need enzymes with food? Yes No Don't know

9) Has your child been diagnosed with CF-related diabetes? Yes No Don't know

10) Based on your most recent clinic visit, what is your child's FEV₁ % predicted/'blow score'?

11) Has your child grown anything on a cough swab/sputum over the last three months? Yes No Don't know

If yes, please indicate which of the following:

Aspergillus Pseudomonas Burkholderia cepacia Other (please specify) _____

12) Over the last two weeks, has your child been: (please tick one)

Unwell Mostly unwell Mixture of well and unwell Mostly well Well

ID Number:

13) Has your child ever had a hospital admission for any of the following? Yes No
If yes then please tick all that apply)

IV Antibiotics Portacath fitted Gastrostomy tube fitted Nasogastric tube Oxygen

Other please specify _____

Child's Character

	Strongly agree	Agree	Neutral	Disagree	Strongly disagree
14) My child is very determined; when s/he wants to do something s/he usually keeps trying until s/he succeeds	1	2	3	4	5
15) My child makes more demands on me than I expected	1	2	3	4	5
16) My child goes to bed easily	1	2	3	4	5
17) My child sleeps throughout the night	1	2	3	4	5
18) It takes a long time for my child to settle with new routines	1	2	3	4	5
19) My child makes friends easily	1	2	3	4	5
20) My child is easily upset by things generally	1	2	3	4	5
21) My child is very moody	1	2	3	4	5
22) My child is so active it exhausts me	1	2	3	4	5
23) My child is popular with his/her peers	1	2	3	4	5
24) My child reacts very strongly when something happens that s/he doesn't like	1	2	3	4	5
	Strongly agree	Agree	Neutral	Disagree	Strongly disagree

Challenges to Family Life

25) How supported do you feel by the following groups of people? (please tick boxes that best reflect your view).

	Very supported				Not at all supported			
Family members	1	2	3	4				
Friends	1	2	3	4				
Another parent whose child has CF	1	2	3	4				

Are there any other key people who are supportive to you?

(For each of the following statements, please circle one number)

26) To reduce the risk of cross infection, the CF team advises that people with CF avoid contact with other people with CF. How much does this affect contact with other CF families?

1 2 3 4 5
 A great deal Some Moderate A little Not at all

27) Caring for a child with CF can involve extra expense. How difficult is it for you to manage this?

1 2 3 4 5
 Very difficult moderately Not at all difficult

28) To what extent do you think CF has changed your work pattern?

1 2 3 4 5
 A great deal Some Moderate A little Not at all

If YES, in what way? _____

ID Number:

29) How often have you had a disturbed night's sleep in the past 2 weeks?

1	2	3	4	5
Every night	Frequent	Some	Few	No nights

30) How do you know when you need a break?

Hopes and Worries

In answering the questions on this and the next section please consider your responses over the past two weeks

31) Some say that living with CF is like a balance of hope and worry:

What hopes do you have for your child?

	Very Hopeful				Not hopeful					Very hopeful				Not hopeful			
S/he will adjust well to secondary school	1	2	3	4					S/he will have a family of his/her own	1	2	3	4				
S/he will go on to higher education	1	2	3	4					S/he will continue to be as well as s/he is now	1	2	3	4				
S/he will have a job	1	2	3	4					There will be an advance in science that will help my child	1	2	3	4				

32) It is difficult to predict what the future holds in relation to CF. To what extent does this uncertainty affect your family's approach to life?

1	2	3	4	5
A great deal		Moderately		Not

If it does, in what way? _____

33) How much does the responsibility of looking after a child with CF affect you?

1	2	3	4	5
A great deal		Moderately		Not at all

34) How much is your child's growth a worry for you?

A) Height?

1	2	3	4	5
A great deal		Moderately		Not at all

B) Weight?

1	2	3	4	5
A great deal		Moderately		Not at all

35) To what extent are you worried that your child might become infected with pseudomonas when s/he is outside the home, e.g. at friends' houses, at school?

1	2	3	4	5
A great deal		Moderately		Not at all

36) How worried are you about a change in your child's lung function?

1	2	3	4	5
A great deal		Moderately		Not at all

37) What is your main worry? (Please specify)

38) What do you feel most positive about? (Please specify)

ID Number:

CF Routines

39) How easy was it to establish the CF care routine after your child was diagnosed?

1	2	3	4	5
Very easy		Moderately easy		Not at all easy

40) How much of a problem is it to manage the daily routines for CF now?

No problem A constant problem

a) Mealtimes—getting him/her to eat enough

1	2	3	4	5

b) Digestion – tummy problems (wind, pain, diarrhoea)

1	2	3	4	5

c) Taking enzymes/creon

1	2	3	4	5

d) Taking vitamins/oral antibiotics

1	2	3	4	5

e) Doing physiotherapy

1	2	3	4	5

d) Doing nebulised medications

1	2	3	4	5

No problem A constant problem

41) With all the things that need to be done, it may be overwhelming at times. How true has this been for you over the last 2 weeks?

1	2	3	4	5
Very true		Neutral		Not at all true

42) Do you think doing all these treatments for your child are justified?

1	2	3	4	5
Completely justified		Not sure		Not at all justified

Please specify any treatment you have a question about?

