

# 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)

**First published:** 20/07/2016

**Last updated:** 19/09/2017

Study

Planned

## Administrative details

### EU PAS number

EUPAS14255

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### Study ID

21000

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## **DARWIN EU® study**

No

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### **Study countries**

- ☐ France
  - ☐ Germany
  - ☐ Italy
  - ☐ Netherlands
  - ☐ Spain
  - ☐ United Kingdom
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### **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.

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### **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

[mtoussi@fr.imshealth.com](mailto:mtoussi@fr.imshealth.com)

### Primary lead investigator

Toussi Massoud

Primary lead investigator

## Study timelines

### Date when funding contract was signed

Planned: 31/08/2016

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### Study start date

Planned: 01/01/2016

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### Data analysis start date

Planned: 03/04/2017

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### **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

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### **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

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**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

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**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)
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## **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

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### 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

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### Check completeness

Unknown

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### Check stability

Unknown

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**Check logical consistency**

Unknown

Data characterisation

**Data characterisation conducted**

No