

# Hereditary angioedema (HAE) multi-national survey study

<b>Submission date</b> 31/10/2022	<b>Recruitment status</b> No longer recruiting	<input type="checkbox"/> Prospectively registered <input checked="" type="checkbox"/> Protocol
<b>Registration date</b> 31/10/2022	<b>Overall study status</b> Completed	<input type="checkbox"/> Statistical analysis plan <input type="checkbox"/> Results
<b>Last Edited</b> 31/10/2022	<b>Condition category</b> Circulatory System	<input type="checkbox"/> Individual participant data <input type="checkbox"/> Record updated in last year

## Plain English summary of protocol

### Background and study aims

Takeda (a pharmaceutical company sponsoring this study) is conducting this study to understand the impact of hereditary angioedema (HAE; a disorder characterized by recurrent episodes of severe swelling) on patients and their caregivers and the burden of illness for individuals with HAE and their caregivers.

### Who can participate?

Adult patients with any type of HAE, caregivers of adult patients, or caregivers of pediatric patients.

### What does the study involve?

**HAE Patients** - If you agree to participate, you will complete an online survey. Patients will be asked questions about their experiences living with HAE as well as questions regarding socio-demographic background and clinical history. These questions give us some basic information about you (e.g. age, employment status, highest level of education), in addition to obtaining more specific information about your clinical condition (e.g. symptoms, type of diagnosis, current medications, etc.). The survey will take approximately 40 minutes to complete.

**Caregivers of Adults** - If you agree to participate, you will complete an online survey. Caregivers will be asked questions about their experiences as a caregiver to an adult patient diagnosed with HAE, as well as questions regarding your socio-demographic background (e.g. age, employment status, highest level of education). These questions give us some basic information about you. The survey will take approximately 30 minutes to complete.

**Caregivers of Children** - If you agree to participate, you will complete an online survey. Caregivers will be asked questions about their child/adolescent's experiences living with HAE as well as questions about your experiences as a caregiver, as well as questions regarding socio-demographic background. These questions give us some basic information about you (e.g. age, employment status, highest level of education). The survey will take approximately 30 minutes to complete.

What are the possible benefits and risks of participating?

There are no known physical risks to participating in this study. There may be questions that make you uncomfortable while completing the online survey. This is not a test—there are no “right” or “wrong” answers; however, if you are uncomfortable answering a question you can leave it blank. There is little risk of loss of confidentiality since we will not collect any identifying information from you on the survey.

Where is the study run from?

Takeda Pharmaceuticals (Switzerland)

When is the study starting and how long is it expected to run for?

September 2019 to February 2023.

Who is funding the study?

Takeda Pharmaceuticals (Switzerland)

Who is the main contact?

Dr Niall Conlon, conlonn1@tcd.ie

## Contact information

### Type(s)

Scientific

### Contact name

Mrs Marie de la Cruz

### Contact details

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## Additional identifiers

### Protocol serial number

ICON 0238-0556

## Study information

### Scientific Title

Burden of hereditary angioedema (HAE) and impact on quality of life: a multi-national survey of patients and caregivers

### Study objectives

Non-interventional cross-sectional web-based survey of 1) adult ( $\geq 18$  years of age) patients with a self-reported diagnosis of HAE; 2) caregivers of pediatric patients ( $\leq 17$  years of age) diagnosed

with HAE; and 3) caregivers of adult ( $\geq 18$  years of age) patients diagnosed with HAE in the following countries: Argentina, Brazil, Colombia, Croatia, Denmark, Hungary, Ireland, Norway, Poland, Portugal, Romania, and Sweden.

### **Ethics approval required**

Old ethics approval format

### **Ethics approval(s)**

Approved 05/09/2022, SJH/TUH Research Ethics Committee (Tallaght University Hospital, Tallaght, Dublin 24, D24NR0A; +353 (01) 414 2199; researchethics@tuh.ie), ref: 1067

### **Study design**

Non-interventional cross-sectional web-based survey

### **Primary study design**

Observational

### **Study type(s)**

Quality of life

### **Health condition(s) or problem(s) studied**

Hereditary angioedema quality of life for patients and caregivers

### **Interventions**

ICON, on behalf of Takeda, is conducting an online survey assessing the quality of life of patients with hereditary angioedema and their caregivers.

The HAE multi-national survey study is an online survey and will target at least 300 adult persons with a self-reported diagnosis of HAE (type I, II, normal functioning C1-INH, and unknown) and up to 250 caregivers of pediatric HAE patients and up to 200 caregivers of adult HAE patients (type I, II, normal functioning C1-INH, and unknown) combined in the following countries: Argentina, Brazil, Colombia, Croatia, Denmark, Hungary, Ireland, Norway, Poland, Portugal, Romania, and Sweden.