Community support

43) What quality of relationship do you have with your local GP/surgery?

1	2	3	4	5
Very good		Moderately good		Not at all good

44) How helpful is your local pharmacist?

1	2	3	4	5
Very helpful		Moderately helpful		Not at all helpful

45) What sort of relationship do you have with your child's minder/nursery/school?

1	2	3	4	5
Very good		Moderately good		Not at all good

ID Number:

46) How comfortable are you with how your child's minder / nursery, or school gives medications to your child?

1 2 3 4 5
|-----|-----|-----|-----|-----|
Very comfortable Moderately comfortable Not at all comfortable

47) Do you get support from your child's minder/nursery or school above and beyond creon or inhalers e.g., physio or nebs?

Yes No

If yes, what kind of support? *Please specify*

CF Clinic & Pharmacy Visits

48) How many clinic visits has your child had since last research point? visits

49) How long on average do you spend at the clinic? (Number of hours) hours

50) How frequently does your child usually attend the CF clinic? every weeks

51) How often do you use the hospital pharmacy? Most visits Sometimes Rarely

52) Please think about your last visit to pharmacy. How acceptable was the wait for medicines?

1 2 3 4 5
|-----|-----|-----|-----|-----|
Very acceptable Very unacceptable

53) How consistent are the messages you get from different members of the CF team?

1 2 3 4 5
|-----|-----|-----|-----|-----|
Very consistent Moderately consistent Not at all consistent

54) How much information would you like to have from the CF team about your child's condition or treatments?

1 2 3 4 5
|-----|-----|-----|-----|-----|
More information The same as now Less information

ID Number:

Inpatient and Day Patient Stays

55) How many times was your child with CF admitted to hospital over the last three months for a day or more?

What was it for?

56) Was this the first time your child was admitted to hospital because of his/her CF? Yes No Don't know

57) Was this admission routine/preventative or for treatment/intervention for symptoms? Routine/prevention Treatment N/A

58) How stressful was this admission for you and your family?
 1 2 3 4 5
 |-----|-----|-----|-----|
 Very stressful Stressful Not at all stressful

59) Here is a list of types of stress you may have experienced during your child's admission. Please rank them in order of their stressfulness.

Not applicable because my child has not had an admission in the last three months.

Type of stress during last admission: Rate 1-6 for stressfulness with 1 high and 6 low

- staying in over night
- disruptions to family life
- getting good care in hospital
- getting the intravenous line in
- child's loneliness
- communication with health care professionals

other type of stress _____

ID Number:

CF Treatments

In answering the questions on this and the next two pages please consider your responses over the past two weeks

60) Over the last two weeks how much has your child needed the following treatments to keep him/her well?	SECTION 1A			SECTION 1B		SECTION 1C		
	Prescribed?			Treatment Taken : Time Required		Treatment Taken : Effort Required		
	<i>Please tick a circle. If yes, complete Section B & C.</i>			<i>Please estimate the time spent doing this treatment</i>		<i>Please tick the circle that represents the amount of effort required to do each treatment</i>		
<i>Please tick one circle next to each question in section A and estimate time taken (section B) and effort (section C) involved</i>	Yes	Yes but not done	No	Minutes per day doing the task	Number of days of treatment over past week	Minimal Effort	Moderate Effort	High Effort
Calculating doses	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>			<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Extra day time feeding /calorie supplements	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>			<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Inhalers	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>			<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Insulin injections for diabetes	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>			<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
IV antibiotics at home	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>			<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
IV antibiotics in hospital	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>			<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Nebulised medications:								
Antibiotics	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>			<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
DNase	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>			<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Hypertonic Saline	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>			<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Salbutamol	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>			<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Non-prescribed (alternative remedies e.g., herbal remedies)						<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Oral antibiotics (back-up & specific)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>			<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Other medicines (lactulose, antacids, vitamins etc)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>			<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Overnight feeds through a gastrostomy or nasogastric tube	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>			<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Oxygen therapy delivered by mask or nasal specs	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>			<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Pancreatic enzyme Supplements (creon)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>			<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Physiotherapy	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>			<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Ursodeoxycholic acid (URSO) for liver involvement	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>			<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Collecting & preparing medicines and cleaning equipment	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>			<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

ID Number:

61) We want to know how hard it has been for YOU to manage these treatments. <i>(Please tick one circle from Section 2A for each treatment type that applies and then consider Section 2B)</i>	Section 2A: How hard has it been for YOU to manage these treatments?				Section 2B: Please tick the circle next to any treatment that YOU would like to talk about the next time you come to clinic. This may be how it is done or whether it is relevant.
	Very Difficult	Somewhat Difficult	Not at all Difficult	Does not apply	Yes, I would like to talk about this treatment at the next clinic or annual review
Extra feeding, calorie supplements	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
Inhalers	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
Insulin injections for diabetes	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
IV antibiotics at home	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
IV antibiotics in hospital	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
Nebulised medications:					
Antibiotics	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
DNase	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
Hypertonic Saline	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
Salbutamol	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
Non-prescribed remedies (e.g. alternative remedies)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
Oral antibiotics (back-up & specific)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
Other medicines (lactulose, antacids, vitamins etc)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
Overnight feeds through a gastrostomy or nasogastric tube	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
Oxygen therapy by mask or nasal specs	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
Pancreatic enzyme supplements (creon)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
Physiotherapy	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
URSO for the liver	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>

