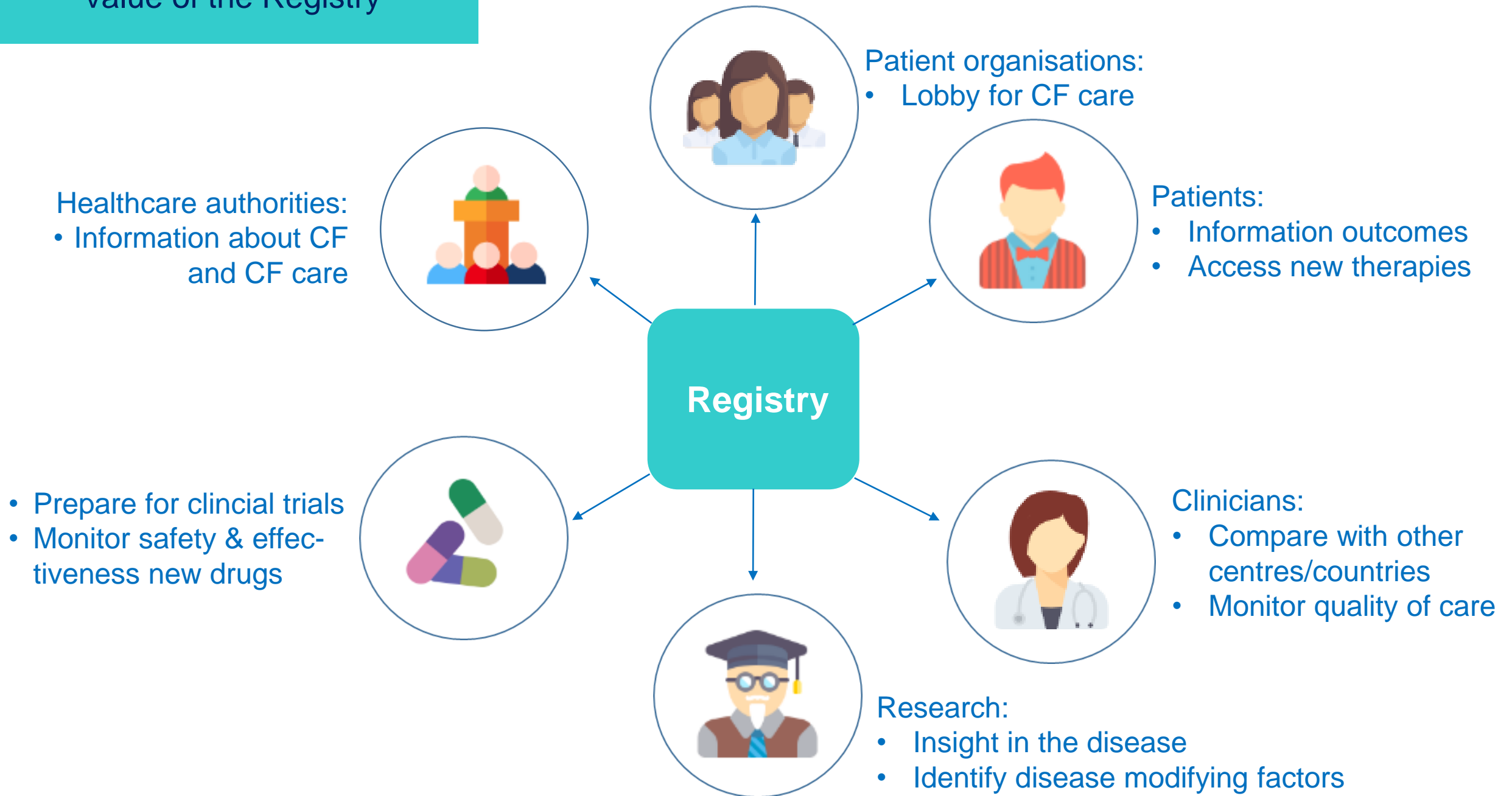


# The European Cystic Fibrosis Society Patient Registry (ECFSPR)



**9th South Eastern European CF meeting**  
Ljubljana, 16 November  
Jacqui van Rens

# Value of the Registry



# The European CF Patient Registry



Good picture of cystic fibrosis: it is important to collect data from as many patients as possible.

