Collaborative Research Protocol to Define Patient-Reported Experience Measures of the Cystic Fibrosis Care Pathway in France: The ExPaParM Study

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Research Article

Keywords: Collaborative research, Patient partnership, Patient Experience, Patient-Reported Experience Measures, Cystic Fibrosis care, Quality Improvement.

Posted Date: November 15th, 2021

DOI: https://doi.org/10.21203/rs.3.rs-1055675/v1

Title *(19 words)*

Collaborative research protocol to define *Patient-Reported Experience Measures* of the cystic fibrosis care pathway in France: the ExPaParM study

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Abstract *(342 words)*

Introduction

In France, the cystic fibrosis (CF) care pathway is coordinated by multidisciplinary teams from specialised CF centres or transplant centres. It includes the care provided at home or out of hospital, risk prevention in daily life and adjustments to social life, which together contribute to the person's quality of life. Patient experience is used to describe and evaluate the care and life of patients living with the disease.

Objectives

Our collaborative research aims to identify the most significant areas and criteria that characterise the CF pathway. It will lead to the development of a questionnaire to collect patients' experience, which can be administered to all patients or parents of children registered and followed in the centres. The article describes the protocol developed in partnership with patients and parents of children living with the disease.

Method

A multidisciplinary research group brings together researchers, patients, parents of children with CF and health care professionals. The patient partnership is involved in the 4 phases of the protocol: 1) setting up the study, recruiting patient and parent co-researchers, training them in qualitative research methods, defining the situations and profiles of patients in the study population, elaborating the protocol; 2) selecting the study sites, recruiting participants, carrying out semi-structured interviews, analysing verbatims using the grounded theory approach; 3) co-elaborating *Patient-Reported Experience Measures* (PREM) questionnaires adapted to the 4 types of participants: parents, adolescents, non-transplanted adults and transplanted adults; 4) validating the construct with participants and professionals from the study centres.

Results

The protocol obtained a favourable opinion from the Ethics Evaluation Committee of INSERM (IRB00003888 - no. 20-700). Training was provided to the 5 patients and 2 parent co-researchers to enable them to participate effectively in the research. Eleven centres participated in the recruitment of participants in mainland France and Reunion Island. Eighty hours of interviews were conducted.

Discussion

The PREM questionnaires will have to undergo psychometric validation before being used by the actors of the CF network to assess the impact on the care pathways of quality approaches or new therapies available in cystic fibrosis.

Keywords
Collaborative research, Patient partnership, Patient Experience, Patient-Reported Experience Measures, Cystic Fibrosis care, Quality Improvement.

Declarations

Trial Registration Registry: IRB00003888 – no. 20-700. Issue date: 06/09/2020

Consent for publication: Not applicable

Availability of Data: Not applicable

Competing interests: The authors declare they have no competing interest

Funding:

- Funding was provided by the Institute of Research in Public Health (IReSP) for the entire project in the frame of the call for project "AAP General 2018" (Decision IReSP 16/07/2019): €197,320.
- Funding was provided by the patient association *Vaincre la Mucoviscidose* (VLM) for the training of patient and parent co-researchers (Decision VLM 22/06/2019): €9,360.

Authors' contribution:

PL is responsible for the project and participated in writing the manuscript. DPB is the study coordinator and was a major contributor in the manuscript. AF contributed largely to the methodology of this qualitative research. GR, CL, QR are physicians-expert in CF care and reviewed the manuscript. ST is a Nurse specialized in Patient Therapeutic Education and contributed to the research conducting interviews and involved in the qualitative analysis. JH and PM contributed to the methodology and participated in the phases of the protocol. AC, NLB, CHR, FM, MS, DS, CF participated in the design of the protocol and the qualitative analysis. NLB, CF, MS, FM conducted interviews with the study participants. All authors read and approved the final manuscript.

Acknowledgments to:

- Kathy Sabadosa, Karen Homa, Pamela Mertz from the US Cystic Fibrosis Foundation for their support in the preparation of the study, their participation in project meetings and discussions about the results from the US Patient and Family Experience survey.
- *Vaincre la Mucoviscidose* for their support, including financial subsidies and their contribution to the recruitment of patients and parents as co-researchers.

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INTRODUCTION/BACKGROUND *(2216 words)*

Patient Experience (PE) has increasingly been taken into account in recent years to evaluate the quality of care through the experiences reported by patients, in addition to patient satisfaction surveys.[1 2 3] Patient experience is a concept that originates from the USA, promoted by the Beryl Institute[4] and widely disseminated by the Patient Experience Journal.[5] PE is defined as *all the interactions and situations experienced by a person or their family or caregivers during the course of their care. These interactions are shaped both by the organisation of this care pathway and by the person's life history* (definition from the French Patient Experience Institute adapted from the Beryl Institute's[6]). This definition is based on the sum of interactions between the patients and their health care system, according to the organisation of their care pathway, and combines an objective approach based on facts and a subjective approach of their experience in these different circumstances. Improving patient experience, as both *a rational and emotional perception specific to each user*,[7] is gradually becoming a challenge and an objective in order to ensure the quality of care. The assessment of quality of care by patients is an outcomes indicator, in the same way as clinical effectiveness or safety of care, which are assessed by professionals. In order to make this concept operational, PE questionnaires were developed to obtain results that can be acted upon, with the aim of improving the quality and safety of care. These questionnaires, named PREM (Patient-Reported Experience Measures), are intended to be completed by patients without the intermediation of health professionals.[8] They can be used to improve the quality of services within a health care organisation, to compare results between several organisations (benchmarking) or to allocate resources to certain organisations, services or professionals (pay for performance) according to the results observed. Depending on the objectives set, generic questionnaires (quality of hospital catering services, evaluation of hygiene measures or reception on arrival in the establishment) or questionnaires that are specific to certain care pathways or pathologies are used. The Picker Institute Europe database offers validated questionnaires covering seven general themes (sharing of information, coordination of care, physical comfort during hospitalisation, emotional support and respect, respect for patients' preferences, involvement of friends and family, continuity of care) as well as tools specific to certain pathways such as the transition to adult care or pathologies such as sickle-cell anaemia.[9]