ID Number:

62) How do you think YOUR CHILD has managed these aspects of the CF routine over the last two weeks? <i>(Please tick one circle from Section 3A for each treatment type and then consider Section 3B)</i>	Section 3A: How hard has it been for YOUR CHILD to manage these treatments?				Section 3B: Please tick the circle next to any treatment that YOUR CHILD would like to talk about the next time you come to clinic. This may be how it is done or whether it is relevant.
	Very Difficult	Somewhat Difficult	Not at all Difficult	Does not apply	Yes, my child would like to talk about this treatment at the next clinic or annual review
Extra feeding, calorie supplements	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
Inhalers	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
Insulin injections for diabetes	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
IV antibiotics at home	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
IV antibiotics in hospital	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
Nebulised medications:					
Antibiotics	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
DNase	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
Hypertonic Saline	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
Salbutamol	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
Non-prescribed remedies (e.g. alternative remedies)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
Oral antibiotics (back-up & specific)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
Other medicines (lactulose, antacids, vitamins etc)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
Overnight feeds through a gastrostomy or nasogastric tube	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
Oxygen therapy by mask or nasal specs	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
Pancreatic enzyme supplements (creon)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
Physiotherapy	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>
URSO for the liver	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="checkbox"/>

THANK YOU

7.3.3. Self-Feedback Form – Carer Copy



Self Feedback Form for Carers **Instructions**

The following form is designed to assess how well you think you are coping with the Challenges of Living with Cystic Fibrosis.

Please respond to the statements by ticking one box in the right column. You should choose your response using the following key.

- | | |
|---|----------------------------------|
|  | This statement is false |
|  | This statement is sometimes true |
|  | This statement is true |

The answers should be your own and should reflect how you feel, no one else. Do not spend too long thinking about the answers.

At your next appointment you will be given a copy of this form to take home.

Domain		Scale		
		True		False
Family Lifestyle "We work well as a family"	"We share challenges"			
	"We work together"			
CF Background "My child's CF is well managed"				
Child's Character "My child is well behaved"				
Challenges of Family Life "We face CF together as a family"	"I have a lot of support"			
	"We don't worry about infections"			
	"CF doesn't impact on our life too much"			
Hopes and Worries "My hopes for everyday life are bigger than my worries about everyday life"	"I am hopeful about our day to day life"			
	"I don't worry about everyday life"			
Hopes and Worries "My hopes for the future are bigger than my worries about the future"	"I have a lot of hope for the future"			
	"I don't worry about the future"			
CF Routines "Managing my child's CF is easy"	"We have a good routine"			
	"I never feel overwhelmed"			
Community Support "I have a good relationship with the CF team in the community"				
CF Clinic and Pharmacy Visits "I find it easy getting my child's medication"				
Inpatient and Day Patient Stays "I am happy with the amount of admissions my child has had"				
CF Treatments "I am happy with my child's CF treatment"				

Self Feedback Form for Carers
Feedback

Thank you for taking the time to complete this feedback form.

Do you have any queries or comments regarding this form or this study?

If you would like to discuss your queries or concerns please contact Miss Latifa Patel on;

Institute of Child Health
Alder Hey
Eaton Road
Liverpool
L12 2AP

E-mail: l.patel@liv.ac.uk
Tel: 0151 228 4811 Ext 3536
Mobile: XXXXXXXXXX

7.3.4. Feedback Form – Researcher Copy



Feedback Form Information

What does this form mean?

The following form has been completed using the information you gave in the Challenges of Living with Cystic Fibrosis-Questionnaire.

A selected group of responses have been statistically analysed and have been allocated a score. This score can be found under the heading, "Score."

The scores have then been graded using the following key.

	This statement is false
	This statement is sometimes true
	This statement is true

This form should give you an idea of how well you are coping with the challenges of living with cystic fibrosis.

What happens next?

You will be given a copy of this form and a copy of, "Self Feedback Form." to take home and review.

The results on this form should be compared to your results on the, "Self Feedback Form."

If you are concerned about your score and would like to know how you could face the challenges better you could talk to someone on the Cystic Fibrosis Care Team.

On the far right column under the heading, "Who can I talk to?" are a list of members of your child's Cystic Fibrosis Care Team who you can talk to, to discuss your results and any concerns you may have.

