Global Patient Registry to Monitor Longterm Safety and Effectiveness of Increlex® in Children and Adolescents With Severe Primary Insulin-like Growth Factor-1 Deficiency (SPIGFD)

First published: 17/10/2014 Last updated: 06/05/2025



### Administrative details

#### **EU PAS number**

EUPAS7708

#### **Study ID**

50235

#### DARWIN EU® study

No

#### **Study countries**

Austria

France

Germany
Italy
Poland
Spain
Sweden
United Kingdom
United States

#### Study description

The Increlex® Global Registry is a descriptive, multicenter, observational, prospective, open-ended, non interventional, post-authorisation surveillance registry.

This registry is a Post-Authorisation Safety Study which is intended primarily to collect, analyse and report safety data during and up to at least 5 years after the end of treatment in children and adolescents receiving Increlex® therapy for Severe Primary IGF-1 Deficiency according to the locally approved product information.

The second objective is to follow the effectiveness of this treatment. Patients who have already started Increlex® therapy before entering this registry may be included and data will be collected retrospectively.

For each subject, the Increlex® treatment period duration is at the discretion of the investigator according to his/her judgment on the basis of clinical needs of the subject.

The Sponsor will attempt to follow the subject until the Final adult height is attained if not reached within the 5 years post treatment period. Safety data analyses are performed every 6 months. In addition, an independent Data Monitoring Committee (DMC) composed of 3 experts in paediatric endocrinology, oncology and statistician are conducting a review of available safety data on at least an annual basis.

#### Study status

Ongoing

### Research institutions and networks

### Institutions

### Ipsen Pharma

First published: 01/02/2024

Last updated: 01/02/2024

Institution

### Contact details

### Study institution contact Medical Director Endocrinology Rare Diseases clinical.trials@ipsen.com

Study contact

clinical.trials@ipsen.com

Primary lead investigator Medical Director Endocrinology

Primary lead investigator

### Study timelines

Date when funding contract was signed Planned: 19/06/2008 Actual: 19/06/2008

**Study start date** Planned: 01/01/2009 Actual: 09/12/2008

Data analysis start date Planned: 01/12/2010 Actual: 01/12/2010

Date of interim report, if expected Planned: 31/12/2025 Actual: 06/12/2023

Date of final study report Planned: 31/12/2028

### Sources of funding

• Pharmaceutical company and other private sector

### More details on funding

Ipsen Pharma

### Study protocol

2-79-52800-002\_Protocol Amendment 8\_Final Redacted\_08Feb22 (3).pdf(6.17 MB)

### Regulatory

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

Yes

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

EU RMP category 2 (specific obligation of marketing authorisation)

# Other study registration identification numbers and links

NCT00903110

Link to ClinicalTrials.gov

### Methodological aspects

### Study type

### Study type list

#### Study topic:

Human medicinal product

#### Study type:

Non-interventional study

#### Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness Effectiveness study (incl. comparative)

#### Main study objective:

To collect, analyse and report safety data during and at least 5 years after the end of the treatment in children and adolescents receiving Increlex therapy for SPIGFD according to the locally approved product information.

### Study Design

**Non-interventional study design** Other

Non-interventional study design, other

Observational Model: Case-Only

### Study drug and medical condition

## Name of medicine

#### Study drug International non-proprietary name (INN) or common name

MECASERMIN

#### Anatomical Therapeutic Chemical (ATC) code

(H01AC03) mecasermin mecasermin

#### Medical condition to be studied

Severe primary insulin like growth factor-1 deficiency Growth failure

### Population studied

#### Age groups

Children (2 to < 12 years) Adolescents (12 to < 18 years)

#### Estimated number of subjects

500

### Study design details

#### Outcomes

Incidence of: serious adverse events (SAEs) including any AESI of neoplasia, all targeted adverse events (AEs), all AEs, deaths and withdrawals due to AEs during Increlex® treatment period up to 30 days after the last dose. Changes from baseline for effectiveness variables, Estimation of difference between predicted & final adult height, Modelling effectiveness parameters, Dose administered & duration of exposure, Biological assessments, genetic tests, Incidence of safety events at 2 and 5 years post-treatment, Description of neoplasias (benign and malignant) and hypoglycaemias, Evolution of QoL using EQ-5D-Y.

#### Data analysis plan

The statistical analyses will be performed in accordance with ICH E9 guideline and will be based on the pooled data from the individual study sites, unless otherwise stated.

Primary analyses during treatment period:

- description and incidence of any serious adverse events including neoplasia,
- incidence of all targeted adverse events
- description and incidence of all adverse events.

### Documents

#### **Study report**

2-79-52800-002 synopsis\_07Jan2022\_no marks (2).pdf(2.73 MB)
2-79-52800-002\_synopsis\_01Dec2011.pdf(477.98 KB)
2-79-52800-002\_synopsis\_10Jan2020.pdf(2.77 MB)
2-79-52800-002\_synopsis\_14Dec2015.pdf(2.28 MB)
2-79-52800-002\_synopsis\_20Dec2017.pdf(2.66 MB)
2-79-52800-002\_synopsis\_22Nov2013.pdf(119.03 KB)
2-79-52800-002\_CSR Synopsis\_06Dec23\_No Redaction Marks\_Final.pdf(2.74 MB)

#### **Study publications**

Bang P, Polak M, Woelfle J, Houchard A, EU IGFD Registry Study Group. Effective... Bang P, Woelfle J, Perrot V, Sert C, Polak M. Effectiveness and safety of rhIGF... Bang P, Polak M, Perrot V, Sert C, Shaikh H, Woelfle J. Pubertal Timing and Gro...

### Data management

### Data sources

#### Data sources (types)

Electronic healthcare records (EHR) Other

#### Data sources (types), other

Prospective patient-based data collection

### 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