However, various criticisms have emerged about these tools, particularly in the case of chronic diseases. These criticisms raise questions about the process of constructing PE surveys, their sensitivity and the degree to which they are used by stakeholders to evaluate and guide measures to improve the quality of care. **On the one hand**, for patients living with a chronic disease, PE develops over the course of their relationship with the disease, which is seen as the patients' pathways of care, health and life in their social environment.[10] This pathway begins around the time of diagnosis and when the disease is announced to the patient, and it includes the successive management by different actors and in different health care or medico-social establishments or structures. However, diagnosis is not always the

beginning of this pathway, especially in the case of rare diseases in which situations of diagnostic wandering can be an integral part of the person's care and life pathways (or the absence of care). Patient experience is also contributed to by that of their family members, particularly those who "naturally" take on the role of caregivers and participate in the patient's care and in improving their quality of life. However, the tools used to collect PE rarely question all the events along this pathway, nor the patients' quality of life, their study or working conditions, but rather particular episodes that are considered to be critical (surgery and postoperative rehabilitation) or subject to a priority care policy (paediatric to adult care transition). This causes the aspects that are ignored to remain unnoticed and PE to be underused to improve patients' pathways. These criticisms also point to the lack of involvement of patients or their representatives in the development of PE collection tools, which was echoed in the report "Being a Patient" published by The Patients' Association.[11] Ways to better take into account the needs of patients in the evaluation of their experience were suggested, such as: including social needs beyond care and focusing on the patient's life with the disease; focusing on the patient's experience rather than on the service provided by health care providers; using new methodologies to discover new aspects of the patient's experience; taking into account the impact of the disease on patients health outcomes when evaluating their experience. **On the other hand**, several publications question the use of the results and the actual usefulness of Patient Experience Surveys to improve the quality of services provided to patients.[12 13] Regardless of how PE is collected, the authors emphasise the need for a clear understanding of patient outcomes and expertise in quality improvement approaches to achieve service improvement. The availability of PE results is not sufficient to drive the changes needed to improve services. The necessary conditions to implement quality improvement initiatives successfully have been widely identified, including the culture of the organisation, leadership style and patients' level of commitment.[14 15] An extensive study conducted by the DUQUE consortium showed that institutional quality management strategies have little impact on generic PREM scores across various European countries.[16] The reasons are diverse: quality management strategies may have only been partially implemented; these strategies may not directly affect PE, which could be more sensitive to direct patient-clinician interactions, or due to the fact that not all patients would benefit from them; and, more fundamentally, this "*loose coupling may reflect a situation where hospitals created a 'facade' of quality management strategies to attract recognition, funding, patients, and status, while not successfully pursuing their implementation".*[17]

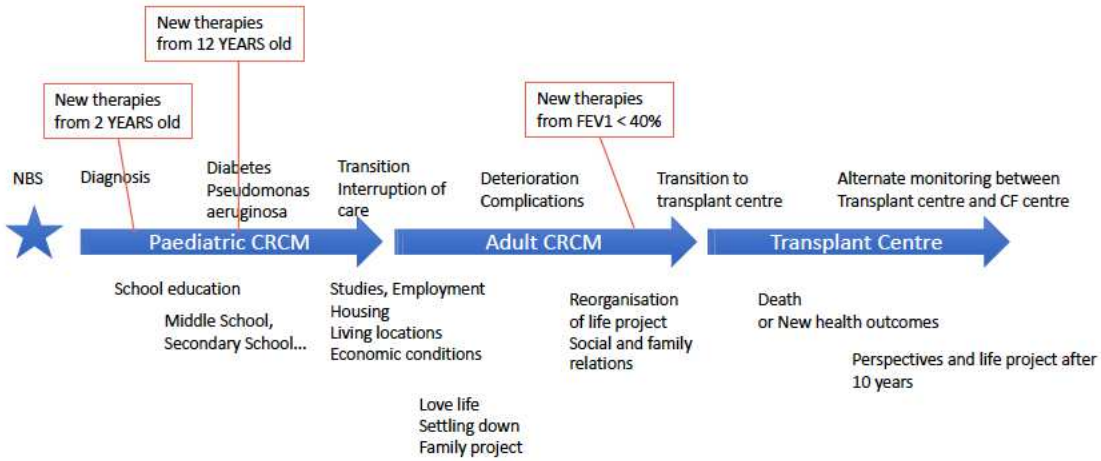
Our research takes place in a rare disease network in France that is structured around paediatric, adult or mixed CF centres and transplantation centres for lung transplant patients. This study is the continuation of a collaborative approach to improve the quality of care carried out between 2011 and 2019, replicated in France from the *Quality Improvement Program (QIP)* deployed by the Cystic Fibrosis Foundation in the USA.[18] This QIP involved patients and parents of children with CF from diagnosis to the implementation and evaluation of improvement actions.[19] The involvement of patients and parents was evaluated in terms of its perceived usefulness in improving care.[20] Questions were raised

by these patient and parent partners[21] about their participation in the process. The first question raised was their fear of not being able to bring into the discussion all the situations and difficulties faced by all patients followed at the centre and that this would lead to a bias in prioritisation of improvement actions, for example to the detriment of socially disadvantaged groups (matter of representativeness). The second question raised was their concern whether all patients would benefit from the improvements implemented as a result of the QIP (matter of equity). These questions highlighted the need for an instrument that can both describe the experience of care and life pathways and be sensitive to the improvement actions implemented. The aim of our study is to create cystic fibrosis PREMs to get a better understanding of the relevant actions required to improve the care and lives of the greatest number of the people living with CF. The design chosen was that of collaborative research, in order to involve representatives of people living with CF together with care professionals in the development of PREMs. Collaborative (or participatory) research offers a collaborative model for studying societal issues by bringing together academic researchers, professionals who act on these issues, and the people affected, including patients when it is related to health matters. Increased levels of participation among patients enhance the relevance and quality of the results, while increased levels of participation among professionals enhance the uptake, sustainability and transferability of improvement programmes developed as a result of the research.[22 23] One of the challenges faced is to ensure that the people concerned are represented on the committee of researchers to guarantee their ability to participate effectively in all stages of the research, and to ensure that the committee integrates their experiential knowledge with the clinical knowledge of professionals and the academic knowledge of researchers, while guaranteeing the scientificity of the research.[24] The necessary patient education to achieve the best possible input from those involved was described in detail elsewhere. [25] The objectives and educational tools of the training, the modalities of collaboration between patients and researchers during the collection and analysis of the data, and the detailed documentation of the study process aim to guarantee the quality and scientificity of the research.[26 27 28] Finally, the results are checked with the study population in order to ensure their reliability and to validate the construct, beyond its co-construction with the patient and parent partners in the research. Thus, the ExPaParM collaborative study sets out to produce cystic fibrosis PREMs in order to gather the experience of the greatest number of patients and parents experiencing diverse circumstances in terms of their care, health and life situations.