Domain		Score	Scale	Who can I talk to?
Family Lifestyle "We work well as a family"	"We share challenges"		True False	Doctor CF Nurse Psychologist
	"We work together"			
CF Background "My child's CF is well managed"			True False	Doctor CF Nurse Physiotherapist Dietician
Child's Character "My child is well behaved"			True False	Doctor Psychologist
Challenges of Family Life "We face CF together as a family"	"I have a lot of support"		True False	Doctor CF Nurse Psychologist
	"We don't worry about infections"			
	"CF doesn't impact on our life too much"			
Hopes and Worries "My hopes for everyday life are bigger than my worries about everyday life"	"I am hopeful about our day to day life"		True False	Doctor CF Nurse Psychologist Physiotherapist Dietician
	"I don't worry about everyday life"			
Hopes and Worries "My hopes for the future are bigger than my worries about the future"	"I have a lot of hope for the future"		True False	Doctor CF Nurse Psychologist Physiotherapist Dietician
	"I don't worry about the future"			
CF Routines "Managing my child's CF is easy"	"We have a good routine"		True False	Doctor CF Nurse Psychologist Physiotherapist Dietician
	"I never feel overwhelmed"			
Community Support "I have a good relationship with the CF team in the community"			True False	Doctor GP Pharmacist School/Minder
CF Clinic and Pharmacy Visits "I find it easy getting my child's medication"			True False	Doctor Pharmacist CF Nurse
Inpatient and Day Patient Stays "I am happy with the amount of admissions my child has had"			True False	Doctor CF Nurse Psychologist
CF Treatments "I am happy with my child's CF treatment"			True False	Doctor CF Nurse Dietician Physiotherapist

Notes:

Feedback Form for Carers
Feedback

Thank you for taking the time to review this feedback form.

Do you have any queries or comments regarding this form or this study?

If you would like to discuss your queries or concerns please contact Miss Latifa Patel on;

Institute of Child Health
Alder Hey
Eaton Road
Liverpool
L12 2AP

E-mail: l.patel@liv.ac.uk
Tel: 0151 228 4811 Ext 3536
Mobile: XXXXXXXXXX

Study Feedback

Thank you for taking the time to participate in this study.

**1. Did you find the results of the Challenges of Living with Cystic Fibrosis-
Questionnaire useful?**

YES/NO (*please circle*)

**1. Did you speak to any members of the Cystic Fibrosis Care Team about your
challenges?**

YES/NO (*please circle*)

**2. Do you have any queries or comments regarding this questionnaire or this
study?**

YES/NO (*please circle*)

Comments:

If you would like to discuss your queries or concerns please contact Miss Latifa Patel on;

Institute of Child Health
Alder Hey
Eaton Road
Liverpool
L12 2AP

E-mail: l.patel@liv.ac.uk
Tel: 0151 228 4811 Ext 3536
Mobile: XXXXXXXXXX

7.4. Letters of Correspondence

7.4.1. Approval Letter for Governance Officer – University of Liverpool



UNIVERSITY OF
LIVERPOOL

Legal Services Ref: SP000458
Faculty of Medicine Ref: UoL000536

Dr Kevin Southern
School of Reproductive and Developmental Medicine

Tuesday, 05 January 2010

Dear Dr Southern

I am pleased to confirm that the University is prepared to act as Co-Sponsor with Alder Hey NHS Foundation Trust under the Department of Health's Research Governance Framework for Health and Social Care (2005) for your study entitled "Does administering a parent reported outcome measure during the annual review improve the self efficacy of the carer of a child who has cystic fibrosis?". This approval for co-sponsorship is subject to the following:

1. The University expects you, as Chief Investigator, to conduct the study in full compliance with the requirements of the Framework so that it is able to meet its obligations as Co-Sponsor.
2. University professional indemnity and clinical trials insurances will apply to the study as appropriate. This is on the assumption that no part of the study will take place outside of the UK.
3. If you wish to conduct any part of the study in a site outside the UK, or the study requires participation of a site other than one belonging to the Co-Sponsor, or you wish to sub-contract any part of the study to a third party you must contact Contract Services in the first instance to ensure that appropriate contractual arrangements are in place.
4. In addition to sponsorship, your study will require NHS ethical approval. If you have not already done so, in order to apply for this please use the Integrated Research Application System (IRAS) at <https://www.myresearchproject.org.uk/Home.aspx>.

As part of the process you will require the "Declaration of the Sponsor" to be signed and completed by the University. This can be done in two ways, either using the IRAS electronic authorisation system or by sending a hard copy to Legal Services for signature.

If using the electronic authorisation service, please ensure that you have inserted the relevant Sponsor contact information as detailed at the top of this letter. If you wish to obtain a hard copy signature, please contact me at ethics@liverpool.ac.uk or on the number above in order to arrange an appointment. You may confirm to NRES that the insurances described in paragraph 2 above will extend to cover for non-negligent harm.

Miss S L Fletcher
Research Governance Officer,
Legal Services

2nd Floor, Block C
Waterhouse Buildings
3 Brownlow Street
Liverpool L69 3GL

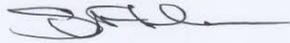
Telephone: 0151 794 8290
Facsimile: 0151 794 8728
Email: s.l.fletcher@liverpool.ac.uk

A member of the
Russell Group

5. As the Chief Investigator, the University expects you to comply, where appropriate, with the University's policy on the use and / or storage of human tissues, details of which may be found at www.liverpool.ac.uk/humantissues.

I trust that this statement will enable you to proceed with your research but if you have any queries please contact me on 0151 794 8290 (email s.l.fletcher@liverpool.ac.uk). For general queries relating to University sponsorship please contact the Faculty of Medicine Research Support Office at medresteam@liverpool.ac.uk.

