The European Cystic Fibrosis Society
Patient Registry
(ECFSPR)

Andreas Jung
ECFSPR Executive Committee

EURODIS Multistakeholder Symposium
February 13th 2019, Brussels
Cystic Fibrosis: a multiorgan disease

- Chronic rhinosinusitis; Sinus infections; Nasal polyps
- Reduced lung function; Frequent lung infections, inflammation, and progressive lung disease; ABPA
- Abnormally high excretion of sodium and chloride (salt) in the sweat; Pseudo Bartter’s syndrome
- Infertility CBAVD in men

- Bile duct obstruction leading to focal biliary cirrhosis
- Exocrine pancreatic insufficiency and resulting malnutrition; Endocrine pancreatic insufficiency (CFRD)
- Failure to thrive / difficulty to gain weight due to pancreatic insufficiency, digestive problems and intestinal blockages (DIOS)

O’Sullivan, Lancet 2009
Westaby, Prog Respir Res.2006
Treating a multiorgan disease

**Respiration**
- Hypertonic saline
- rhDNAse
- Physiotherapie
- Nasal wash / sinus inhalation
- Bronchodilators

**Infection**
- Eradikation
  - Inhal. Tobramycin
- Suppression
  - Inhal. tobramycin, colistin, aztreonam
- Exazerbation
  - i.v. combination, p.o. mono
  - Azithromycin
  - Antimycotis, syst. Steroids

**Intestine**
- Pancreatic enzymes
  - Vitamines (ADEK)
- NaCl substitution
  - Hypercaloric shakes
  - Macrogolium
  - Ursodeoxycholic acid
  - Insuline

**CFTR modifier**
- Ivacaftor
- Ivacaftor + lumacaftor
- Ivacaftor + Tezacaftor
- More to come
Cystic Fibrosis care in Europe

ecfs-ctn@uzleuven.be

Site needs to refer company to the CTN
Site needs to wait notification from CTN before completing the questionnaire
Cystic Fibrosis clinical trials
The European CF Patient Registry

Aim of international patient registries: Getting a good picture of rare diseases

Mission: To collect data to compare aspects of CF and its treatment

Encourage new standards of CF care
Inform public health planning
Enable research
Value of a patient registry

Registry

Patient organisations:  
- Lobby for CF care

Patients:  
- Information outcomes  
- Access new therapies

Healthcare authorities:  
- Information about CF and CF care

Clinicians:  
- Compare with other centres/countries  
- Monitor quality of care

Industry:  
- Prepare for clinical trials  
- Monitor safety & effectiveness new drugs

Research:  
- Insight in the disease  
- Identify disease modifying factors
38 countries
>48,000 patients

Longitudinal data 2008-2016
Software

Data-collection software: ECFSTracker

A platform for the collection of CF data for all purposes

- Online / web-based
- Data-collection once a year, and/or
- Encounter data at each patient visit
- Advanced Security Technology
- Remote updates
- Add-on modules
Cross-comparison of indicators of quality of care (= benchmarks), e.g. lung function, BMI, proportion adult patients

Comparison between:
- Centre vs Country / multiple Countries
- Centre vs other centres within the country (upon approval)
- Years

Direct feedback to centre staff

Identify areas for improvement
Projects

1. Data Quality Group
   Ensure accuracy and quality of data

2. Definitions Group
   Review variables and definitions

3. Global CF harmonisation project
   Armonise variables & definitions to allow comparison worldwide

4. Pharmacovigilance project
   Developing a procedure with EMA

5. Patient awareness project
   Joint effort with CF Europe to bring data closer to patients
Within 18 months after the close of the follow-up year
At-a-Glance Reports

- **Children:** 47.9%
  - **Lung Function:** 90%
- **Adults:** 52.1%
  - **Lung Function:** 67%
- **Pseudomonas:** 30%
- **Median Age at Diagnosis:** 4.3 months
  - **F508del homozygote:** 41%
  - **F508del heterozygote:** 41%
  - **Other mutations:** 18%
Social Media

Facebook

Twitter

ECFS Registry
@ECFSRegistry
Research: data requests

Data-requests 2011 - June 2017

- Industry
- Independent Researchers
- ECFS researchers
Manuscripts in the pipeline

1. International and pan-European comparison of survival in CF
2. Risk Factors for the decline in FEV1 among Patients with CF in Europe
3. Changing Epidemiology of the Respiratory Bacteriology of Patients with CF in Europe
4. Clinical characteristics of CFRD: Lessons from the ECFSPR (HV Olesen)
5. The effect of CFTR nonsense mutations on phenotype and mortality in patients with CF
6. Impact of Dornase Alfa on rate of decline in lung function in patients with CF
   CF-specific reference equations for FEV1 and BMI: an updated analysis
7. Incidence, morbidity and mortality of CF liver disease and cirrhosis

Publications: www.ecfs.eu/ecfspr
Safety and efficacy studies

New drugs on market need to be monitored:

**Post-marketing studies**

2016: Start discussions with European Medicine Agency; ECFSPR model for other rare diseases

2018: Qualification of ECFSPR as appropriate platform for the collection of data for long-term safety and efficacy of new therapies (post-authorisation safety surveillance (PASS) und efficacy (PAES) studies)
Partners & Sponsors

- CF Europe
- ECFS
- Università degli Studi di Milano
- Gilead
- Vertex
Contact

www.ecfs.eu/ecfspr

ecfs-pr@uzleuven.be
andreas.jung@kispi.uzh.ch