METHODS *(4339 words)*

The study focuses on the experience of patients in various care and life situations throughout the cystic fibrosis pathway, in the context of their care at a CF Centre, in hospital, out of hospital, and in their normal living environment. It will be undertaken inductively[29] with a sample of patients and parents of minor patients. The methodology is based on a descriptive view of the current pathway (Fig. 1) by means of a juxtaposition of cross-sections resulting from investigations carried out with patients and parents of sick children at each stage of the pathway. Four groups are identified along the cystic fibrosis pathway: parents of children with cystic fibrosis from birth (median age at diagnosis: 1.1 months) to 15 years of age, adolescents between 16 and 18 years of age followed in a paediatric CF centre, thus preparing their transition to adult care, non-transplanted adult patients followed in an adult CF centre, and transplanted adult patients followed in a transplant centre for post-transplant care. Therefore, our study does not constitute a longitudinal analysis of biographical patient pathways. This methodological choice serves the construction of a PREMs instrument to describe and evaluate the current care and living conditions with the disease, in order to improve them. This cross-sectional approach is consistent with the presentation of the health outcomes in the French Cystic Fibrosis Registry, which also reflects the therapeutic progress and changes in the conditions of care for this disease over the last 40 years. The period chosen for the survey of patients and parents is therefore the last 18 months, complemented by the experience of transitions that have occurred in the last 3 to 5 years (Diagnosis, Adolescent-Adult Care Transition, Transition to transplant).

Figure 1: Diagram of the cystic fibrosis pathway from neonatal screening to post-transplant care



Collaborative research

This research is conducted using a collaborative approach[30] involving health professionals, researchers, patients, and parents of minor patients in a multidisciplinary research group, all of whom are referred to as co-researchers. Patients and parents of children with CF were integrated into the research group following a recruitment process organised with the association *Vaincre la Mucoviscidose*

through a call for interest on social media. Volunteers were recruited based on their CV and a motivation letter and following an interview with academic researchers. The candidates had to meet certain prerequisites to be eligible for the first-year master's degree level of training in qualitative research methods that is planned to be provided to them. Compensation for the time spent on the project is provided in addition to the reimbursement of their travel expenses.

Main objective of the study

- Defining a questionnaire to collect patient experience of the cystic fibrosis pathway (PREM) and the methods for its administration, allowing patients and parents of children to evaluate their care pathway and living conditions with the disease.

Secondary objectives of the study

- Identifying domains and quality standards to evaluate the cystic fibrosis pathway, based on the experience of the patients and parents surveyed, throughout the cystic fibrosis pathway and in various situations when living with the disease;
- Highlighting the differences between the patient experience questionnaire (PREM) developed during the study and the international questionnaires available for this condition.

Study design

The research is based on a qualitative approach[31] in four phases.

PHASE 1: Setting up the research

1.1. **Creating a multidisciplinary group of co-researchers**[32] including patients and parents of sick (minor) children, in charge of contributing at different stages of the study: validation of protocol tools, submission to ethics committee, data analysis methods, synthesis of the results, elaboration of PREM questionnaire, valorisation of research.

1.2. **Research training for patient/parent co-researchers** by researchers from the LEPS/USPN. This way, patient and parent co-researchers can learn new skills throughout the research process on:

- drafting a protocol: defining participant inclusion criteria, elaborating interview guides based on participant profiles, drafting information forms;
- submission procedure to the ethics committee according to the research methodology (RM) used;
- conducting mock semi-structured interviews with an academic researcher;
- thematic analysis of interviews with an academic researcher;
- elaborating and testing PREM questionnaires based on the themes resulting from the analyses;
- getting the questionnaires completed and processing the patients' and parents' answers;
- validating the construct with the participants and teams of the centres involved in the study.

During this phase, the different ***situations*** experienced by cystic fibrosis patients or their parents along the paediatric and adult care pathways were identified using the nominal group technique[33], which makes it possible to extrapolate from the situations described by the people concerned, patients and

professionals. The *situations* experienced by the population to be studied were defined according to different variables: health status, age, transplant status, socio-cultural determinants, where they live (mainland France and overseas departments, urban setting or countryside), type of centre for follow-up care. These were then translated into participant *profiles* (Table 1), complemented by the necessary *family, socio-professional and geographic criteria* to ensure that the situations in the sample of participants were representative (Table 2).

Table 1: ***List of profiles***

Paediatric:
P1 = patient between 0 and 5 years
P2 = patient between 6 and 11 years
P3 = patient between 12 and 15 years
P4 = patient > 15 years and < 18 years (adolescents)
P5 = patient who has changed CF centre in the last two years
P6 = patient who received a new *CFTR* protein therapy
P7 = minor patient diagnosed with COVID-19
P8 = minor patient in the paediatric transplant process
Adults:
P10 = patient who came to the adult centre following transition of care between 18 and 22 years of age
P11 = patient with late diagnosis in the last 4 years
P12 = 22–26-year-old patient stabilised at CF centre
P13 = patient with complications and progression of disease severity
P14 = patient travelling abroad for >3 months in the last 3 years
P15 = patient planning to have a child or in the process of MAP
P16 = patient with children
P17 = patient who received a new *CFTR* protein therapy
P18 = adult patient diagnosed with COVID-19
P19 = patient without major complications following transplant
P20 = patient with long-term complications following transplant

Table 2: ***Additional criteria for the patient and parent sample***

Geographic data

- Distance between home and centre:

□ 0-5 km □ between 5 and 20 km □ between 20 and 50 km □ over 50 km

- Duration of travel (hours, minutes) from home to the centre (*Hour, minutes*):

Family characteristics

- Language spoken in the family of the child or adult patient:
- Marital status of the patient or parent responding to the survey:

□ single □ married or de facto relationship □ divorced or separated □ widower/widow

- Number of children living in the home:
- Siblings with cystic fibrosis (*number of children*): ...
- Other medical conditions in the family (*name the condition*): .....