Yours sincerely,



Miss Sarah Fletcher
Research Governance Officer, Legal Services

Cc Head of School, Reproductive and Developmental Medicine
Mrs Lindsay Carter, Research Coordinator, Faculty of Medicine Support Office

7.4.2. Letter from Statistician – University of Liverpool



Dr Kerry Dwan
Centre for Medical Statistics and
Health Evaluation
Shelley's Cottage
Brownlow Street
Liverpool
L69 3GS

Telephone: 0151 794 4315
Facsimile: 0151 794 5130
Email:
kerry.dwan@liverpool.ac.uk

To the Ethics committee

I have reviewed the study proposed by Latifa Patel. Although the sample size is relatively small, it is sufficient for an exploratory pilot study. This will provide information for a sample size and power calculation to be made for a definitive study. Summary statistics will be used to analyse the data.

Yours sincerely

Kerry Dwan

University switchboard
Telephone: 0151 794 2000
Facsimile: 0151 708 6502

Director and Reader
Professor Paula Williamson

Co Directors and Senior Lecturers
Dr Carrol Gamble and Dr Catrin Tudur Smith

7.4.3. Approval Letter from Research and Development – Alder Hey

Alder Hey Children's 
NHS Foundation Trust

Department Name
Direct Line: 0151 252 5673
Direct Fax No: 0151 252 5673
Email: Matthew.Peak@alderhey.nhs.uk
Dot.Lambert@alderhey.nhs.uk
Katherine.Jopson@alderhey.nhs.uk
Emma.Masango@alderhey.nhs.uk

Eaton Road
Liverpool
L12 2AP
www.alderhey.com
Main Switchboard
0151 228 4811

Dr Kevin Southern
Consultant in Respiratory Paediatric
Medicine
Institute for Child Health
Alder Hey Children's NHS Foundation
Trust

4th February 2010

Our ref: 09/60/RE
Your ref:

Re: Do Parent Reported Outcome Measures Improve Self Efficacy? (PROMISE study)

Thank you for submitting the above application to the Research & Development Office. It was considered by the Research Review Committee on 4th January 2010. In accordance with the requirements of the Research Governance Framework for Health and Social Care, and relevant legislation, and I am pleased to confirm approval for it to go ahead within the Alder Hey Children's NHS Foundation Trust.

It will be the responsibility of the Chief Investigator to comply with the responsibilities laid down, in the Research Governance Framework for Health and Social Care (2001 and 2005), by the Department of Health. Please see the enclosed leaflet for further information.

A full copy of the Research Governance Framework for Health and Social Care can also be obtained from the Department of Health website at www.dh.gov.uk, the R&D Office, or the Alder Hey Children's NHS Foundation Trust Intranet.

If you are using the Trust's standard Research Consent and Assent forms, please contact the R+D Administrator, Katherine Jopson on ext 2673 to arrange a time for collection.

The R&D Office is monitoring all research activity within the Trust and will contact you within 6 months time to ask whether the study has started and whether the start date has changed. **Please inform the R&D office immediately if the study starts within this 6 month period.**

Timely submission of reports is a condition of continuing authorisation to support this study by the Trust.

Yours sincerely


P.P. Eileen Baildam
Chair Research Review Committee

Cc: Latifa Patel, Institute of Child Health AH



RESEARCH GOVERNANCE FRAMEWORK FOR HEALTH AND SOCIAL CARE

RESPONSIBILITIES OF THE PRINCIPAL INVESTIGATOR

It is the principal investigator's responsibility to ensure that:

- The dignity, rights, safety and well being of participants are given priority at all times by the research team.
- The research is carried out in accordance with the research governance framework.
- When a study involves participants under the care of a doctor, nurse or social worker for the condition to which the study relates, those care professionals are informed that their patients or users are being invited to participate and agree to retain overall responsibility for their care.
- When the research involves user or carer or a child, looked after or receiving services under the auspices of the local authority, that the agency director or her deputy agrees to the person (and/or their carer) being invited to participate and is fully aware of the arrangements for dealing with any disclosure or other relevant information.
- Unless participants or the relevant research ethics committee request otherwise participants' care professionals are given information specifically relevant to their care which arises in the research.
- The study complies with all legal and ethical requirements.
- A Material Transfer Agreement is in place with the receiving organisation for any samples sent outside of the Trust.
- Each member of the research team is qualified by education, training and experience to discharge his/her role in the study.
- Students and new researchers have adequate supervision, support and training.
- The research follows the protocol approved by the research committee.
- Any proposed changes or amendments to or deviations from the protocol are submitted for approval to the ethics committee, the research sponsor and any other appropriate body.
- Procedures are in place to ensure collection of high quality, accurate data and the integrity and confidentiality of data during processing and storage.
- Arrangements are made for the appropriate archiving of data when the research has finished.
- The findings from the work are opened to critical review through the accepted scientific and professional channels.
- Once established, findings from the work are disseminated promptly and fed back as appropriate to participants.
- All data and documentation associated with the study are available for audit at the request of an auditing authority.