## **Mission:**

To collect data to compare aspects of CF and its treatment

Encourage  
new  
standards  
of CF care

Inform  
public  
health  
planning

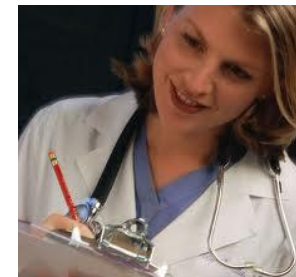
Enable  
research

Data

Collection



**Anonymised  
data**



**Only patients who consent!**

**36 countries**  
**>44,000 patients**

Longitudinal data

2008-2016



**Demographic**

age, gender, status of patient

**Diagnosis**

age at diagnosis, sweat test, meconium ileus, neonatal screening

**Genotype**

1st and 2nd mutation

**Growth / lung function**

value of best FEV1 and FVC, height and weight at best FEV1

**Microbiology**

Pseudomonas aeruginosa, Staphylococcus aureus, Burkholderia cepacia

**Complications**

Diabetes, liver disease, pancreatic status, malignancy

**Therapy**

antibiotic, bronchodilators, oxygen therapy, pancreatic enzymes

**Transplant**

Lung / liver transplantations



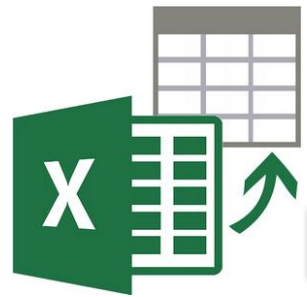
**A platform for the collection of CF data for all purposes**

- ✓ Online
- ✓ Data-collection once a year
- ✓ Encounter data at each patient visit
- ✓ Advanced Security Technology
- ✓ Add-on modules
- ✓ Remote updates



Software

# Collection



**Excel-file Upload**

**Direct  
Data-Entry**



**WEBSERVER**



Software

# ECFSTracker – the software



Albania



Armenia



Austria



Belgium



Bosnia  
and  
Herzegovina



Bulgaria



Croatia



Cyprus



Czech  
Republic



Denmark



France



Georgia



Germany



Greece



Hungary



Ireland



Israel



Italy



Latvia



Lithuania



Luxembourg



Macedonia



Moldova



Netherlands



Norway



Poland



Portugal



Romania



Russia



Slovakia



Slovenia



Serbia



Spain



Sweden



Switzerland



Turkey



Ukraine



UK

ECFSTracker BR | Brussels Trg Centre 1 Jacqui Van Rens

### Patient Encounters

Recent Patients

- 9238352936

Enrol Patient | Browse Patient List

- Clinical use
- Real-time data-input of individual patient visits throughout the year

### Annual Summaries : Brussels Trg Centre 1

Year	Brussels Trg Centre 1 Status
2016	Open
2015	Uninitialised
2014	Complete
2013	Admitted
2012	Admitted
2011	Admitted

Data once a year to Europe

### Brussels Trg Centre 1 Reports

- Number of patients
- Age at follow-up / diagnosis
- Growth and lung function
- Therapy and Microbiology
- Complications


- patient
- centre
- country

### Miscellaneous

Manage Centre | Manage Forms

- Demographics
- Registry
- Diagnosis**
- Transplants

## DEMOGRAPHICS

\* Date of Birth:  

\* Gender of patient:  Male  Female


Is the patient still alive?  He/She is alive  He/She died  Unknown



## REGISTRY

Patient Consented to inclusion in the ECFS Patient Registry:  Consent Given  Consent not Given  Consent Refused

\* Centre Patient Code: 208 

## DIAGNOSIS

Diagnosis confirmed:  No  Diagnosis to be confirmed  Missing  Yes 

Date of Diagnosis:   


Neonatal Screening:  Not done  Unknown  
 Missing (variable not collected)  Yes

Meconium ileus at birth:  No  Unknown  Missing (variable not collected)  
 Yes

## Mutation 1

Not done/missing:

Unknown (unidentified):

Mutation 1:  

Other (Not in list):

Other (not in list):

Date of Mutation 1 result:  

Date	●
<b>Status / Transplant</b>	●
Respiration	●
Therapy	●
Exacerbation History	●
Microbiology	●
Complications of CF	●
Liver Chemistries	●
Re-consent / Assent	●
Socio-Economic Status	●

## DATE

Name of clinic: Centre One  
 Date of encounter: 21/Oct/2018  
 Doctor: Centre1 Admin



## STATUS / TRANSPLANT

## Status

Has the patient died since the last encounter: He/She is alive

- Launch January 2019

## Transplants

Has the patient been assessed for, or had an active transplant since the last encounter: Missing

- Webinars December & January



## RESPIRATION

Weight: 60 kg  
 Height: 165 cm  
 FEV1: 2.5 litres  
 FVC: 3 litres  
 Date of FEV1 measurement: 21/Oct/2016  
 FEF 25 - 75: 3 litres/sec  
 FEF 25 - 75 % of predicted: 3.2 %  
 Seen by physiotherapist: No  
 Seen by dietician: No



## DECISION SUPPORT METRICS

Age as a decimal: 52.18  
 Age in months: 626  
 Weight z-score: 0.18  
 Weight age percentile: 50  
 Height z-score: 0.26  
 Height age percentile: 50  
 BMI: 22.04  
 BMI z-score: 0.09  
 BMI percentile: 50  
 FEV1 / FVC: 0.83  
 FEV1 % of predicted: 89.4  
 FVC % of predicted: 85.43