Socio-professional characteristics

- Employment status of the patient or parent responding to the survey:

□ salaried worker □ no professional activity

□ self-employed □ on disability benefits

□ student □ retired

- Socio-professional category of the patient or parent responding to the survey:

□ farmer or farm operator □ intermediate profession

□ craftsman or trader □ employee

□ executive or intellectual profession □ worker

□ student

- Education level of the patient or parent responding to the survey:

□ no diploma □ undergraduate degree

□ French certificate of general education □ master's degree

□ French vocational qualifications (CAP-BEP) □ PhD level

□ French general baccalaureate or professional baccalaureate

Specific criteria

□ activities with associations □ other:

1.3. Drafting of the detailed study protocol by the group of researchers and co-researchers, including interview guides for each patient or parent profile, for submission to and approval by the

INSERM ethics review committee before recruiting the study population. These guides were pre-tested with patient and parent co-researchers.

PHASE 2: Collecting patient experience from study participants

2.1. **Selecting the study sites**: CF centers were contacted if their patient population was likely to meet the inclusion criteria and if they were expected to accept to participate in the study.

2.2. **Recruiting the study population**: the recruitment process was explained to the physician and a healthcare provider participating in each associated centre during two videoconferences, one on paediatric care and one on adult care. Each CF centre team suggested a list of eligible patients by profile, by filling in the data relating to the complementary criteria for each patient likely to participate (Appendix A). Upon receipt of all the lists, the data were harmonised nationally by the research project's scientific coordinator in order to ensure the representation of all complementary criteria in the various profiles.

2.3. **Collecting data from patient experience** during interviews conducted by academic researchers and by patient or parent co-researchers and transcribing their verbatim. Following the withdrawal of some participants and in order to achieve data saturation, the recruitment was completed using the lists suggested by the centres in order to ensure the diversity of situations investigated.[34]

2.4. **Data analysis using an inductive approach**[35] in the qualitative analysis of interviews, using the grounded theory approach, conducted jointly by the academic researchers and the patients and parents in the research group.[36] The coding of each interview using NVivo® by a researcher, in collaboration with a patient or parent, allowed the identification and aggregation of units of meaning. The phased approach allows for the assessment of data saturation and the adjustment, if necessary, of the number of interviews required in a participant profile. The coding tree obtained by participant profile (parents, adolescents, non-transplanted adults, and transplanted adults) resulted in the characterisation of domains and criteria of PE. The results of the interview analyses were shared at a meeting attended by the entire research group, including care professionals, to report on the domains of patient experience related to care and to living with the disease, as well as the main transitions in the pathway.

2.5. **Putting the results into perspective** with the domains and items of the *Patient and Family Experience of Care* (PFEC) survey conducted in the USA in order to examine the similarities and differences with the domains and criteria highlighted by our research, which may be related to differences in care, health care systems or study methodology. A meeting with the extended research group, including the US CFF researchers, is planned to discuss these differences and similarities and to identify points of convergence between the questionnaires, and in particular, the potential benefit of defining common scores.

This phase should lead to the results being published in an international peer-reviewed journal and presented in a scientific conference, as well as to a communication via the French cystic fibrosis network, particularly via associations.

PHASE 3: Development of the Questionnaire to collect Patient Experience of their pathways of care, state of health, and life with cystic fibrosis.

3.1. **Development of the PREM questionnaire** based on the domains and criteria described in Phase 2. The criteria of PE in the different domains are broken down into items with a suitable response format (free response, response scale for each item: 4-degree Likert scale, or a predefined list of answer options). A customisation of the questionnaire allows the targeting of each of the 4 audiences (parents of children under the age of 15, adolescents between 15 and 18, non-transplanted adult patients and transplanted adult patients) either at item level (questions) or in the answers suggested, to ensure the relevance of the questions (for example, the state of health and type of care required evolve with the patient's age, as well as their employment or study conditions). The questionnaire for each targeted audience is tested and amended with patient and parent co-researchers to ensure that the terms and response formats used are relevant and comprehensible. The questionnaire includes items to assess whether it is understandable and useful to respondents. The questionnaire and its administration process must be approved by the Ethics Review Committee before being sent to study participants.

3.2. **Completion of the online questionnaire** developed with Lime Survey via a link sent by email to the study participants. The data collected will be analysed by the research group and a synthesis of the results will be made available to the teams of the associated CF centres. This is a preliminary step before the evaluation of construct validity of the Questionnaire.

3.3. **Putting the questionnaire into perspective with other questionnaires used** in the USA and in Europe[37 38] to highlight the common points as well as the specificities of the French questionnaire.

PHASE 4: Construct validity of the Questionnaire

This phase aims to establish the construct validity of the Questionnaire according to the criteria of usefulness and appropriation for the different audiences targeted:[39]

1) for patients: useful in evaluating their own patient pathway in all its various dimensions, through the inclusion of specific questions at the end of the questionnaire;

2) for the care teams and the QI coordinators in the CF centres: useful in understanding the main elements of patient experience of the cystic fibrosis pathway and the appropriation of the results to improve the quality of care and services for patients;

Focus group discussions including the CF centre teams, and based on the synthesis of the responses, will assess the usefulness of the Questionnaire to them. Psychometric validation of the questionnaire was not performed for this study.