### **Intervention Type**

Other

### **Primary outcome(s)**

Measured using an online survey at a single time point:

1. Perceived control of angioedema measured with the Angioedema Control Test (AECT)
2. Quality of Life measured with the Angioedema Quality-of-Life Questionnaire (AE-QoL)
3. Work impacts measured with the Work Productivity and Activity Impairment Questionnaire - General Health (WPAI-GH)
4. Overall health measured with the Medical Outcomes Study Short Form 12-Item Questionnaire (SF-12)
5. Anxiety and Depression measured with the Hospital Anxiety and Depression Scale (HADS)
6. De novo questions about HAE attacks, medications used, and life impacts of the condition

### **Key secondary outcome(s)**

There are no secondary outcome measures

### **Completion date**

28/02/2023

## Eligibility

### Key inclusion criteria

1. Self-reported diagnosis of HAE (Types I, II, normal functioning C1-INH, or unknown type)
2. Age 18 years or older
3. Experienced at least 1 episode of angioedema or prodromal symptoms within the last year
4. Has been treated with a prescription medication for an angioedema attack within the last 2 years
5. Able to understand and provide consent
6. Willing to complete a web-based survey
7. Adequate fluency in the target language in which the survey is designed:
  - 7.1. Spanish (Argentina & Colombia)
  - 7.2. Brazilian Portuguese (Brazil)
  - 7.3. Croatian (Croatia)
  - 7.4. Danish (Denmark)
  - 7.5. Hungarian (Hungary)
  - 7.6. English (Ireland)
  - 7.7. Norwegian (Norway)
  - 7.8. Polish (Poland)
  - 7.9. Portuguese (Portugal)
  - 7.10. Romanian (Romania)
  - 7.11. Swedish (Sweden)

### Participant type(s)

Patient

### Healthy volunteers allowed

No

### Age group

Adult

### Lower age limit

18 years

### Sex

All

### Key exclusion criteria

Does not meet inclusion criteria

### Date of first enrolment

01/08/2022

### Date of final enrolment

31/01/2023

## Locations

## **Countries of recruitment**

Argentina

Brazil

Colombia

Croatia

Denmark

Germany

Hungary

Ireland

Norway

Poland

Portugal

Romania

Sweden

## **Study participating centre**

### **Consultorio Privado Dr. Zwiener**

R. Caamaño 1060

Buenos Aires

Argentina

B1631BUV

## **Study participating centre**

### **Fundação do ABC - Centro Universitário FMABC**

Av.Lauro Gomes, 2000. Prédio CEPES. 1º Andar. Sala 60. Vila Sacadura Cabral.

Santo André/SP

Brazil

CEP 09060-870

## **Study participating centre**

### **Clínica San José**

Cl. 13 #5-86, Cúcuta, Norte de Santander

San José de Cúcuta

Colombia  
540001

**Study participating centre**

**St James Hospital**

James Street  
Dublin  
Ireland  
DO8 NHY1

**Study participating centre**

**Centro Hospitalar Universitário Lisboa Norte**

Av. Prof. Egas Moniz MB  
Lisbon  
Portugal  
1649-028

**Study participating centre**

**Semmelweis Egyetem; Belgyógyászati és Hematológiai Klinika**

Szentkirályi utca 46.  
Budapest  
Hungary  
1088

**Study participating centre**

**University of Southern Denmark**

Hudklinikken Kolding, Buen 3  
Kolding  
Denmark  
6000

**Study participating centre**

**General Hospital Šibenik**

Ul. Stjepana Radića 83  
Šibenik  
Croatia  
22000

**Study participating centre**

**University of Stavanger, Faculty of Health Sciences**

Hudavdelningen Helse Stavanger HF Stavanger Universitetssykehus Postboks 8100  
Stavanger  
Norway  
4068

**Study participating centre****Cabinet Medical Alergologie si imunologie clinica**

Strada Teiului 14  
Timișoara  
Romania  
300681

**Study participating centre****Wojskowy Instytut Medyczny Klinika Chorób Wewnętrznych, Pneumonologii, Alergologii i Immunologii Klinicznej**

Ul. Szaserów 128  
Warszawa  
Poland  
04-141

**Study participating centre****HZRM Häemophilie-Zentrum Rhein Main GmbH**

Hessenring 13a, Geb. G  
Mörfelden Walldorf  
Germany  
64546

**Study participating centre****General Hospital in Falun**

Falu lasarett Hudavdelningen  
Falun  
Sweden  
791 82

**Sponsor information****Organisation**

Takeda (Switzerland)

ROR

<https://ror.org/002ysmy84>

## Funder(s)

### Funder type

Industry

### Funder Name

Takeda Pharmaceutical Company

### Alternative Name(s)

Takeda, Takeda Pharmaceutical Company Limited, Chobei Takeda & Co., Ltd., Takeda Pharmaceutical Industries, Ltd., Takeda Chemical Industries, Ltd., , Takeda Yakuhin Kōgyō kabushiki gaisha, TPC

### Funding Body Type

Government organisation

### Funding Body Subtype

For-profit companies (industry)

### Location

Japan

## Results and Publications

### Individual participant data (IPD) sharing plan

The datasets generated during and/or analysed during the current study are not expected to be made available due to the data being the proprietary information of Takeda Pharmaceuticals.

### IPD sharing plan summary

Not expected to be made available

### Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
<a href="#">Protocol file</a>	version 2.0	25/03/2022	31/10/2022	No	No