

# Additional risk minimisation measures for Ruconest - European survey of educational materials

**First published:** 21/07/2025

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Study

Ongoing

## Administrative details

### EU PAS number

EUPAS1000000680

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### Study ID

1000000680

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### DARWIN EU® study

No

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### Study countries

 European Union

 United Kingdom

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### Study description

Study is to evaluate the effectiveness of the risk minimisation materials for Ruconest distributed to treatment centres/prescribing physicians in EU/UK countries where Ruconest self-administration kit is launched

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### **Study status**

Ongoing

## Research institutions and networks

### Institutions

#### Pharming Technologies

**First published:** 01/02/2024

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**Institution**

## Contact details

### **Study institution contact**

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**Study contact**

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### **Primary lead investigator**

Delphine Vandenberghe

## Study timelines

### **Date when funding contract was signed**

Planned: 28/02/2020

Actual: 11/02/2020

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### **Study start date**

Planned: 18/09/2020

Actual: 18/12/2020

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### **Date of final study report**

Planned: 18/01/2026

## Sources of funding

- Pharmaceutical company and other private sector

## Study protocol

[Protocol PHARMEUaRMM01 v1.0 20180320.pdf](#) (283.34 KB)

## Regulatory

### **Was the study required by a regulatory body?**

Yes

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

## Methodological aspects

### Study type

### Study type list

**Study topic:**

Human medicinal product

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**Study type:**

Non-interventional study

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**Scope of the study:**

Assessment of risk minimisation measure implementation or effectiveness

**Data collection methods:**

Combined primary data collection and secondary use of data

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**Study design:**

This is a cross-sectional survey among physicians who have received the updated educational materials for Ruconest for self-administration, prescribe Ruconest, and practice in one of the countries where Ruconest for self administration was formally launched and has been available for one year.

**Main study objective:**

The main objectives of this study are:

- to evaluate the HCPs awareness of the need to take a careful history of rabbit

allergy, the need for monitoring for hypersensitivity reactions and knowing what action to take as a measure of the effectiveness of the educational materials.

- to evaluate whether the patient and prescriber checklists, and patient diary have been useful in training patients to enable safe and effective use of Ruconest and that key safety messages are understood by the prescriber and communicated to their patients as a measure of the effectiveness of the educational materials.

A secondary study objective of this study is to evaluate whether the reporting rate of adverse events related to hypersensitivity reactions after administration of Ruconest has changed (based on data from routine pharmacovigilance reporting and PAS study C1 1412).

## Study Design

### **Non-interventional study design**

Other

## Study drug and medical condition

### **Medicinal product name**

RUCONEST

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

CONESTAT ALFA

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### **Anatomical Therapeutic Chemical (ATC) code**

(B06AC04) conestat alfa

conestat alfa

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## **Medical condition to be studied**

Hereditary angioedema with C1 esterase inhibitor deficiency

## Population studied

### **Short description of the study population**

All physicians who have received the educational materials in a country where the selfadministration kit for Ruconest has been launched, will be informed on the study by an appropriate Pharming representative.

One year after receipt of the educational materials, the physicians will be asked to participate in an online survey.

All physicians who have prescribed Ruconest (vial-only and/or self-administration kit) to patients with hereditary angioedema (HAE) at least once during the 12 preceding months will be eligible for participation.

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### **Estimated number of subjects**

20

## Study design details

### **Setting**

The CRO, PAINT-Consult, or directly the Sponsor Pharming Technologies will send an invitation to participate in the survey to the HCPs who received the educational materials.

The survey has been designed to take no more than 30 minutes.

The response rates will be monitored to keep track of the number of completed questionnaires.

Participating HCPs will have the option of receiving compensation for their time

and effort.

The amount varies by country and is determined by national laws and reimbursement policies.

Similarly, it will take time for physicians to treat a sufficient number of patients and gain experience in prescribing patients with Ruconest for self-administration and receive feedback from patients.

Therefore, the questionnaire will be distributed one year after launch of the self-administration kit.

Furthermore, the frequency of HAE attacks varies between patients (ranging from one attack per year, up to more than once-weekly attacks) and physicians are known to see the patients generally once or twice a year.

Due to the limited number of EU treatment centres and physicians treating HAE patients, the duration and extent of this study is intended to encompass a minimum of 20 completed questionnaires from at least 4 different countries.

The distribution and collection of the questionnaires will continue for at least one year after first start of data collection.

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## **Comparators**

None

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## **Outcomes**

The outcome of the survey is the proportion of physicians that correctly respond to individual items of the questionnaire.

The proportion responding correctly will be tabulated separately for each item.

Physician's demographic information will be collected in order to further characterise the respondent population.

This will include country, type of medical practice, and (range of) number of HAE patients treated.

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## **Data analysis plan**

Data analysis will be descriptive. Awareness, knowledge, and adherence will be evaluated and results will be expressed as percentages and means by question and HCP, as applicable. No formal hypothesis testing will be conducted.

The following items will be reported, as appropriate:

- number of HCPs receiving the (updated) educational materials pack
- number of questionnaires sent out to HCPs
- number and percentage of HCPs eligible and ineligible for participation
- number and percentage of HCPs who completed the questionnaire
- frequency distribution of responses to each question

The outcomes will be summarised for all countries combined, and per country if possible. Additional analyses may be performed as needed.

Physicians' general medical practice and demographic data are intended to explore possible differences between physician's subsets in understanding, knowledge and use of the educational materials.

## 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 source(s), other**

The primary data source will consist of the completed questionnaires that are filled in by the HCPs (surveys).

Other data sources include the company's ADR safety database and data from the European registry study (PAS study C1 1412) for the evaluation of adverse events related to hypersensitivity reactions and other immunogenic/allergy related adverse events.

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### **Data sources (types)**

Other

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### **Data sources (types), other**

Survey 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