Study population

The number of patients to be included was estimated by patient profile using purposive sampling to achieve data saturation.[40] The number of targeted inclusions is 57 participants divided into: 20 parents (including 7 parents of children <5 years old (P1), 7 parents of children between 6 and 11 years old (P2), 6 parents of children between 12 and 15 years old (P3)), 6 adolescents between 16 and 18 years old (P4), 19 non-transplanted adult patients (including 6 patients between 18 and 22 years old (P10), 2 patients with late diagnosis (P11), 6 patients between 23 and 30 years stabilised at the CF centre (P12), 5 patients having evolved to a severe state (P13)) and 12 transplanted adult patients (6 patients without major complications (P19) and 6 patients with major post-transplant complications (P20)). This distribution approximates that of the French Cystic Fibrosis Registry (RFM - 2019 Data)[41] whose total population of 7,200 patients includes 58% (4,192) of adult patients. It gives significant importance to transplanted patients (about 24% (900) of adults in the RFM) due to the complexity and diversity of these pathways. No compensation is planned for study participants.

Patient exclusion criteria

- Patients who cannot be interviewed in English or French.

Recruitment process

Recruitment of the study sample was conducted by the partner centres. The following steps were taken in the recruitment process:

1. an anonymised list of participants was suggested by the centres to the scientific coordinator of the study for the different patient profiles;
2. the lists suggested were consolidated by the coordinator to balance the number of individuals between the different profiles and according to the complementary socio-demographic criteria, on a national level;
3. following this adjustment, the coordinator informed the centres of the candidates to be recruited from the list proposed. The individuals who were not recruited were kept as a list of potential participants;
4. the recruitment process was then undertaken by the centres, including informing the participants and providing information materials. It is worth noting that teenagers from the age of 15 were included in person, whereas the parents were included in the case of children under the age of 15;
5. once a participant was included by a centre, their contact details were transmitted to the scientific coordinator of the study via a secure messaging system to enable them to contact the participant by e-mail or telephone to schedule the interview; their consent to participate in the study was obtained at the beginning of the recording of the interview with the researcher;
6. the participants in the study were asked by the researchers to take part in a semi-directive interview lasting about 1.5 hours (phase 2) and then, to answer the questionnaire and give their point of view

on its usefulness for evaluating their own patient pathway of care and their experience of living with the disease, using the specific questions provided for this purpose (phase 3).

Data collection

The interview guide is structured in five parts to address the recent health status and life situations of patients:

- **an introductory part**, which is common to all participants, reminds the main information on the study, the time period covered (the last 18 months or five years for Transitions), the rules of confidentiality regarding the information entrusted by the participants and the recording procedures used for the purpose of analysing the interviews;
- **a section on cystic fibrosis care and treatment**, including care at the CF centre, at home, in hospital, and out of hospital; this part helps to understand each participant's experience of the organisation of care at the CF centre, the therapeutic education of parents or patients and shared decision making, the continuity of care outside the hospital, care during hospitalisation, the burden of care carried out by the parent or patient themselves, care carried out by out-of-hospital professionals, psychological needs and support, experience with a new *CFTR* protein therapy, and pathway to procreation;
- **a section on life with cystic fibrosis**, including work or study conditions, financial situation and social security benefits, changes in living environment and follow-up centres, housing conditions, social relations, possible experiences of living abroad, expectations of and outlook on research and new therapies available;
- **a section on the Transition phase** before reaching their current stage of the cystic fibrosis pathway: the child's diagnosis of cystic fibrosis (as part of neonatal screening or based on symptoms), the period of arrival at the adult CF centre (following the paediatric to adult care transition or late diagnosis), the deterioration phase and transition to transplant;
- **a section on changes experienced during the COVID-19 pandemic**, including adaptations to their follow-up at the centre, daily living conditions since the beginning of the crisis, information provided on COVID-19 prevention and stress management during the crisis.

The guides include open-ended and follow-up questions adapted to the profiles of the respondents, based on the characteristics of health and complications frequently found in the data of the French Cystic Fibrosis Registry corresponding to the patients' profiles (onset of diabetes in adolescence, frequency of intravenous (IV) treatments according to the patient's health status, gastrostomy, etc.).

Qualitative analysis

The interviews were transcribed and analysed using N'Vivo® in an iterative manner, according to a coding framework that emerged during the process of analysis, for each participant profile: parent, adolescent, non-transplanted adult patient and transplanted adult patient. Coding is done by the researcher, in collaboration with a patient or parent co-researcher. The grounded theory approach allows

the defining of categories without an *a priori* theoretical framework. The coding framework established in NVivo® is articulated in categories, called domains and sub-domains, which emerge from the interviews and the experiences are gathered under these domains.[36]

The grounded theory approach allows the description of each category (= domain): its properties (what it is composed of: sub-domain, items), the conditions of its existence, its various possible forms and dimensions. The questions asked are: *What is going on here? What is it about? What phenomenon am I dealing with?* Relationships between categories can be identified according to the logical or chronological links observed, thus allowing to explain the ongoing phenomenon. The questions asked are: *Is what I observe here related to what I can observe there? In what way and how is it related?* This questioning can be found at two levels: at collection level, the interviewees themselves connect the phenomena in their answers, or at conceptual level, the analysis of the researcher allows to connect the phenomena together. Dynamics may therefore be highlighted: for example, a negative impact on parents' social lives and family relations caused by the dietary recommendations and hygiene precautions given at the time of diagnosis of the child's disease; or an impact on the lives of relatives due to the recovery of the patients' physical capacities after lung transplantation, meaning that they do not need the assistance from caregivers that was required while waiting for a transplant any longer.[36]

Development & validation of the Cystic Fibrosis PREM construct

The development of the questionnaire is based on the domains identified (N'Vivo® categories) by breaking them down into items formatted as questions. For example, "*Continuity of remote patient care*" domain can be broken down into: What are the reasons for contacting the CF centre? How quickly can the team be reached? Patient perception of the professional's consideration for the problem raised? How long does it take to get a response? What types of responses are provided? Scientific recommendations or best practices can be integrated into the wording of items or response proposals for their scoring: for example, a response provided within 24 hours to a telephone call or an e-mail from a patient (maximum score reflecting best practice), or within two days, within one week or more than a week (scoring = 0).