## Categories of report

- Number of patients
- Age at follow-up / diagnosis
- Growth and lung function
- Therapy and Microbiology
- Complications

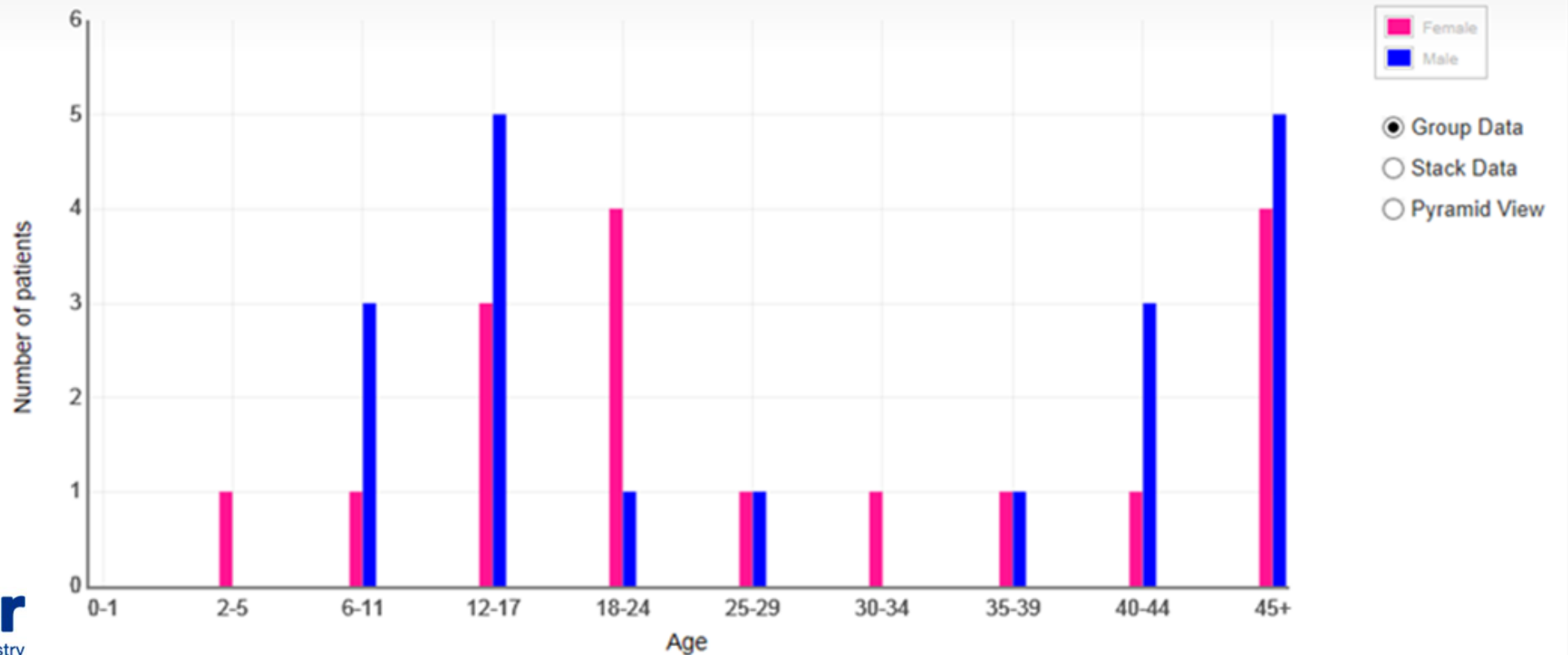
Number of patients by gender (Year: 2014) (All age groups)

