

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

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

**Last updated:** 03/02/2026

Study

Finalised

## Administrative details

### EU PAS number

EUPAS1000000710

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### Study ID

1000000710

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### DARWIN EU® study

Yes

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### Study countries

-  France
  -  Hungary
  -  Italy
  -  Norway
  -  Spain
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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

Finalised

## 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

[n.yefimenkonosova@darwin-eu.org](mailto:n.yefimenkonosova@darwin-eu.org)

### 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

Actual: 10/12/2025

## 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 topic, other:**

cystic fibrosis

**Study type:**

Non-interventional study

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**Data collection methods:**

**Study design:**

A cohort study will be conducted using routinely collected health data from 6 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

## **Medicinal product name, 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 recorded 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.

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## **Age groups**

- **In utero**

- **Adult and elderly population ( $\geq 18$  years)**

- Adults (18 to < 65 years)
  - Adults (18 to < 46 years)
  - Adults (46 to < 65 years)
- Elderly ( $\geq 65$  years)
  - Adults (65 to < 75 years)
  - Adults (75 to < 85 years)
  - Adults (85 years and over)

## Documents

### **Study report**

[DARWIN EU\\_Report\\_P4-C1-009\\_P4-C2-007\\_Characterisation CF\\_V3.pdf](#) (4.49 MB)

[Shiny App](#)

## 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)

IATROS

Norwegian Linked Health registry at University of Oslo

Research Repository @Fondazione IRCCS Ca' Granda Ospedale Maggiore

Policlinico

The Information System for Research in Primary Care (SIDIAP)

Semmelweis University Clinical Data

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### Data source(s), other

Hospital Universitario 12 de Octubre

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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