ECFS Patient Registry (ECFSPR)
Terms of Reference

1. Aim

The aim of the European Cystic Fibrosis Society Patient Registry (ECFSPR) is to gather data that are accurate (measured against an internationally agreed standard) and that can be used for the benefit of people with Cystic Fibrosis (pwCF), CFTR-related disorder (pwCFTR-RD) and who were CFSPID (CF Screening Positive, Inconclusive Diagnosis) at birth (pwCFSPID), as defined in the Informed Consent that must be signed by pwCF, pwCFTR-RD, pwCFSPID or their parent/s or legal guardian/s. To achieve this aim the ECFSPR undertakes to:

- Measure and compare epidemiological and clinical aspects of CF, CFTR-related disorder, CFSPID in the participating countries in order to facilitate the development of improved or new standards of care and management;
- Provide data for epidemiological research and identify groups of pwCF, pwCFTR-RD, pwCFSPID who may be eligible for multi-centre trials.

2. Deliverables

a) Data that are of sufficient quality to permit epidemiological research;

b) Annual epidemiological reports to be submitted to the ECFS and made available to the community and the public:

- The annual epidemiological reports will be maintained at the ECFS office and will be published on both the ECFS and ECFSPR webpages;
- The annual epidemiological report will include epidemiological presentation of core data;
- Tables and graphs will show data aggregated on a European level and on individual country level. Data aggregated on individual centre level will be accessible only to the centre's director and to a national registry steering committee in the event that such a recognised organisation exists for the country.

3. Governance

Contributors group:

The ECFSPR Contributors Group is defined as anyone who contributes data to the ECFSPR on behalf of pwCF, pwCFTR-RD, pwCFSPID.

Steering Group:

The ECFSPR Steering Group sets the strategic direction and areas of priority of the ECFSPR. Responsibilities of the Steering Group:

- Ensure the project is aligned with the general ECFS strategy;
- Approve formal collaborations of ECFSPR with external organisations;
- Recommend individuals to serve on the Executive Committee to replace members rotating off. Such recommendations are subject to the approval of the ECFS Board;

- Nominate and elect the ECFSPR Director through a voting process. Nominations and elections are subject to approval by the ECFS Board;
- Ensure the project makes good use of assets;
- Assist in the resolution of strategic level issues and risks;
- Provide advice and guidance on business issues facing the project;
- Use influence and authority to assist the project in achieving its outcomes;
- Review and approve final project deliverables.

The ECFSPR Steering Group will meet twice a year, in conjunction with the European CF Conference in June and in January at the combined ECFSPR and ECFS-CTN meeting.

The ECFSPR Steering Group may create committees amongst its members to focus on specific aspects of the Registry.

The ECFSPR Steering Group is composed as follows:

Voting members:

- ECFSPR Director, chairperson;
- For participating countries from the WHO European Region:
  - 1 national representative per country (a representative of a national CF Registry or an individual from a CF centre/hospital/clinic that has been elected by other centres in a country to represents them, known as the country coordinator or national representative);
  - 2 individuals appointed by Cystic Fibrosis Europe, the European Federation of Patients organisations.

The Steering Group will be augmented by the following non-voting members:

- a statistical expert;
- an expert on the legal and ethical aspects of data protection (on an ad-hoc basis);
- a representative from the ECFS Clinical Trials Network;
- The ECFSPR Operations Manager;
- a “Trusted third party” (an ECFSPR employee who is party to ECFSPR centre codes and centre names. ECFSPR participating centres and countries are anonymised to comply with data protection regulations and to help protect patient privacy).

The ECFSPR Steering Group is chaired by the Director.

The responsibilities of the ECFSPR Director are to:

- Set the agenda for each meeting;
- Ensure that agendas and supporting materials are delivered to members in advance of meetings;
- Make the purpose of each meeting clear to members and explains the agenda at the beginning of each meeting;
- Clarify and summarise what is happening throughout each meeting;
- Encourage broad participation from members in discussion by calling on different people;

- End each meeting with a summary of decisions and assignments;
- Follow-up with absent members;
- Report to the ECFS Board and Annual General Meeting (AGM).

The ECFSPR Director is appointed by the ECFS Board, based on a nomination from the ECFSPR Steering Group. The tenure is of 3 years. In the case of non-compliance with the ECFS Strategy and the responsibilities outlined in the previous paragraph and points above, the ECFS Board has the right to dismiss the Director with a four weeks' notice period. In the last year of tenure the Director will train the new Director to the position. The ECFSPR Director is a co-opted member of the ECFS Board.