7.4.4 Validation Letter from Research Ethics Committee – Liverpool East

North West 3 Research Ethics Committee - Liverpool East

Bishop Goss Complex
Victoria Building
Rose Place
Liverpool
L3 3AN

Telephone: 0151 330 2051
Facsimile: 0151 330 2075

03 February 2010

Dr Kevin W Southern
MPhil Student (Intercalated Degree)
Alder Hey Children's Hospital
Institute of Child Health
Eaton Road
Liverpool
L12 2AP

Dear Dr Southern

Full title of study: Does administering a parent reported outcome measure during the annual review improve the self efficacy of the carer of a child who has cystic fibrosis? A pilot study.
REC reference number: 10/H1002/10

Thank you for your application for ethical review, which was received on 01 February 2010. I can confirm that the application is valid and will be reviewed by the Committee at the meeting on 18 February 2010.

Meeting arrangements

The meeting will be held in the Boardroom, Royal Liverpool Children's NHS Trust, Alder Hey, Eaton Road, Liverpool, L12 2AP on 18 February 2010. The Committee would find it helpful if you could attend the meeting to respond to any questions from members. Other key investigators and a representative of the sponsor are also welcome to attend. This may avoid the need to request further information after the meeting and enable the Committee to make a decision on the application more quickly.

If you have a disability and need any practical support when attending the REC meeting you may wish to contact the REC office so appropriate arrangements can be made if necessary.

If you are unable to attend the meeting the Committee will review the application in your absence.

The review of the application has been scheduled for 5.30pm. Would you please let me know whether or not you would be available to attend at this time? Please note that it is difficult to be precise about the timing as it will depend on the progress of the meeting. We would kindly ask you to be prepared to wait beyond the allocated time if necessary.

Committee meetings are occasionally attended by observers, who will have no vested interest in the applications under review or take any part in discussion. All observers are required to sign a confidentiality agreement.

Documents received

The documents to be reviewed are as follows:

<i>Document</i>	<i>Version</i>	<i>Date</i>
Covering Letter		31 January 2010
REC application	1.0	01 February 2010
Protocol	7.0	27 January 2010
Investigator CV		01 February 2010
Participant Information Sheet	2.0	04 November 2009
Participant Consent Form	4.0	27 January 2010
Letter of invitation to participant	1.0	01 January 2010
GP/Consultant Information Sheets	1.0	01 January 2010
Letter from Sponsor		05 January 2010
Letter from Statistician		15 January 2010
Summary/Synopsis	1.0	01 February 2010
Interview Schedules/Topic Guides	3.0	27 January 2010
Questionnaire: Validated Questionnaire		27 January 2010
Questionnaire: Non-Validated Questionnaire	4.0	27 January 2010
Student CV		01 February 2010
CV - Dr Claire Glasscoe		01 February 2010
CV - Dr Clare Dixon		01 February 2010

No changes may be made to the application before the meeting. If you envisage that changes might be required, we would advise you to withdraw the application and re-submit it.

Notification of the Committee's decision

You will receive written notification of the outcome of the review within 10 working days of the meeting. The Committee will issue a final ethical opinion on the application within a maximum of 60 days from the date of receipt, excluding any time taken by you to respond fully to one request for further information or clarification after the meeting.

R&D approval

All researchers and local research collaborators who intend to participate in this study at sites in the National Health Service (NHS) or Health and Social Care (HSC) in Northern Ireland should apply to the R&D office for the relevant care organisation. A copy of the Site-Specific Information (SSI) Form should be included with the application for R&D approval. You should advise researchers and local collaborators accordingly.

The R&D approval process may take place at the same time as the ethical review. Final R&D approval will not be confirmed until after a favourable ethical opinion has been given by this Committee.

Guidance on applying for R&D approval is available at <http://www.rdforum.nhs.uk/rdforum>.

There is no requirement for separate Site-Specific Assessment as part of the ethical review of this research. The SSI Form should not be submitted to local RECs.

Communication with other bodies

All correspondence from the REC about the application will be copied to the research sponsor and to the R&D office for the lead site. It will be your responsibility to ensure that other investigators, research collaborators and NHS care organisation(s) involved in the study are kept informed of the progress of the review, as necessary.

10/H1002/10	Please quote this number on all correspondence
--------------------	---

Yours sincerely

Miss Robyn Duncan
Committee Administrator

Email: Robyn.duncan@liverpoolpct.nhs.uk

Copy to: *Miss Sarah Fletcher, University of Liverpool
Dot Lambert, R&D, Alder Hey*

7.4.5. Provisional Approval from Research Ethics Committee – Liverpool East

North West 3 Research Ethics Committee - Liverpool East

Bishop Goss Complex
Victoria Building
Rose Place
Liverpool
L3 3AN

Telephone: 0151 330 2077
Facsimile: 0151 330 2075

03 March 2010

Dr Kevin W Southern
MPhil Student (Intercalated Degree)
Alder Hey Children's Hospital
Institute of Child Health
Eaton Road, Liverpool
L12 2AP

Dear Dr Southern

Study Title: Does administering a parent reported outcome measure during the annual review improve the self efficacy of the carer of a child who has cystic fibrosis? A pilot study.
REC reference number: 10/H1002/10
Protocol number: 7.0

The Research Ethics Committee reviewed the above application at the meeting held on 18 February 2010. Thank you for attending to discuss the study. A further meeting was held on 03 March 2010 to ratify the decision made

Documents reviewed

The documents reviewed at the meeting were:

