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

First published: 21/07/2025

Last updated: 01/06/2026

Study Finalised

Administrative details

EU PAS number

EUPAS1000000680

Study ID

1000000680

DARWIN EU® study

No

Study countries

European Union

United Kingdom

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

Study status

Finalised

Research institutions and networks

Institutions

Pharming Technologies

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Institution

Contact details

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Primary lead investigator

Susan Kantenwein

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 28/02/2020

Actual: 11/02/2020

Study start date
Planned: 18/09/2020

Actual: 18/12/2020

Date of final study report
Planned: 31/01/2026

Actual: 26/01/2026

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

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

EU RMP category 3 (required)

Methodological aspects

Study type

Study topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Safety study (incl. comparative)

Data collection methods:

Combined primary data collection and secondary use of data

Study design:

This is a cross-sectional survey among physicians who have received the updatededucational 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

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

CONESTAT ALFA

Anatomical Therapeutic Chemical (ATC) code

(B06AC04) conestat alfa

conestat alfa

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.

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.

Comparators

None

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.

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.

Summary results

The small number of participating HCPs were generally aware of the need to take a careful
history of rabbit allergy, the need to monitor for hypersensitivity reactions, and to verify whether
the safety messages were understood by the patient and HCP, and what actions need to be taken
when hypersensitivity reactions occur.
The patient/prescriber checklists and patient diary were used by the participants 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, but not always provided to the patient.
Overall, the educational materials were generally well used and perceived as useful, and the main
safety messages were understood.
Based on the low numbers of responders and of the available safety information, no conclusions
can be drawn from the impact of the educational materials on the safety of using Ruconest in
relation to hypersensitivity.

Documents

Study report

PASS Ruconest Educ Mat Survey Report - redacted.pdf (364.14 KB)

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.

Data sources (types)

Other

Data sources (types), other

Suvey 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