ECFSPR Validation Status: No

Graph

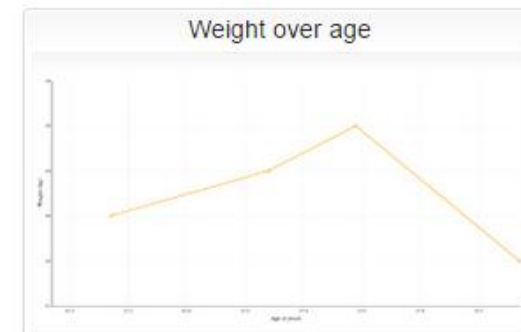
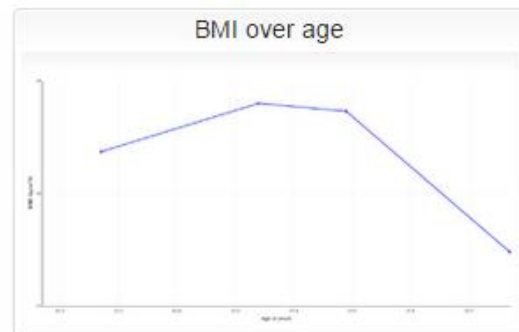
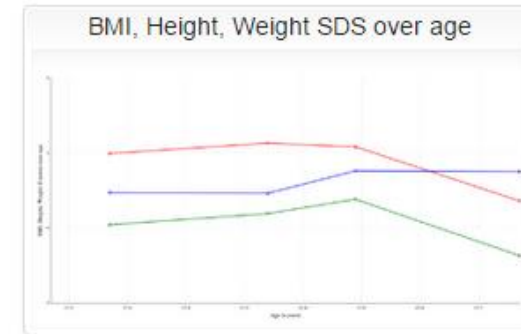
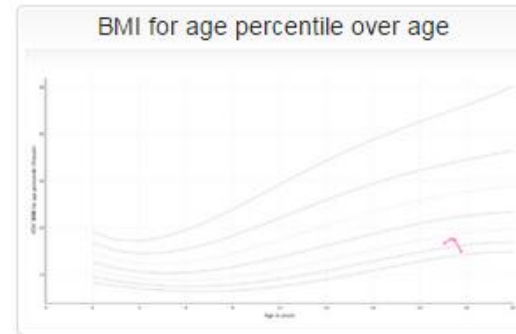
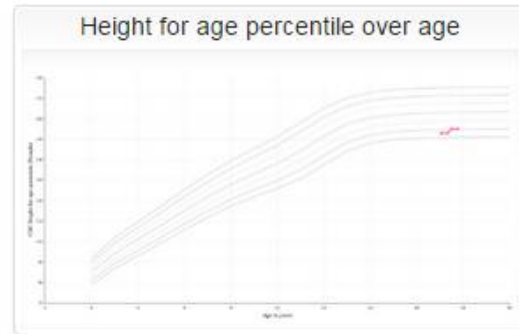
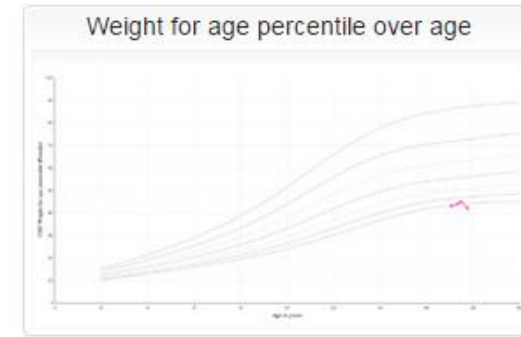
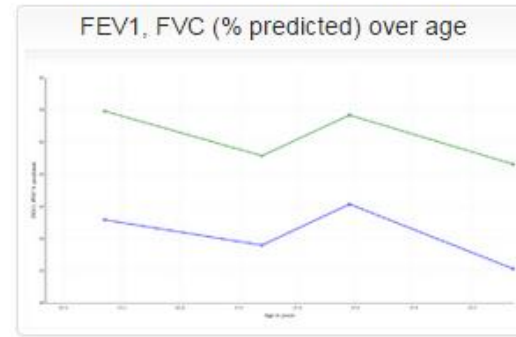
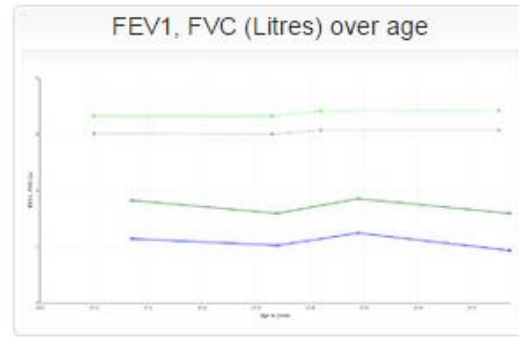
Table

Export



# Output

# Patient Reports



## Benchmarking module

Cross-comparison of indicators of quality of care (= benchmarks),  
e.g. lungfunction, median BMI, proportion adult patients



Comparison between:

- Centre vs Country / multiple Countries
- Centre vs other centres within a country (upon approval)
- Years