<i>Document</i>	<i>Version</i>	<i>Date</i>
Covering Letter		31 January 2010
REC application	1.0	01 February 2010
Protocol	7.0	27 January 2010
Investigator CV		01 February 2010
Participant Information Sheet	2.0	04 November 2009
Participant Consent Form	4.0	27 January 2010
Letter of invitation to participant	1.0	01 January 2010
GP/Consultant Information Sheets	1.0	01 January 2010
Letter from Sponsor		05 January 2010
Letter from Statistician		15 January 2010
Summary/Synopsis	1.0	01 February 2010
Interview Schedules/Topic Guides	3.0	27 January 2010
Questionnaire: Validated Questionnaire		27 January 2010
Questionnaire: Non-Validated Questionnaire	4.0	27 January 2010
Student CV		01 February 2010
CV - Dr Claire Glasscoe		01 February 2010

CV - Dr Clare Dixon

01 February 2010

Provisional opinion

The Committee would be content to give a favourable ethical opinion of the research, subject to receiving a complete response to the request for further information set out below.

The Committee delegated authority to confirm its final opinion on the application to the Chair.

Further information or clarification required**IRAS form**

- A69 - the dates will need to be changed

Patient Information Sheet (PIS):

- Needs to be clearer regarding the use of the tapes.
- Explain more about the 'moderator' and the 'scribe'.
- Further information required about the use of personal data
- Mention the consent process.
- Provide a separate PIS for the interview group.
- It was stated that you may use quotes and will ask permission to use the quotes; this needs to be mentioned.
- Provide details of a person independent of the study who can be contacted for further information on the study

Consent forms

- Required to have the NRES wording for audit purposes 'I understand that relevant sections of my medical notes and data collected during the study, may be looked at by individuals from regulatory authorities or from the NHS Trust, where it is relevant to my taking part in this research. I give permission for these individuals to have access to my records'.

Consent – for interview form

- Consent form for interviews; clarify or re-word 'moderator' and 'scribe'.

When submitting your response to the Committee, please send revised documentation where appropriate **underlining or otherwise highlighting the changes you have made and giving revised version numbers and dates.**

If the committee has asked for clarification or changes to any answers given in the application form, please do not submit a revised copy of the application form; these can be addressed in a covering letter to the REC.

The Committee will confirm the final ethical opinion within a maximum of 60 days from the date of initial receipt of the application, excluding the time taken by you to respond fully to the above points. A response should be submitted by no later than 01 July 2010.

Membership of the Committee

The members of the Committee who were present at the meeting are listed on the attached sheet.

Statement of compliance

The Committee is constituted in accordance with the Governance Arrangements for Research Ethics Committees (July 2001) and complies fully with the Standard Operating Procedures for Research Ethics Committees in the UK.

10/H1002/10	Please quote this number on all correspondence
--------------------	---

Yours sincerely

Mrs Jean Harkin
Chair

Email: Ronald.Wall@liverpoolpct.nhs.uk

Enclosures: List of names and professions of members who were present at the meeting and those who submitted written comments.

Copy to: Miss Sarah Fletcher, University of Liverpool
D Lambert, R & D, Alder Hey

North West 3 Research Ethics Committee - Liverpool East

Attendance at Committee meeting on 18 February 2010

Committee Members:

<i>Name</i>	<i>Profession</i>	<i>Present</i>	<i>Notes</i>
Dr Paul Baines	Vice Chair / Consultant Intensivist	No	
Mr John Bridson	Lecturer	No	
Mr Colin Bruce	Consultant Paediatric Orthopaedic Surgeon	No	
Mrs Elizabeth Gilkes	Lay Member	No	
Ms Carole Griffith	Physiotherapist Case Manager	Yes	
Mrs Jean Harkin	Chair / Lay Member	Yes	
Mrs G J Hunt	Lay Member	Yes	
Mr Ed Ladusans	Consultant Cardiologist	No	
Dr Omnia Marzouk	Consultant Paediatric A&E Medicine	No	
Professor Neil Pender	Professor of Orthodontics	Yes	
Dr Richard Sarginson	Consultant (Anaesthesia/PICU)	Yes	
Dr Peter Walton	Lay Member	Yes	

Written comments received from:

<i>Name</i>	<i>Position</i>
Ms Carole Griffith	Physiotherapist Case Manager
Mrs G J Hunt	Lay Member
Professor Neil Pender	Professor of Orthodontics
Dr Richard Sarginson	Consultant (Anaesthesia/PICU)
Dr Peter Walton	Lay Member

Attendance at Committee meeting on 03 March 2010

Committee Members Present:

<i>Name</i>	<i>Profession</i>
Mrs Jean Harkin	Chair / Solicitor
Mr John Bridson	Lecturer
Dr Peter Owen	Retired Lecturer
Mrs M Hendry	Pharmacist
Ms Carole Griffith	Physiotherapist Case Manager
Mrs G J Hunt	Solicitor
Mr Ed Ladusans	Consultant Cardiologist

7.4.6. Approval Letter from Research Ethics Committee – Liverpool East

North West 3 Research Ethics Committee - Liverpool East

Bishop Goss Complex
Victoria Building
Rose Place
Liverpool
L3 3AN

Telephone: 0151 330 2051
Facsimile: 0151 330 2075

09 March 2010

Dr Kevin W Southern
Alder Hey Children's Hospital
Institute of Child Health
Eaton Road
Liverpool
L12 2AP

Dear Dr Southern

Study Title: Does administering a parent reported outcome measure during the annual review improve the self efficacy of the carer of a child who has cystic fibrosis? A pilot study.
REC reference number: 10/H1002/10
Protocol number: 7.0

Thank you for your letter of 09 March 2010, responding to the Committee's request for further information on the above research and submitting revised documentation.

