ISRCTN85479564 https://doi.org/10.1186/ISRCTN85479564

Hereditary angioedema (HAE) multi-national survey study

Submission date	Recruitment status	[_] Prospectively registered
31/10/2022	No longer recruiting	[X] Protocol
Registration date	**Overall study status**	[_] Statistical analysis plan
31/10/2022	Completed	[X] Results
Last Edited	**Condition category**	[_] Individual participant data
02/11/2026	Circulatory System	

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
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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 (≥18 years of age) patients with a self-reported diagnosis of HAE; 2) caregivers of pediatric patients (≤17 years of age) diagnosed

with HAE; and 3) caregivers of adult (≥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

Primary study design
Observational

Study design
Non-interventional cross-sectional web-based survey

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 an 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
Mixed

Lower age limit
18 Years

Upper age limit
100 Years

Sex
All

Total final enrolment
0

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 Sibenik
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 HÃ¤mophilie-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

IPD sharing plan summary
Not expected to be made available

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Results article		12/02/2026	02/11/2026	Yes	No
Results article		19/02/2026	02/11/2026	Yes	No
Protocol file	version 2.0	25/03/2022	31/10/2022	No	No