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

First published: 18/08/2025

Last updated: 11/02/2026

Study Finalised

Administrative details

EU PAS number

EUPAS1000000709

Study ID

1000000709

DARWIN EU® study

Yes

Study countries

- [ ] Croatia
- [ ] Denmark
- [ ] France
- [ ] Germany
- [ ] United Kingdom

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.

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

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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

Study start date

Planned: 25/07/2025

Actual: 25/07/2025

Date of final study report

Planned: 28/11/2025

Actual: 10/12/2025

Sources of funding

- EMA

Study protocol

DARWIN EU_Protocol_P4-C1-009_Characterisation CF patients_V3.0.pdf (1.05 MB)

Regulatory

Was the study required by a regulatory body?

Yes

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

Not applicable

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Disease epidemiology

Drug utilisation

Data collection methods:

Secondary use of data

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

Medicinal product name, other

• Ivacaftor

• Ivacaftor and lumacaftor

• Ivacaftor and tezacaftor

• Ivacaftor, tezacaftor and elexacaftor

Medical condition to be studied

Cystic fibrosis

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.

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)

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)

Data sources (types)

Electronic healthcare records (EHR)

Use of a Common Data Model (CDM)

CDM mapping

Yes

CDM Mappings

CDM name

OMOP

CDM website

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

CDM version

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

Data quality specifications

Check conformance

Unknown

Check completeness

Unknown

Check stability

Unknown

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

No