Global Patient Registry to Monitor Long-term 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

Study Ongoing

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

INCRELEX

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