

# DARWIN EU® - Characterisation of individuals with cystic fibrosis in Europe

**First published:** 18/08/2025

**Last updated:** 29/09/2025

Study

Ongoing

## Administrative details

### EU PAS number

EUPAS1000000709

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### Study ID

1000000709

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### DARWIN EU® study

Yes

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### Study countries

- ☐ Croatia
  - ☐ Denmark
  - ☐ France
  - ☐ Germany
  - ☐ United Kingdom
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## Study description

Cystic fibrosis (CF) is a progressive genetic disorder associated with significant morbidity and premature mortality, primarily affecting the respiratory and gastrointestinal systems. It leads to chronic lung infections, pancreatic insufficiency, and other complications requiring comprehensive, lifelong management. This study aims to generate epidemiological evidence on the clinical characteristics and monitoring of individuals with a record of CF diagnosis across Europe between 2015 and 2024.

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## Study status

Ongoing

## Research institutions and networks

### Institutions

Department of Medical Informatics - Health Data Science, Erasmus Medical Center (ErasmusMC)

☐ Netherlands

**First published:** 03/11/2022

**Last updated:** 02/05/2024

Institution

Educational Institution

ENCEPP partner

### Networks

## Data Analysis and Real World Interrogation Network (DARWIN EU®)

- ☐ Belgium
- ☐ Croatia
- ☐ Denmark
- ☐ Estonia
- ☐ Finland
- ☐ France
- ☐ Germany
- ☐ Greece
- ☐ Hungary
- ☐ Italy
- ☐ Netherlands
- ☐ Norway
- ☐ Portugal
- ☐ Spain
- ☐ Sweden
- ☐ United Kingdom

**First published:** 01/02/2024

**Last updated:** 30/04/2025

Network

## Contact details

### Study institution contact

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Study contact

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**Primary lead investigator**

Ellen Gerritsen

Primary lead investigator

## Study timelines

**Date when funding contract was signed**

Planned: 09/04/2025

Actual: 09/04/2025

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**Study start date**

Planned: 25/07/2025

Actual: 25/07/2025

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**Date of final study report**

Planned: 28/11/2025

## Sources of funding

- EMA

## Study protocol

## Regulatory

**Was the study required by a regulatory body?**

Yes

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**Is the study required by a Risk Management Plan (RMP)?**

Not applicable

## Methodological aspects

### Study type

#### Study type list

**Study topic:**

Human medicinal product

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**Study type:**

Non-interventional study

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**Scope of the study:**

Disease epidemiology

Drug utilisation

## **Data collection methods:**

Secondary use of data

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### **Study design:**

A cohort study will be conducted using routinely collected health data from 5 data sources.

### **Main study objective:**

1. To characterise all individuals with a CF diagnosis recorded during the study period in terms of demographics, prespecified comorbidities, *Pseudomonas aeruginosa* colonisation, CF screening, genotyping test, and CFTR modulator treatment use, overall and stratified by paediatric and adult populations.
2. To characterise timing and availability of key clinical measurements, including Forced Expiratory Volume (FEV), height, weight, Body Mass Index (BMI) measurements, sweat chloride levels, and genotyping tests in individuals who initiated any CFTR modulator treatment after CF diagnosis, overall and stratified by paediatric and adult populations.
3. To estimate the background incidence rates of pre-specified events of special interest: cataract, depression, and anxiety in the CF population, overall and stratified by paediatric and adult populations and by calendar year.
4. To measure the annual incidence of pulmonary exacerbation among all individuals with a CF diagnosis recorded during the study period, overall and stratified by paediatric and adult populations.

## Study Design

### **Non-interventional study design**

Cohort

## Study drug and medical condition

**Name of medicine, other**

- Ivacaftor
- Ivacaftor and lumacaftor
- Ivacaftor and tezacaftor
- Ivacaftor, tezacaftor and elexacaftor

## Population studied

**Short description of the study population**

For patient-level characterisation (objective 1), incidence analyses of selected events of special interest and pulmonary exacerbation (objectives 3 and 4), the study population will include all individuals with a CF diagnosis record in the period between 1st January 2015 and 31st December 2024 (or latest date available).

To ensure sufficient follow-up, only individuals with a CF diagnosis record no later than 180 days prior to the end of data availability in each database will be included. Eligible individuals must have at least one year of data visibility prior to the record of CF diagnosis. The requirement of one year prior data availability will not hold for children below 1 year of age.

Additionally, for the incidence analysis of pulmonary exacerbation (objective 4), individuals should not have experienced pulmonary exacerbation in the 60 days prior to study inclusion, defined as a SNOMED disease code of upper or lower respiratory tract infection, requiring treatment with antibiotics or antiviral medications.

For clinical characterisation (objective 2), the study population will include individuals with a first recorded CFTR modulator treatment in the period between 1st of January 2015 and 31st of December 2024 (or latest date available) after CF diagnosis.

Only individuals with a first recorded CFTR modulator treatment at least 180

days prior to the end of data availability in each database will be included. Eligible individuals must have at least one year of data visibility prior to the first recorded CFTR modulator treatment and no prior use of CFTR modulator treatment at active ingredient level. This requirement of 1 year of prior data history will not hold for children below 1 year of age.

## Data management

### ENCePP Seal

The use of the ENCePP Seal has been discontinued since February 2025. The ENCePP Seal fields are retained in the display mode for transparency but are no longer maintained.

## Data sources

### Data source(s)

Clinical Data Warehouse of the Bordeaux University Hospital

Clinical Practice Research Datalink (CPRD) GOLD

Danish Health Data Registries

IQVIA Disease Analyzer Germany

Croatia National Public Health Information System (Nacionalni javnozdravstveni informacijski sustav)

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### Data sources (types)

[Electronic healthcare records \(EHR\)](#)

## Use of a Common Data Model (CDM)



## CDM mapping

Yes

## CDM Mappings

### CDM name

OMOP

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### CDM website

<https://www.ohdsi.org/Data-standardization/>

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### CDM version

<https://ohdsi.github.io/CommonDataModel/index.html>

## Data quality specifications

### Check conformance

Unknown

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### Check completeness

Unknown

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### Check stability

Unknown

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### Check logical consistency

Unknown

## Data characterisation

**Data characterisation conducted**

No