EVALUATION OF THE EFFECTIVENESS OF RISK MINIMISATION MEASURES: A SURVEY AMONG HEALTH CARE PROFESSIONALS AND PATIENT/CAREGIVERS TO ASSESS THEIR KNOWLEDGE AND ATTITUDES ON PRESCRIBING AND HOME ADMINISTRATION CONDITIONS OF VELAGLUCERASE ALPHA (VPRIV®) IN 6 EUROPEAN COUNTRIES (VPRIV Home infusion)

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Study Planned

Administrative details

EU PAS number

EUPAS14255

Study ID

21000

DARWIN EU® study

No

Study countries

- [ ] France
- [ ] Germany
- [ ] Italy
- [ ] Netherlands
- [ ] Spain
- [ ] United Kingdom

Study description

Whether educational material (EM), implemented as additional risk minimisation measures (aRMMs), were effective to ensure that prescribers of velaglucerase alfa - are knowledgeable about risks related to home infusion of the product and its conditions of use, - appropriately select patients eligible for home infusion of the product, - communicate to patients and caregivers the requirements for home infusion, organisation of home infusion/preparation of infusion, safety information, infusion diary, and personalized emergency plan - give the appropriate educational materials to the patients And whether patients/caregivers appropriately understand and implement the educational material, infusion diary and emergency plan.

Study status

Planned

Research institutions and networks

Institutions

IMS Health

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Institution

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Toussi Massoud

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 31/08/2016

Study start date

Planned: 01/01/2016

Data analysis start date

Planned: 03/04/2017

Date of final study report
Planned: 30/06/2017

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Shire Pharmaceuticals

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)

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Effectiveness study (incl. comparative)

Main study objective:

to assess the proportion of prescribers treating patients with Gaucher Disease who receive, understand and implement the Educational Material messages.

Study Design

Non-interventional study design

Cross-sectional

Study drug and medical condition

Anatomical Therapeutic Chemical (ATC) code

(A16AB10) velaglucerase alfa

velaglucerase alfa

Medical condition to be studied

Gaucher's disease

Population studied

Age groups

Infants and toddlers (28 days – 23 months)

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)

Adults (75 to < 85 years)

Adults (85 years and over)

Estimated number of subjects

96

Study design details

Data analysis plan

The statistical results of the six countries will be presented in the same report, overall, per country and per physician’s specialty group Continuous variables will be described by their number (of valid cases, of missing values), mean, standard deviation, and median, Q1, Q3, minimum and maximum.Categorical variables will be described as the total number and relative percentage per category. These will be the percentage per category.In case of multiple choice questions, the frequency of each option provided by the physicians will be reported in the statistical results. Different combinations of the answers provided will not be considered. Confidence intervals of 95% will be evaluated, when relevant.The proportions of correct and appropriate answers to selected questions asked in the questionnaire will be expressed among physicians with complete analysable web

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

Survey is a primary data collection conducted through a web and paper questionnaire

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