The focus is on the patients' or parents' recent experience (last 12 or 18 months) or the last 3 or 5 years for events related to a transition in pathway (diagnosis of the disease, transition from paediatric to adult care, entering the transplant transition) in order to reflect the current conditions of the patient pathway.

Four questionnaires will be developed, one for each respondent profile (parents of minor children, adolescents, non-transplanted patients, and transplanted patients) to focus more precisely on the characteristics of each pathway. The questionnaires should also be designed in order to avoid unnecessary questions and to limit completion time during its administration. For example, if the patient has not been hospitalised in the past 12 months, the questions about their hospitalisation experience should be skipped. Questionnaires are tested with patient and parent co-researchers experiencing

different pathways in order to assess the response time. The questionnaire can be administered in 3 parts in order to reduce the completion time and improve the response rate. The questionnaire's ease of use will be assessed by the respondents in this study. To this end, additional questions are announced at the beginning of the questionnaire and can be answered at the end about the relevance of the domains, how comprehensible they are, and how appropriate and useful they are in relation to one's pathway, in order to allow improvements to be made.

Questionnaires used in other countries[42 43] will be taken into account with regard to the domains and the items, particularly by applying score calculations by domains of care. For example, certain scores are calculated in the American survey and used for benchmarking US CF centres as part of the national quality approach: a score for the *control of cross-infections* (**Infection Control**) during hospital visits, a score for *collaboration between the patient or parent and health care team* (**CollaboRate**)[44], a *coordination score between caregivers* (**IntegRate**) and a *global care score* (**HolisticRate**) including patients' or parents' psychological and social-economic aspects.

Construct validation is based on the study participants' responses to the questionnaire. On the one hand, the answers will be processed as for a routine use of the questionnaire by consolidating the results by item and category. These results will be put into perspective with the results obtained from interviews conducted with the same participants in Phase 2 of the study in order to assess their capacity to report on the patients' or parents' experience of their pathways. On the other hand, the answers to the questions on the questionnaire's usefulness and ease of use will help to suggest improvements, such as simplifying or rewording certain parts, or to its administration process. Finally, all these results will be analysed in a focus group with the teams of the associated CF centres in order to discuss the usefulness of the questionnaire-based survey in evaluating the quality of care and identifying how it can be used by CF centres or the National CF network.

RESULTS *(604 words)*

Recruitment and training of patient and parent co-investigators

Seven patients and parents of minor children were recruited in January 2020 to join the research group: 2 parents of children aged 4 and 9 years; 2 non-transplanted patients (20 years and 30 years); 3 lung transplant patients at different times since transplantation (2 years, 5 years, 15 years). All were informed about the process of the study and the expected level of participation, and their formal consent to participate was obtained. They alone represent a diversity of health and life situations in the cystic fibrosis pathway that has great potential for enriching the thinking process undertaken in the study. The research group includes a total of 7 patients and parents, 4 professionals (3 doctors and 1 nurse) and 5 researchers from two laboratories.

During the course of the study, the group of patients and parents was trained in the use of qualitative research methods and tools developed by the researchers, first in person and then remotely during the pandemic, for a total of 35 hours per person (Appendix B). In addition to theoretical, methodological and ethical contributions, this training included practical applications related to the ExPaParM research project. Mock interviews enabled the future co-researchers to appropriate the interview guides in order to conduct interviews with actual participants recruited in the study.

Ethics and Approval

The protocol drafted and revised with the patient and parent co-researchers was submitted to the INSERM Ethics Evaluation Committee and received a favourable opinion during the session held on 9th June 2020.

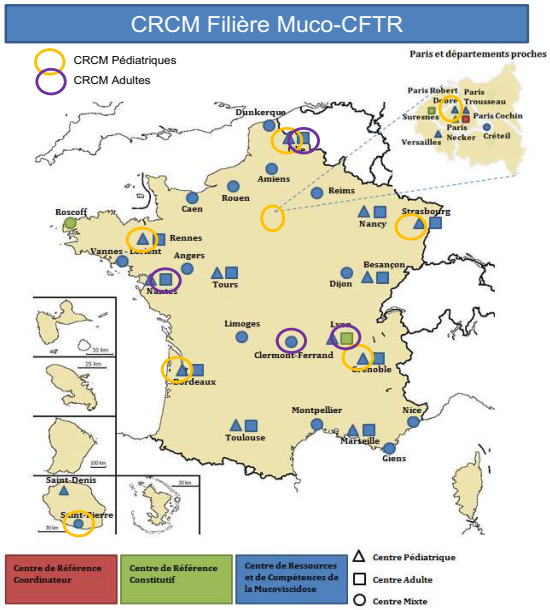
IRB Agreement n° IRB00003888 - Notice n°20-700
Issue date: 9th June 2020

Study Setting

In order to ensure that the complementary profiles and criteria of the study participants were represented, the following CF Centres were contacted and agreed to participate (Fig 2):

- Paediatric Centres: Bordeaux, Paris R. Debré, Grenoble, Lille, Rennes, Saint-Pierre La Réunion, Strasbourg
- Adult Centres: Clermont-Ferrand, Lille, Lyon, Nantes

Figure 2: Geographical distribution of partner centres of the study



Inclusions

The recruitment process according to participant profiles led to the inclusion of 67 participants from August 2020 to April 2021: 20 parents of children <15 years of age, 11 adolescents between 15 and 18, 25 non-transplanted adult patients and 11 transplanted patients. Due to the diversity of patient experiences collected, we included 11 more patients than planned to achieve our data saturation objective: the complementary lists suggested by the CF centres were therefore used. Of the included patients, 8 tested positive for COVID-19 during the study.