**A powerful tool to improve of CF care**



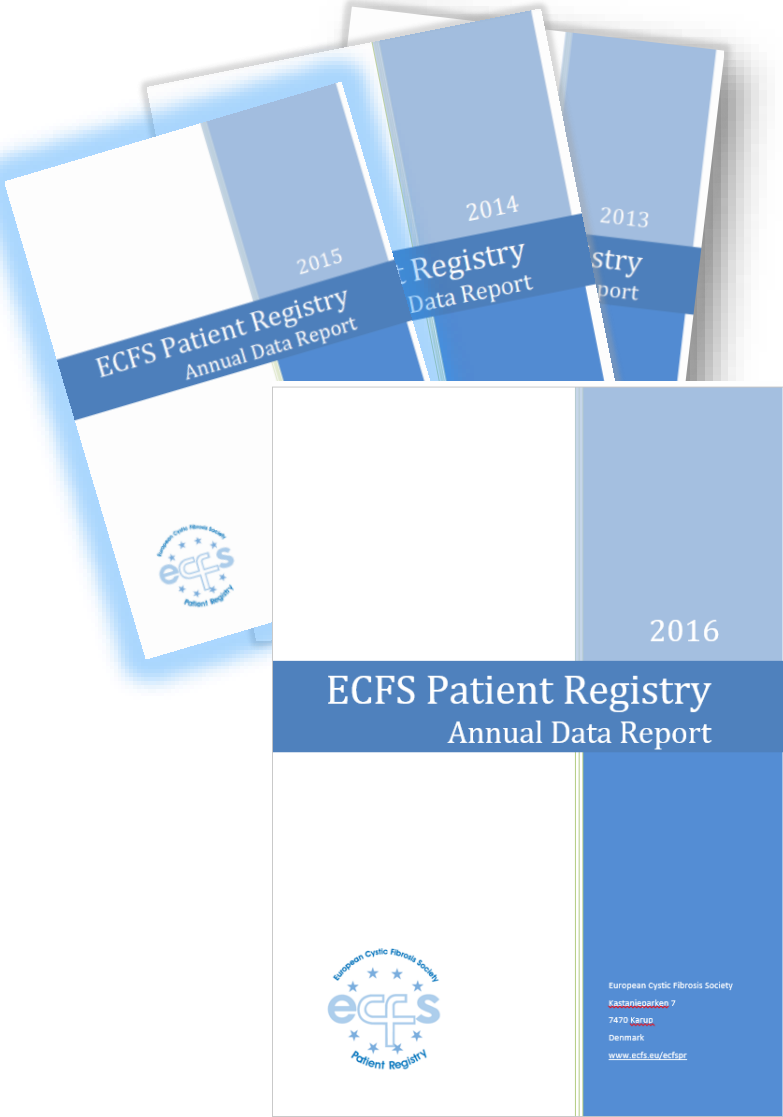
Direct feedback to Centre Staff



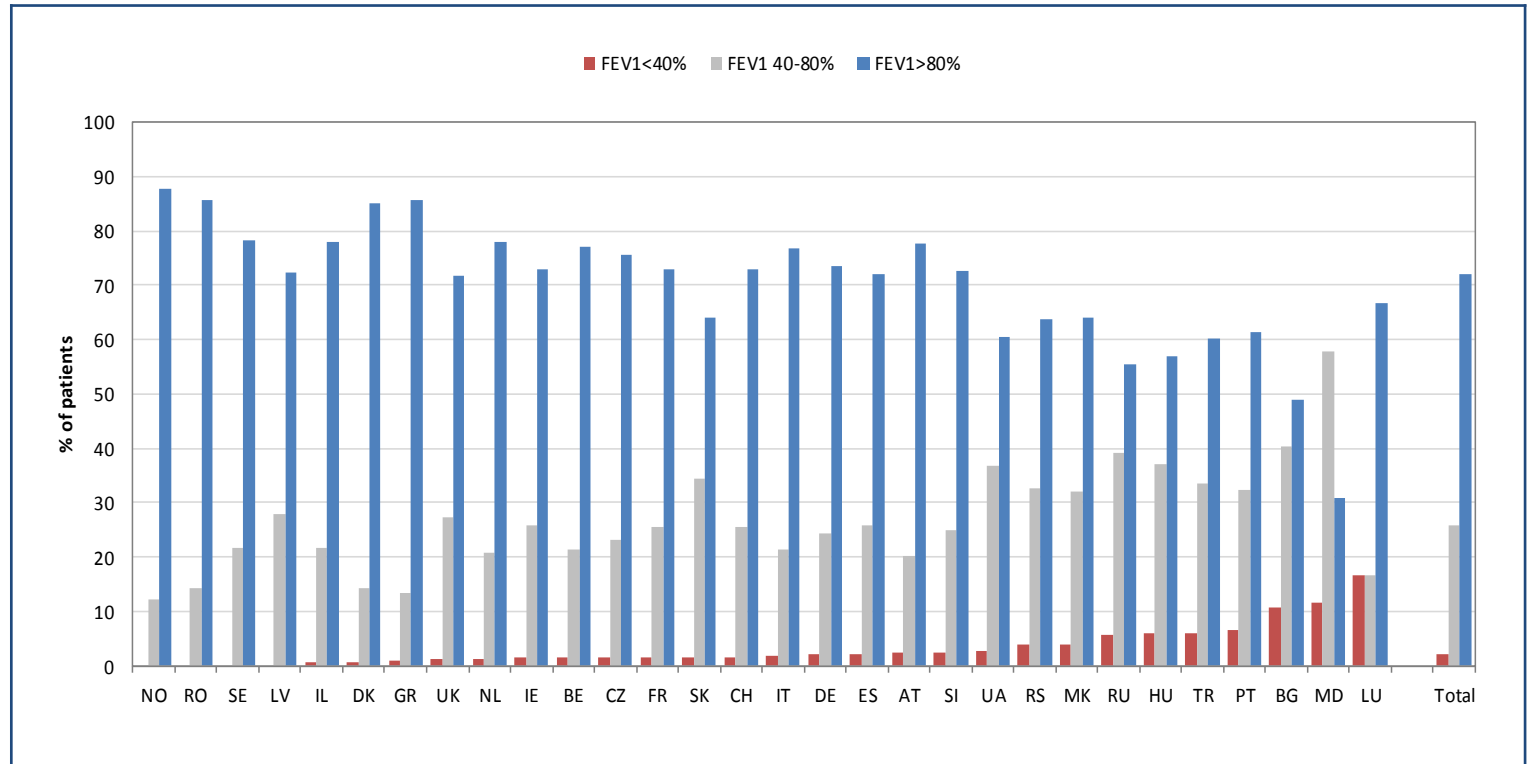
Identify areas for improvement

# Output

# Annual Data Reports



## Variation of severity in CF lung disease in patients 6 – 17 years



**Within 18 months after the close of the follow-up year!**



# Output

# Research

## Database:

- anonymised data of 40.000 patients
- agreed inclusion criteria, definitions & coding
- Linked data from 2008 to 2016

**Good reflection of the natural heterogeneity in CF**

## Data-applications welcome!

- Handled according procedure
- Detailed information on website



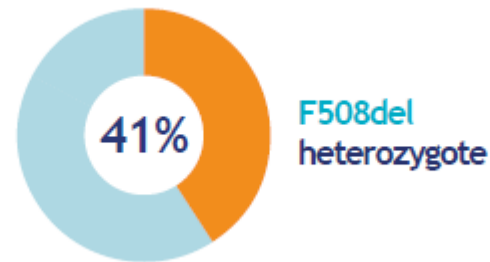
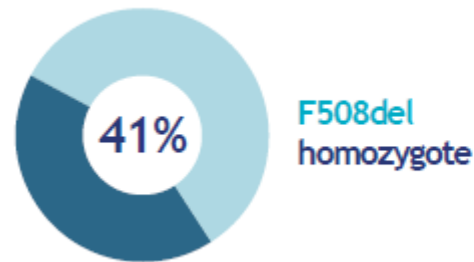
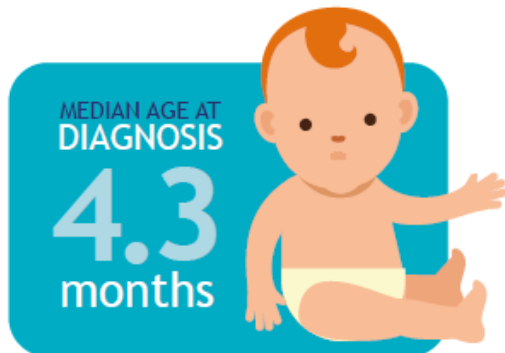
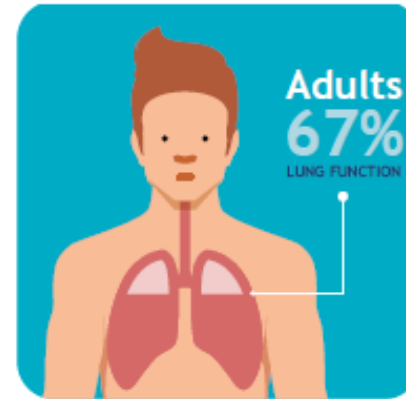
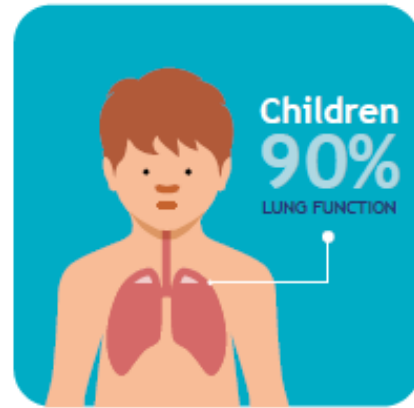
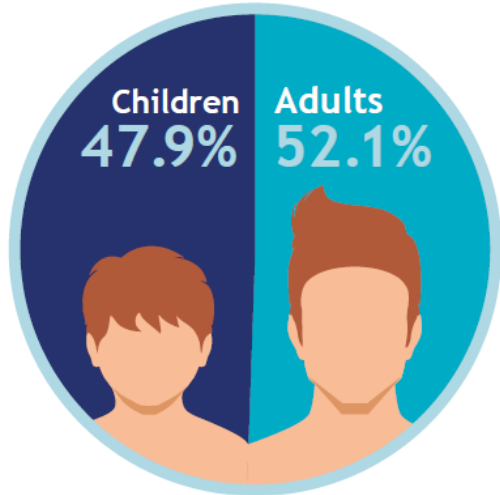
APPLICATION FORM ECFSPR DATA	
All requests for data extractions/data analysis from the European Cystic Fibrosis Society Patient Registry (ECFSPR) must be submitted with this form. We advise you to read all information and complete the form as best as possible to ensure review of your application. You can email the completed form to <a href="mailto:ecfs-pr@uzleuven.be">ecfs-pr@uzleuven.be</a> .	
<b>Applicant details</b>	
Name Primary Investigator (incl. title):	
Primary contact (if not PI):	
Institution/Organisation:	
Position:	
Address:	
Email:	Phone:
Date of request:	
<b>Disclosure</b>	
Please disclose any associations with private companies, especially pharmaceutical companies:	
<b>Research Project Information</b>	
Project Name:	
Project Objective(s):	
Description of the Project:	
Brief Background Information (incl. justification of the clinical relevance):	
Research Question/Hypothesis:	
Study Design:	
Cohort Definition:	
Method of Analysis:	
Start and End Date:	