Executive Committee:

Responsibilities of the Executive Committee:

- Implement the strategy and policies decided by the ECFS Board;
- Implement the ECFS Board's overall recommendations for change;
- Propose new strategy and policies to the Steering Group;
- Monitor the ECFSPR activities, the operation, and all activities where ECFSPR undertakes legal obligations;
- Reviews report and budgets prepared by the ECFSPR Operations Manager and makes recommendations to the Steering Group;
- Prepare an annual strategic and financial report to be submitted to the ECFSPR Steering Group and subsequently to the ECFS Board for approval.

The Executive Committee is composed as follows:

- ECFSPR Director;
- 3 Members of the ECFSPR Steering Group;
- one representative of the patient associations nominated by CF Europe.
- The following ex officio (non-voting) members:
- ECFS Executive Director;
- ECFSPR Operations Manager;
- one statistical expert;
- one representative of the ECFS Clinical Trials Network;
- one ECFSPR Liaison Officer to the European Platform for Rare Disease Registries (EPIRARE) and other EU initiatives for rare diseases;
- an external legal expert who will act as a consultant to the Executive Committee on an adhoc basis.

Members of the Executive Committee will be appointed for 3 years and on a rotational basis. In order to implement the rotation system, the members of the first Executive Committee will be appointed for either 2 years or 3 years. No Executive Committee member can stand for election for more than two consecutive terms of office.

The Executive Committee is accountable to the ECFS board.

The Executive Committee will hold online meetings twice a month and the minutes will be circulated to the entire ECFSPR Steering Group.

The ECFSPR Operations Manager provides a central role for information exchange, project coordination, management of sensitive timelines and general administration.

Scientific Committee:

All applications to the ECFSPR for data for Research Project will be reviewed and assessed and approved, or refused, by the ECFSPR Scientific Committee. Research Project application forms are to be sent by email to the ECFSPR Epidemiological Research Liaison Manager (coordination@ecfregistry.eu) who will liaise with the Scientific Committee

The data of an individual participating centre will be accessible only to the Director and Centre Administrators of the centre and to the national representative on the Steering Committee (of the country the centre is in). Data extraction for individual centres are thus not subject to Scientific Committee assessment and approval..

Any applications from countries outside of the European Union must demonstrate compliance with an adequate level of data protection as stipulated in General Data Protection Regulation (EU) 2016/79, Articles 3.1, 3.2, 5, and must be approved by the Danish Data Protection agency, before release of data.

The Scientific Committee is composed as follows::

- ECFSPR Epidemiological Research Liaison Manager;
- 6 elected members from the ECFSPR Steering Group, one of whom will be the elected as chairperson;
- one representative of pwCF (individual with CF, parent/guardian of individual with CF);
- two to three statisticians;
- Representative of the Executive Committee which may be the Registry Director;
- Pharmacoepidemiological Studies Director.

The Scientific Committee will review all applications. Applications from Industry will also be reviewed by the ECFS Clinical Trials Network (CTN). Taking into account the recommendations of the Scientific Committee, the ECFSPR Steering Group will decide whether or not to approve the research project application. This decision will be final. For further details on the procedure please refer to the ECFSPR Code of Conduct. All publications will be listed on the publications section of the ECFSPR website. Applications should be scientific projects headed by individual independent scientists.

All associations with private companies, especially pharmaceutical companies, should be acknowledged in the application.

All applicants are also required to sign a Use of Data Agreement. There will be an application fee for research projects. Data extracts will be subject to a fee calculated on hourly cost basis.

For further details on the application process and fees please refer to the ECFSPR Research Project Applications page on the website

4. ECFS CTN and ECFSPR cooperation

The Director of the CTN will be their representative on the ECFSPR Executive Committee. A member of the ECFSPR Executive Committee is appointed as the ECFSPR representative on the CTN Executive Committee.

Research Project applications from the Pharmaceutical Industry (Industry) will be review by the CTN. The process is as follows:

- The CTN Executive Committee assesses the Research Project application submitted by Industry.
- If the CTN Executive Committee approves the application it is reviewed by the ECFSPR Scientific Committee.
- The ECFSPR Scientific Committee has 1 week to explore feasibility, estimate the cost for analysis and approve/refuse the application;

Research Project applications from Industry will be subject to a fee for processing and analysis. There will be an application fee for research projects from the pharmaceutical industry.