Table 3: Number of Patients involved in the study

CF Centre Investigator	# Patients interviewed (including Ado or Adult TxP)	# COVID+ Patients interviewed
Lille Ped	3 (2)	1 TxP
Paris R. Debré	5 (3)	
Strasbourg Ped	5 (2)	
Saint-Pierre LR	5 (1)	
Rennes Ped	6 (1)	
Bordeaux Ped	3 (1)	
Grenoble Ped	4 (1)	
S/TOTAL Paed.	**31 (11)**	**1**
Lille Ad	9 (3)	2 TxP
Nantes Ad	12 (5)	2 Non TxP
Lyon Ad	7 (2)	1 Non TxP
Clermont-Ferrand	6 (1)	
Other CRCMs	2	2 Non TxP
S/TOTAL Adult	**36 (11)**	**7**
TOTAL	**67**	**8**

Data collection through semi-structured interviews

Given the rules of social distancing due to the pandemic and the geographical dispersion of patients, the interviews, which lasted approximately 1.5 hours on average, were conducted by telephone and were recorded. The 67 interviews conducted by researchers and some of the patient and parent co-researchers represent over 80 hours of recording, for an average duration of one and a quarter hour (Table 4).

Table 4: Average duration of interviews

	total	number	average	mini	maxi
Paediatric	39:34:44	31	1:16:36	42'	2h07'
Adult	44:37:00	37	1:12:21	27'	2h02'

DISCUSSION *(1678 words)*

The aim of this study is to develop cystic fibrosis PREMs in the context of collaborative research involving patients and parents trained in qualitative research, CF centres care professionals, and academic researchers. This study should lead to the development a common construct that will be easily used by the actors of the CF network.

An innovative patient partnership in research

The patient-caregiver partnership is used by different groups or dynamics of the cystic fibrosis network: the national ETP group (GETHEM) since its creation in 2005, the quality improvement programme deployed between 2011 and 2019, the structured committees for the governance of the CF network (medical council, national council, patient/parent group within the VLM Research Department). The ExPaParM project innovates by working on a patient partnership in a research project that includes patients and parents as co-researchers. This partnership enhances the experiential knowledge of patients and parents and develops their knowledge and understanding of the scientific methodology. It responds to their wish to evaluate the patient pathways of their peers in order to find possible improvements that could meet the needs of the greatest number. This partnership aims to improve the quality and relevance of the results of the study in order to achieve the upmost scientificity. In particular, the participation of patients and parents of children in the research group is not a substitute for the validation of the construct by study participants (patients or parents recruited in the centres), which guarantees the credibility of the results obtained from a broader sample of people living with the disease and the appropriation of these results by the professionals, beyond the research group.

As they are aware of the challenges posed by the partnership between patients and health professionals,[45] health researchers have tried to put the concept of partnership into practice in the field of research[46] by developing frameworks for patient engagement in research suitable to their research context.[47] Patients are increasingly involved in different stages or activities of research,[48] from suggesting research themes that are relevant to them, to interpreting data or in the valorisation of results. However, certain difficulties can hinder researchers from implementing this participation or from carrying it out effectively at all stages of the research.[24 49] In our collaborative research, the patient partnership is used to: enrich the research tools and the variety of situations to be investigated; conduct interviews, collect data; participate in the thematic analysis of interviews and in the development of the coding framework; co-construct the PREM questionnaires and test them; participate in the interpretation of results and in their valorisation; promote the project to the relevant authorities. This participation in a research project can contribute to empowering the patients and parents taking part, but certain conditions, for which the academic researchers are the guarantors, must be fulfilled: patients and parents must receive training in research methods using various teaching resources; the partnership must be a space for reflection that allows for the joint elaboration of tools and analyses; the research committee must show them due consideration and be willing to take their suggestions into account; the availability

required of them must be flexible enough to be compatible with their personal and professional activities and their health status; transparency on progress and decision-making during the study must be guaranteed, and their contribution to the study must be recognised. Anecdotally, the time of the pandemic became a positive factor for the patient and parent co-researchers as the setting up of remote communication tools allowed them to continue the work undertaken and they were able to take advantage of the time freed up by the health crisis (partial unemployment, working from home or remote studies). The conditions of collaboration in the study were presented at an international scientific symposium,[50] in which some of the patient partners took part, and which will be the subject of a publication on the contribution of patient partnership in collaborative research. This may contribute to a better understanding of the conditions suitable for patient participation in research and for their personal development in terms of individual empowerment.

Potential limitations

The variety of care and living situations to be investigated led to a sample of participants in the study that is not representative of the frequency of occurrence of these situations in the total population of patients followed in France. As a result, the themes that arise from analysing the interviews cannot be weighted in terms of importance related to their occurrence. No such calculation was retained, although N'Vivo® allows it. All the themes were translated into the PREM questionnaire submitted for construct validation. This choice, which is inherent to the method, allows for the collection of positive or negative experiences that might seem exceptional. On the one hand, some experiences might be more frequent than expected in a larger sample. On the other hand, the questionnaire resulting from the study could be used as a guide to detect specific individual difficulties and needs that are usually not well researched, in the context of a care relationship between medical staff and a patient or in a support relationship.

The PREM questionnaires developed and tested with patient and parent co-researchers may prove difficult to understand and to complete for the study participants, as the recruited co-investigators all had a minimum of a bachelor's degree level of education in order to be able to complete the qualitative research training and participate fully in the research phases. Hence, the purpose of the construct validation phase is to evaluate their comprehensibility and usefulness to the patients and parents included in the study. This validation may lead to modifying the questionnaires in order to obtain a final, more appropriate version at the end of the study.

For large-scale use, subsequent psychometric validation allows the weighting of the themes according to their frequency of occurrence in a large sample, the respondents' understanding of the questions and to reduce the length of questionnaires or ensure they can be used in parts, depending on the objectives sought.

Perspectives

After psychometric validation, the use of the PREM questionnaires developed by the cystic fibrosis network will allow the evaluation of patients' care and life pathways, as well as the assessment of the impact on these pathways of changes in practices related to a quality improvement programme or the introduction of therapeutic or technological innovations (mHealth and telemedicine).

