Prospective and retrospective, single-cohort, multicenter observational long-term study in short children born small for gestational age (SGA) after treatment with Saizen® or with other recombinant human growth hormone (r-hGH) products (SALTO)

First published: 02/12/2016

Last updated: 07/06/2024

Study Ongoing

Administrative details

EU PAS number

EUPAS16520

Study ID

24336

DARWIN EU® study

No

Study countries

- [ ] France
- [ ] Germany
- [ ] Spain
- [ ] Sweden
- [ ] United Kingdom

Study description

Prospective and retrospective, single-cohort, multicentre, multinational observational long-term follow-up study in subjects born SGA who received Saizen® or other r-hGHproducts for the treatment of short stature. The study will comprise a 10-year safety follow-up period after cessation of r-hGH treatment in short children born SGA who had received Saizen® or other r-hGH products in the frame of a sponsored clinical study or in the post-marketing setting. The subjects may be enrolled up to 5 years after treatment cessation.

Study status

Ongoing

Research institutions and networks

Institutions

CHU de Toulouse - Hôpital des Enfants

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Institution

Contact details

Study institution contact

Communication Center Merck KGaA

service@merckgroup.com

Study contact

service@merckgroup.com

Primary lead investigator

Communication Center Merck KGaA

Primary lead investigator

Study timelines

Date when funding contract was signed

Actual: 23/06/2010

Study start date

Actual: 04/01/2011

Date of interim report, if expected

Planned: 30/09/2026

Date of final study report

Planned: 30/09/2031

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Merck KGaA

Study protocol

20180528_EMR200098-008_EnCepP_Final protocol_Redacted.pdf (7.03 MB)

20210628_EMR200098-008_EnCepP_Final protocol_Redacted.pdf (1.51 MB)

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 1 (imposed as condition of marketing authorisation)

Other study registration identification numbers and links

EMR 200098_008Saizen® Long Term Observational study

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Main study objective:

To assess the long-term safety of Saizen® or other r-hGH treatment for 10 years after cessation of treatment, in terms of occurrence of type 2 diabetes mellitus and malignancies, in a minimum of 200 subjects born SGA who received Saizen® or other r-hGH products for the treatment of short stature.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name, other

Saizen

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

SOMATROPIN

Anatomical Therapeutic Chemical (ATC) code

(H01AC01) somatropin

somatropin

Medical condition to be studied

Gestational age test abnormal

Population studied

Age groups

- Children (2 to < 12 years)
- Adolescents (12 to < 18 years)
- Adults (18 to < 46 years)

Estimated number of subjects

300

Study design details

Outcomes

The number and proportion of subjects diagnosed at any time during the 10 year follow-up will be tabulated for the two primary endpoints:- Type 2 diabetes mellitus- Any malignancy, - Metabolic syndrome and/or changes in glucose metabolism parameters as per Glycaemia parameters, fasting plasma glucose and postprandial glucose, and/or fasting plasma insulin levels as specified in the protocol.- Malignancies- Correlation of T2DM and/or malignancies will be established against BPI, BMI and others as specified in the protocol.

Data analysis plan

No statistical significance testing will be performed. The study will contribute at the estimation of the incidence of type 2 diabetes mellitus and the incidence of any malignancy among study participants.For primary endpoints, details for each subject of the safety population will be listed and will include at least, time

on follow-up, onset date(s) of event(s) and description of event(s).A sensitivity analysis will be performed on the completer population.All secondary endpoints will be analysed using appropriate statistics. For quantitative variables, statistics are the number of non-missing values (N), number of missing values, mean, standard deviation (SD), minimum (Min), first quartile (Q1), median, third quartile (Q3) and maximum (Max). For qualitative variables, statistics within categories are number ofnon-missing values (N), number of missing values and percentages of subjects.

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 sources (types)

Other

Data sources (types), other

Prospective and retrospective 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