[www.ecfs.eu/projects/ecfs-patient-registry/data-request-application](http://www.ecfs.eu/projects/ecfs-patient-registry/data-request-application)



- International and pan-European comparison of survival in CF
- Changes in demography and clinical outcomes in CF in Europe
- Risk factors of FEV1 decline in patient with CF across Europe
- Changing epidemiology of the respiratory bacteriology in CF in Europe
- Impact of Dornase Alfa on rate of decline in lung function

Publications: [www.ecfs.eu/ecfspr](http://www.ecfs.eu/ecfspr)



### Zemljevid držav, ki so leta 2014 prispevale podatke za Register evropskega združenja za cistično fibrozo

#### Zakaj je potreben Evropski register CF bolnikov?

Cistična fibroza (CF) je redka bolezen. Da bi dobili dobro predstavlo o CF v Evropi, potrebujemo čim več podatkov. Ti podatki so pomembni za boljše razumevanje bolezni, spodbujanje novih evropskih standardov oskrbe in zdravstvenja, trajanje raziskav ter oblikovanje javnega zdravstvenega načrta.

#### Register evropskega združenja za cistično fibrozo

Zbiraj, meri in primerjaj podatke otrok in odraslih s cistično fibrozo, ki živijo v Evropi in sosednjih državah, in se vključijo v register. Te informacije uporabljajo za izboljšanje zdravja in dobrega počutja ljudi s CF.

#### Kako lahko uporabim podatke iz registra?

ECFSPR objavlja letna poročila z demografskimi in kliničnimi podatki po vsej Evropi in letna poročila s ključnimi informacijami o CF v Evropi.

#### Ta poročila omogočajo:

- lokalnim združenjem bolnikov, da razpravljajo z odločevalci v zdravstvu in drugimi udeleženci, o različnih izbirljavih obravnave CF v vsili državi.
- možnost primerjave CF centrov (v) s drugimi državami in določiti področij z možnostjo izboljšave.
- da se pogovorite s svojim zdravnikom o brivih in pričakovjih.

### CF v Evropi

### CF v Sloveniji

#### Mediana vrednost

Mediana je statistična mera, ki kaže na točko, pri kateri polovica populacije ima nižjo vrednost in polovica višjo.

#### ITM

ITM je test, ki meri, kako hitro se diha. Če je vrednost ITM nižja, pomeni, da je dihanje težje.

#### z-vrednost

z-vrednost je statistična mera, ki kaže na točko, pri kateri polovica populacije ima nižjo vrednost in polovica višjo.

#### Pišljave funkcije:

- FEV1 je merilo pišljave funkcije. Predstavlja največji volumen zraka, ki ga lahko izdihne oseba v prvi sekundi po globokem vdihu.
- FEV1 je delovni pokazatelj zdravja ljudi iste starosti, spola in višine, ki je normalna za 100%.

#### Opombe:

- Opombe: CF ima dve mutaciji, značilni za CF, vasi: podoben ali manj in drugo podobno ali nič.
- Heterozigotni: dve mutaciji sta enaki.
- Homozigotni: mutaciji sta različni.

#### Kateri podatki se zbirajo?

- leto/mesec rojstva, spol
  - genotip, simptomi ob postavitvi diagnoze
  - pišljave funkcije, teža, višina, vrsta oskrbe, terapija, določeni zapleti
- Podatki so anonimni in shranjeni v zaščitni podatkovni zbirki. Za morebitne dodatke uporabljamo natančno opredeljene smernice, ki jih naštrva odbor strokovnjakov.