In France, the *Haute Autorité de Santé* (HAS) has conducted a study on PREMs. It aims to produce and implement indicators, particularly in the context of experiments with cooperation protocols between health care actors, and published a report on international experiences in setting up PREMs and the lessons learned on patients' perception of quality of care.[51] Like other studies that use a qualitative approach to PE,[52] ExPaParM proposes a method of co-construction of PREMs based on the patient experience collected from a sample of patients and parents that is representative of a large number of health and life situations in the context of a rare disease. The patient pathways of people living with a rare disease have distinctive characteristics beginning from (and sometimes before) the diagnosis, with multiple transitions and a level of complexity due to the necessary care and to living with the disease that can only be reported by the people themselves and their relatives.[53] PREMs are therefore as much a descriptive tool for developing a typology of patient pathways and potential problem areas, as they are a tool for evaluating the quality of the interventions of various actors in these pathways. In that respect, these PREMs draw from indicators of patient health status collected in rare disease registries or via PROMs, as suggested in the report "Being a Patient". This tool may be used in many different ways to improve the quality of patient pathways: in the context of the individual caregiver-patient relationship, to identify difficulties encountered by the patient in dealing with the disease; at the level of a health care centre, to identify organisational or structural problems, analyse the impact of changes in practices and identify groups of patients who are experiencing difficulties; at the level of a rare disease network, to identify the organisations and practices that contribute to improving patients' experience along their care pathway; at the level of patient associations, to identify the type of support to be offered to groups of patients experiencing difficulties and the areas of advocacy to be developed.

Internationally, the development of PREMs, including quality scores on common themes of cystic fibrosis pathways, would allow benchmarking between countries with different health systems and organisations.

Appendix A - Proposal sheet for applicants

Patient's follow-up CRCM code (Centre's code in the Registry list): ..
Date: .. / .. / ….

Demographics for the survey

- **Patient study code** *(3 letters City + CRCM type: A,,M,P + sequential number) :.....*
- **Patient profile(s)** *(Pxx, multiple profiles possible): ... ; ... ; ... ;*
- Patient's age *(years, months):* ...., .
- Gender of patient *(F or M):* .
- Type of respondent *(patient or parent):* ..... ;
- Gender of responding parent *(F or M)*: .

Geographic data

- Distance between home and centre:

□ 0-5 km □ between 5 and 20 km □ between 20 and 50 km □ over 50 km

- Duration of travel (hours, minutes) from home to the centre (*Hour, minutes*):

Family characteristics

- Language spoken in the family of the child or adult patient:
- Marital status of the patient or parent responding to the survey:

□ single □ married or de facto relationship □ divorced or separated □ widower/widow

- Number of children living in the home:
- Siblings with cystic fibrosis (*number of children*): ...
- Other medical conditions in the family (*name the condition*): .....

Socio-professional characteristics

- Employment status of the patient or parent responding to the survey:

□ salaried worker □ no professional activity
□ self-employed □ on disability benefits
□ student □ retired

- Socio-professional category of the patient or parent responding to the survey:

□ farmer or farm operator □ intermediate profession
□ craftsman or trader □ employee
□ executive or intellectual profession □ worker
□ student

- Education level of the patient or parent responding to the survey:

□ no diploma □ undergraduate degree
□ French certificate of general education □ master's degree
□ French vocational qualifications (CAP-BEP) □ PhD level
□ French general baccalaureate or professional baccalaureate

Specific criteria

□ activities with associations □ other:

Appendix B: Educational programme for patient and parent co-researchers

TRAINING OF EXPAPARM "CO-RESEARCHERS"

→ equivalent UE6 "Questioning research", Master's degree - year 1, Quality and Safety of Care Pathways QSPS

Detail of ExPaParM training:
11 sessions (34.5 hours)

TUESDAY 4 FEBRUARY 2020
At the premises of Vaincre la Mucoviscidose, Paris 13th
9:30 am-5 pm
Programme:
- Presentation of the project and participants
- Situate yourself in the types of commitment required and in the project
- Describe the steps of a research method
- Differentiate between purposes and types of research
- Positioning yourself as a patient in a protocol

WEDNESDAY 5 FEBRUARY 2020
At the premises of Vaincre la Mucoviscidose, Paris 13th
9:30 am-5 pm
Programme:
- Characterizing the Cystic Fibrosis Pathways
- Problematizing a research object and setting an objective
- Building a theoretical framework from a literature review (videos)
- Defining the main tools of quantitative and qualitative research
- Justifying a research project in terms with regard to founding principles

WEDNESDAY 11 MARCH 2020
Remote (Zoom)
9 am-12:30 pm
Programme:
- Interviewing patients based on the situations of the Cystic Fibrosis Pathways
- Writing a research protocol

1

THURSDAY 9 APRIL 2020
Remote (Zoom)
2:30-5:00 pm
Programme:
- Knowing the ethical and regulatory elements of research
- Characterising a search and type of search

FRIDAY 10 APRIL 2020
Remote (Zoom)
2:30-5:00 pm
Programme:
- Reviewing the protocol and future course of the study
- Reviewing the Annexes of the protocol in detail

→ Work required by the end of April

IN THE COURSE OF APRIL 2020
Remote (Zoom), one-to-one
2-hour slots
Programme: Testing out the interview guides

WEDNESDAY 16 SEPTEMBER 2020
Remote (Zoom)
6-8 pm
Programme:
- Progress report on the project
- The main tools of qualitative research: Different types of research, Quality criteria for qualitative research, Data collection method, Triangulation
- The Participant in Qualitative Research: Ethical Issues, Sampling Techniques

THURSDAY 17 SEPTEMBER 2020
Remote (Zoom)
5:30-7:30 pm
Programme:
- Feedback on the interview guide test: Conducting the test, Lessons learned from the test
- Principles for conducting an interview: The interviewer's skills, Technical rules
- The ExPaParM interview guide: according to the different profiles, potential problem areas, practical matters of organisation

2

IN OCTOBER 2020
Remote (Zoom), one-to-one
2-hour slots
Programme: Mock interviews

WEDNESDAY 18 NOVEMBER 2020
Remote (Zoom)
9:30 am to 12:30 pm
Programme:
- Theoretical overview on the content analysis of semi-directive or non-directive interviews
- Presentation by Sophie Thérouanne of her Master's thesis
- Crossed perspectives on qualitative analysis of patient pathways based on patient experience

WEDNESDAY 25 NOVEMBER 2020
Remote (Zoom)
9:30 am to 12:30 pm
Programme:
- Content analysis of ExPaParM interviews: group application work
- Conclusion of the training

3

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