The Impact of Achondroplasia on Quality of Life, Healthcare Resource Use, Clinical, Socio-economic and Psychosocial state of the Individual (LIAISE)

First published: 23/01/2017

Last updated: 14/01/2021

Study Finalised

Administrative details

EU PAS number

EUPAS17347

Study ID

39022

DARWIN EU® study

No

Study countries

- ☐ Austria
- ☐ Denmark
- ☐ Germany

- [ ] Italy
- [ ] Spain
- [ ] Sweden

Study description

Observational studylooking at the burden of illness in achondroplasia patients aged 5-70. The study will include a 5 year review of historical clinical data as well as a a single point collection of questionnaire data to look at the impact on the following in individuals with achondroplasia versus a normative population: - Quality of life - Clinical burden - Healthcare resource use Socio-economic burden - Psychosocial burden Up to 300 subjects will be included in 13 sites in Germany, Austria, Spain, Italy, Denmark and Sweden

Study status

Finalised

Research institutions and networks

Institutions

BioMarin Pharmaceuticals

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Institution

Multiple centres: 13 centres are involved in the study

Contact details

Study institution contact

Director Program medinfoeu@bmrn.com

Study contact

medinfoeu@bmrn.com

Primary lead investigator

Director Program

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 03/11/2016

Actual: 03/11/2016

Study start date

Planned: 30/10/2017

Actual: 22/12/2017

Data analysis start date

Planned: 28/04/2018

Actual: 31/08/2018

Date of interim report, if expected

Planned: 29/06/2018

Actual: 19/10/2018

Date of final study report

Planned: 14/08/2020

Actual: 19/11/2020

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

BioMarin Pharmaceutical Inc

Regulatory

Was the study required by a regulatory body?

No

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

Scope of the study:

Disease epidemiology

Data collection methods:

Combined primary data collection and secondary use of data

Main study objective:

The objectives of the study are to describe the impact on the following in individuals with achondroplasia versus a normative population in Denmark, Germany, Spain and Sweden: • Quality of life• Clinical burden• Healthcare resource use• Socio-economic burden • Psychosocial burden

Study Design

Non-interventional study design

Cross-sectional

Other

Non-interventional study design, other

Retrospective

Study drug and medical condition

Medical condition to be studied

Dwarfism

Population studied

Short description of the study population

Achondroplasia patients aged 5-70 years.

Age groups

Children (2 to < 12 years)

Adolescents (12 to < 18 years)

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Adults (65 to < 75 years)

Special population of interest

Other

Special population of interest, other

Achondroplasia patients

Estimated number of subjects

300

Study design details

Outcomes

1. Patient Reported Outcomes (PRO) and Activities of Daily Living (ADL)2. Clinical burden for at least the last five years3. Healthcare resource use for at least the last five years 4. Socio-economic burden5. Psychosocial burden

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Data analysis plan

Statistical analysis will be carried out using SAS software. An interim analysis will be performed after nine months of enrolment.Continuous variables will be described (distribution) by their mean, standard deviation, median, quartiles one and three, extreme values (minimum and maximum) and the number of missing data and will be compared to normative data.Categorical variables will be described (counts) by the absolute and relative (%) frequency of each class, and the number of missing data and will be compared to normative data.

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 patient-based data collection, Historical data from clinical records

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