DARWIN EU® Comparing direct and indirect methods to estimate prevalence of chronic diseases using real-world data

First published: 27/03/2024

Last updated: 25/09/2024

Study Finalised

Administrative details

EU PAS number

EUPAS1000000088

Study ID

1000000088

DARWIN EU® study

Yes

Study countries

Netherlands

Spain

United Kingdom

Study description

In the context of chronic diseases with relatively low prevalence, how do direct and indirect RWD-based estimates of prevalence compare with each other?

The specific objectives of this study are to:
1) Estimate the disease prevalence (direct estimate based on the proportion of individuals with the condition).
2) Estimate the disease incidence rate.
3) Estimate duration of disease using Kaplan-Meier survival curves. Of particular interest is the estimate of median survival as a summary measure of disease duration.
4) Produce an indirect estimation of prevalence as the product of incidence and median survival.
for the following diseases:
- Cystic fibrosis
- Haemophilia (A and/or B)
- Pulmonary arterial hypertension
- Pancreatic cancer
- Sickle cell disease

Results will be provided overall and where possible stratified by age group: paediatrics (0-17 years old) and adults (18 years old and above).

Study status

Finalised

Research institutions and networks

Networks

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

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

First published: 01/02/2024

Last updated: 05/11/2026

Network

Contact details

Study institution contact

Ilse Schuemie study@darwin-eu.org

Study contact

study@darwin-eu.org

Primary lead investigator

Maria de Ridder

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 12/12/2023

Actual: 12/12/2023

Study start date

Planned: 01/01/2010

Actual: 01/01/2010

Date of final study report

Planned: 22/04/2024

Actual: 26/06/2024

Sources of funding

- EMA

Study protocol

DARWIN EU_D2.2.3_Protocol_P2-C1-013_Direct and indirect prevalence estimation V2.pdf (659 KB)

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:

Disease /health condition

Study type:

Non-interventional study

Study design:

A retrospective cohort design to estimate disease point prevalence and incidence. A retrospective cohort design to estimate median survival as a proxy

for disease duration. Data from three databases with routinely-collected electronic healthcare records of general practices will be used.

Main study objective:

The objective of this study is to compare direct and indirect estimations of prevalence of some rare, chronic diseases.

Study drug and medical condition

Medical condition to be studied

Cystic fibrosis

Haemophilia

Pulmonary arterial hypertension

Pancreatic carcinoma

Sickle cell disease

Population studied

Age groups

- **Paediatric Population (< 18 years)**
  - Preterm newborn infants (0 – 27 days)
  - Term newborn infants (0 – 27 days)
  - Infants and toddlers (28 days – 23 months)
  - Children (2 to < 12 years)
  - Adolescents (12 to < 18 years)
- **Adult and elderly population (≥18 years)**
  - Adults (18 to < 65 years)
    - Adults (18 to < 46 years)

        - Adults (46 to < 65 years)
    - Elderly (≥ 65 years)
        - Adults (65 to < 75 years)
        - Adults (75 to < 85 years)
        - Adults (85 years and over)

Documents

Study report

DARWIN EU_D2.2.4_Report_P2-C1-013_IndirectPrevalence_V3.pdf (952.36 KB)

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 Practice Research Datalink (CPRD) GOLD

Integrated Primary Care Information (IPCI)

The Information System for Research in Primary Care (SIDIAP)

Use of a Common Data Model (CDM)

CDM mapping

Yes

CDM Mappings

CDM name

OMOP

CDM website

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

Data quality specifications

Check conformance

Unknown

Check completeness

Unknown

Check stability

Unknown

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

No