The further information has been considered on behalf of the Committee by the Chair.

Confirmation of ethical opinion

On behalf of the Committee, I am pleased to confirm a favourable ethical opinion for the above research on the basis described in the application form, protocol and supporting documentation as revised, subject to the conditions specified below.

Ethical review of research sites

The favourable opinion applies to all NHS sites taking part in the study, subject to management permission being obtained from the NHS/HSC R&D office prior to the start of the study (see "Conditions of the favourable opinion" below).

Conditions of the favourable opinion

The favourable opinion is subject to the following conditions being met prior to the start of the study.

Management permission or approval must be obtained from each host organisation prior to the start of the study at the site concerned.

For NHS research sites only, management permission for research ("R&D approval") should be obtained from the relevant care organisation(s) in accordance with NHS research governance arrangements. Guidance on applying for NHS permission for research is available in the Integrated Research Application System or at <http://www.rdforum.nhs.uk>.

Where the only involvement of the NHS organisation is as a Participant Identification Centre, management permission for research is not required but the R&D office should be notified of the study. Guidance should be sought from the R&D office where necessary.

Sponsors are not required to notify the Committee of approvals from host organisations.

It is the responsibility of the sponsor to ensure that all the conditions are complied with before the start of the study or its initiation at a particular site (as applicable).

Approved documents

The final list of documents reviewed and approved by the Committee is as follows:

Document	Version	Date
Covering Letter		31 January 2010
REC application	1.0	01 February 2010
Protocol	7.0	27 January 2010
Investigator CV		01 February 2010
Participant Information Sheet	3.0	08 March 2010
Participant Consent Form	5.0	08 March 2010
Letter of invitation to participant	1.0	01 January 2010
GP/Consultant Information Sheets	1.0	01 January 2010
Letter from Sponsor		05 January 2010
Letter from Statistician		15 January 2010
Summary/Synopsis	1.0	01 February 2010
Interview Schedules/Topic Guides	3.0	27 January 2010
Questionnaire: Validated Questionnaire		27 January 2010
Questionnaire: Non-Validated Questionnaire	4.0	27 January 2010
Student CV		01 February 2010
CV - Dr Claire Glasscoe		01 February 2010
CV - Dr Clare Dixon		01 February 2010
Participant Information Sheet: Interview Only	1.0	08 March 2010
Participant Consent Form: Interview	2.0	08 March 2010
Response to Request for Further Information		09 March 2010

Statement of compliance

The Committee is constituted in accordance with the Governance Arrangements for Research Ethics Committees (July 2001) and complies fully with the Standard Operating Procedures for Research Ethics Committees in the UK.

After ethical review

Now that you have completed the application process please visit the National Research Ethics Service website > After Review

You are invited to give your view of the service that you have received from the National Research Ethics Service and the application procedure. If you wish to make your views known please use the feedback form available on the website.

The attached document "*After ethical review – guidance for researchers*" gives detailed

guidance on reporting requirements for studies with a favourable opinion, including:

- Notifying substantial amendments
- Adding new sites and investigators
- Progress and safety reports
- Notifying the end of the study

The NRES website also provides guidance on these topics, which is updated in the light of changes in reporting requirements or procedures.

We would also like to inform you that we consult regularly with stakeholders to improve our service. If you would like to join our Reference Group please email referencegroup@nres.npsa.nhs.uk.

10/H1002/10

Please quote this number on all correspondence

Yours sincerely

Mrs Jean Harkin
Chair

Email: Robyn.duncan@liverpoolpct.nhs.uk

Enclosures: "After ethical review – guidance for researchers" [SL-AR1 for CTIMPs, SL- AR2 for other studies]

Copy to: Miss Sarah Fletcher, University of Liverpool
Dot Lambert, R&D, Alder Hey
Latifa Patel, Medical Student, University of Liverpool

The following is a list of publications derived from this thesis:

Glasscoe C, Quittner AL, Patel L, Dale C, Burrows EF, Hope HF, Lancaster GA, Southern KW, Nunn AJ. Components of risk associated with home intravenous antibiotic therapy (H-IVAT) in the UK. *J Cyst Fibros* 2010;9(1):S105

Dyer KL, Glasscoe C, Hope H, Patel L, Southern KW. The impact of pulmonary exacerbation on the challenge of living with cystic fibrosis. *J Cyst Fibros* 2010;9(1):S100

Patel L, Glasscoe C, Dixon C, Dyer K, Southern K. Does administering a parent reported outcome measure during the annual review process improve the self efficacy of the carer of a child with cystic fibrosis? *Ped Pulmon* 2010

“The trouble with research is that it tells you what people were thinking about yesterday, not tomorrow. It's like driving a car using a rear view mirror.” - Bernard Loomis