A long-term non-interventional study to assess the incidence of skin malignancies in patients with dystrophic and junctional epidermolysis bullosa receiving treatment with Filsuvez (FOSTER)

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Study Planned

Administrative details

EU PAS number

EUPAS1000000244

Study ID

1000000244

DARWIN EU® study

No

Study countries

- [ ] Austria
- [ ] France

- ☐ Germany
- ☐ Greece
- ☐ Italy
- ☐ Netherlands
- ☐ Spain
- ☐ United Kingdom

Study description

In patients with epidermolysis bullosa (EB), collagen does not form properly, so their skin is very fragile and blisters easily. Such patients are also at greatly increased risk of developing skin cancers. Filsuvez is a topical gel used to promote healing of skin lesions in patients with certain types of EB. In this observational study, patients with either dystrophic EB (DEB) or junctional EB (JEB) will receive standard of care treatment, whether Filsuvez or something else, and will be followed for up to 5 years. The main purpose is to see if the use of Filsuvez affects the likelihood of developing skin malignancies in these patient populations.

Study status

Planned

Research institutions and networks

Institutions

Amryt Pharmaceuticals DAC

☐ Ireland

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Institution | Pharmaceutical company

Contact details

Study institution contact

Anna Rozova a.rozova@chiesi.com

Study contact

a.rozova@chiesi.com

Primary lead investigator

Jemima Mellerio

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 01/10/2024

Study start date

Planned: 01/11/2024

Data analysis start date

Planned: 01/07/2026

Date of interim report, if expected

Planned: 01/11/2026

Date of final study report
Planned: 01/12/2032

Sources of funding

- Pharmaceutical company and other private sector

Study protocol

ed-amryt-iab23965protocolpassv30 (1).pdf(1.26 MB)

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 3 (required)

Other study registration identification numbers and links

NCT06423573

Link to Clinicaltrials.gov

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Data collection methods:

Combined primary data collection and secondary use of data

Study design:

Post-authorization safety study (PASS) with a non-interventional cohort design, based on primary data collection from patients enrolled at participating dermatology sites and secondary use of registry data from patients included in EB registries.

Main study objective:

To estimate the incidence of first skin malignancy during follow-up in patients exposed to Filsuvez.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Name of medicine

FILSUVEZ

Name of medicine, other

Oleogel-S10

Birch bark extract

Anatomical Therapeutic Chemical (ATC) code

(D03AX13) Betulae cortex

Betulae cortex

Medical condition to be studied

Epidermolysis bullosa

Population studied

Short description of the study population

Patients with either dystrophic epidermolysis bullosa (DEB) or junctional epidermolysis bullosa (JEB) who are considered at risk for developing skin malignancies.

Age groups

All

Paediatric Population (< 18 years)

Preterm newborn infants (0 – 27 days)

Term newborn infants (0 – 27 days)

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)

Estimated number of subjects

580

Study design details

Setting

Patients who are receiving standard of care from their physician or who are enrolled in EB registries will be followed for the occurrence of skin malignancies from the date of study enrollment to the date of discontinuation or end of the study. Follow-up will be for approximately 5 years.

Patients with a confirmed diagnosis of either DEB or JEB are eligible. There are no exclusion criteria. The incidence of skin malignancies will be assessed both in patients receiving Filsuvez and those not receiving it.

Comparators

Patients will receive whatever treatment their doctor is prescribing for them, whether Filsuvez or something else. Out of the planned total of 580 patients, it is expected that approximately half will be using Filsuvez.

Outcomes

The primary outcome measure is incidence of first skin malignancy during follow-up in EB patients receiving Filsuvez. Patients will be followed for the occurrence of skin malignancies from the date of study enrolment until either the date of discontinuation or termination of the study. The main secondary outcome measures is the incidence of first skin malignancy during follow-up in EB patients not receiving Filsuvez over this same time period.

Data analysis plan

This study is purely descriptive, and will not include any statistical testing of outcomes between Filsuvez-exposed and unexposed patients. Demographic, clinical characteristics, Filsuvez use patterns, and characteristics of skin malignancies will be included in the descriptive analyses of the Filsuvez-exposed and unexposed patients. Continuous variables will be described using mean, standard deviation, median, first and third quartiles, interquartile range, range, as appropriate. Categorical variables will be described with counts and percentages (relative to the number of non-missing observations for each variable).

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), other

Primary data sources: Participating dermatology clinics

Secondary data sources: EB registries

Data sources (types)

Disease registry

Non-interventional study

Use of a Common Data Model (CDM)

CDM mapping

No

Data quality specifications

Check conformance

Unknown

Check completeness

Unknown

Check stability

Unknown

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

No