#### Kako se lahko vključim v ECFSPR?

Preverite pri svojem lokalnem CF centru. Če center sodeluje v ECFSPR, bi morali že biti vključeni, če ste podpisali obrazec za privolitev. Če vaš CF center ne sodeluje, prosite svojega zdravnika, da nas kontaktira, ali pa nam pošljite kontaktne podatke, da bomo lahko stekli v stik z vami.



# Output

# Social Media



# Facebook

# Twitter



**ECFS Registry**  
@ECFSRegistry



New drugs on market need to be monitored.

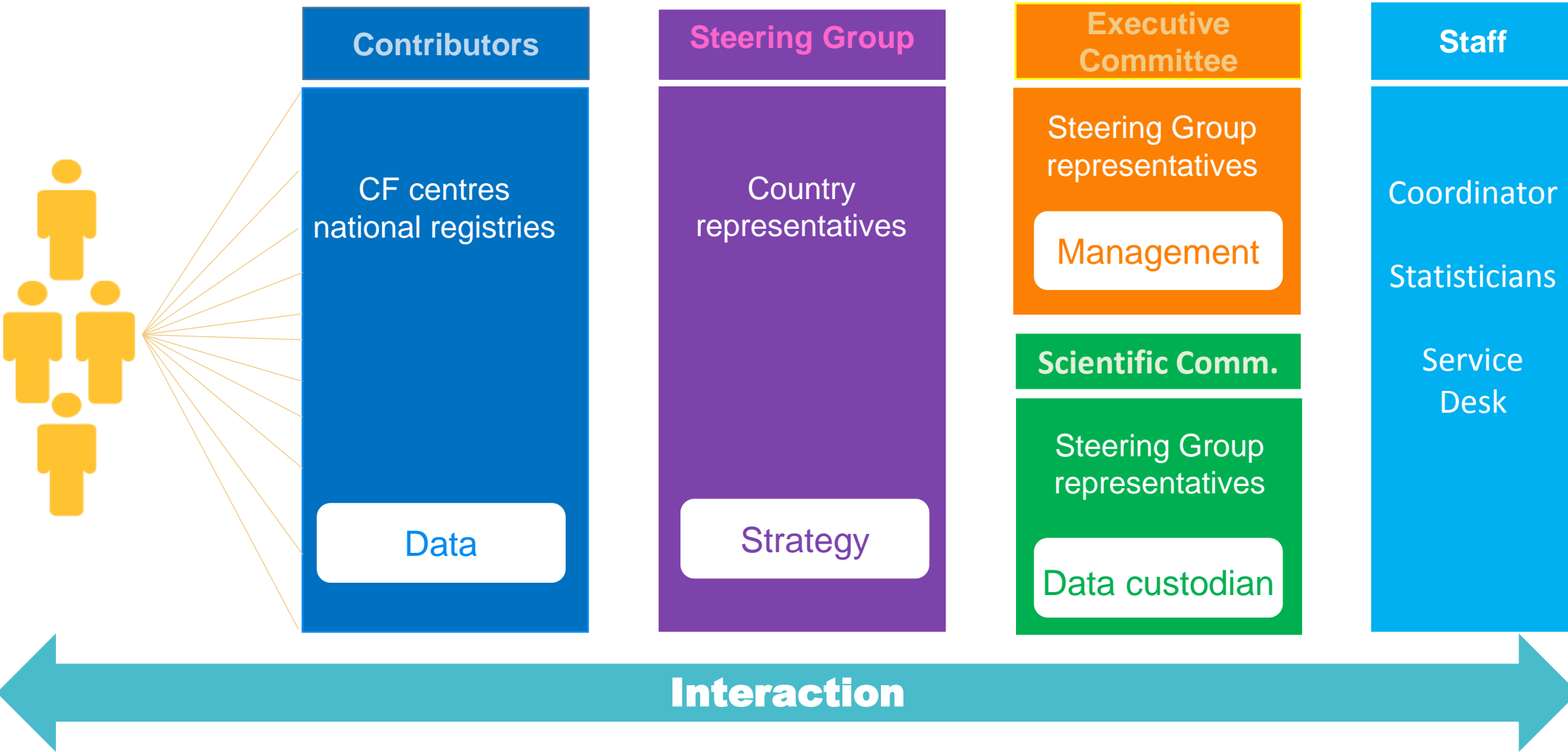
## How?

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.

Further improve quality of data ->request EMA

1. Complete standard operating procedures for all activities
  - National Registries + Countries using direct data-entry
2. Start validation visits in centres using direct data-entry in EU from 2019
  - 20% of the countries, 10% of the data
  - Check informed consents







Software & Training & Support

Opportunities to be engaged in research projects

Network to share experience & exchange ideas

Information & documents

Support to promote the value of a (European) registry at national CF meetings





[www.ecfs.eu/ecfspr](http://www.ecfs.eu/ecfspr)



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