Garadacimab Real-World Treatment Outcomes of Effectiveness, Safety, and Quality-Of-Life in Patients With HAE (GREAT) Study Design

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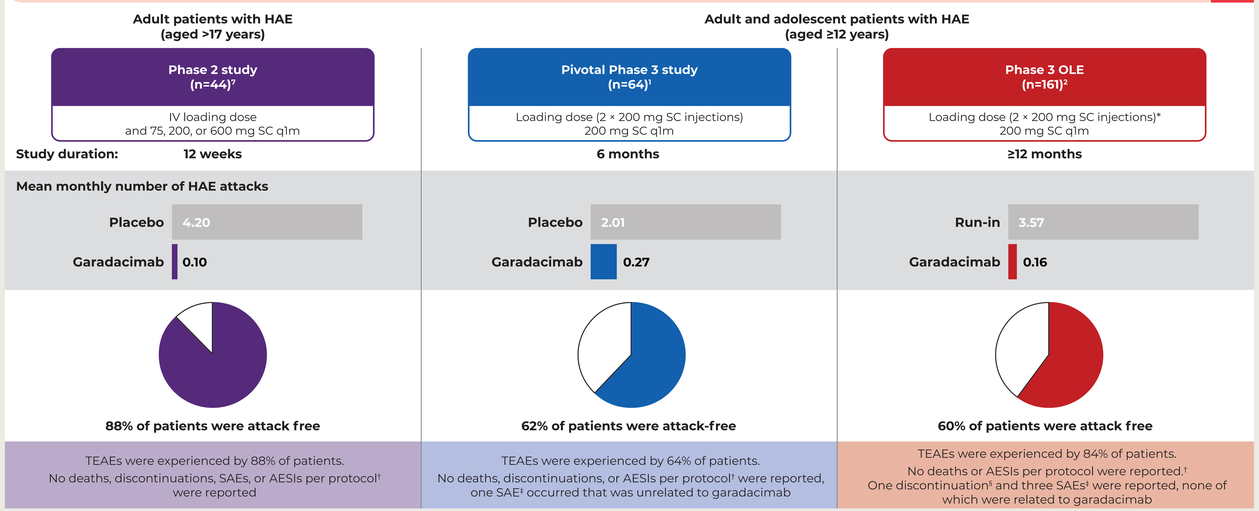
CONCLUSIONS

- **This prospective, noninterventional observational cohort study (the GREAT study) will gather the first real-word data on effectiveness, safety, health-related quality of life (HRQoL), and healthcare resource utilization (HCRU) in patients with hereditary angioedema (HAE) receiving garadacimab long-term prophylaxis (LTP) in routine clinical practice**

BACKGROUND

- HAE is characterized by recurrent, unpredictable, debilitating, and potentially life-threatening attacks of angioedema[1-6]
- Garadacimab is a first-in-class, fully human, anti-activated factor XII monoclonal antibody under evaluation as LTP for HAE attacks[1,2,6]
- Garadacimab has demonstrated durable efficacy and favorable long-term safety and tolerability profiles in Phase 2, pivotal Phase 3 (VANGUARD), and Phase 3 open-label extension (OLE) studies[1,2,7]
- Garadacimab has been approved for use in Australia and in the UK, and the European Medicines Agency (EMA) Committee for Medicinal Products for Human Use (CHMP) has adopted a positive opinion, recommending granting regulatory approval for garadacimab as once-monthly LTP for HAE[8]
- Real-world evidence can support treatment decision-making for HAE LTP by providing critical insights from usage in routine clinical practice

GARADACIMAB VANGUARD CLINICAL PROGRAM



*Newly enrolled patients received a loading dose of garadacimab (2 × 200 mg) SC as their first dose; †Abnormal bleeding, thromboembolic, hypersensitivity, or anaphylaxis events; ‡Pivotal Phase 3 study: laryngeal attack (n=1) managed with overnight hospitalization, unrelated to garadacimab, patient made a full recovery; Phase 3 OLE: COVID-19, n = 2; abdominal HAE attack, n = 1 unrelated to garadacimab, all recovered/resolved; §Moderate ISR: irritation.
AESI, adverse event of special interest; HAE, hereditary angioedema; ISR, injection site reaction; IV, intravenous; OLE, open-label extension; q1m, once monthly; SAE, serious adverse event; SC, subcutaneous; TEAE, treatment-emergent adverse event.

OBJECTIVE

- Following granting of regulatory approval for garadacimab, the aim of the GREAT study is to generate long-term effectiveness, safety, HRQoL, and HCRU data on garadacimab for HAE LTP in a real-world setting

PROSPECTIVE, NONINTERVENTIONAL, OBSERVATIONAL COHORT STUDY DESIGN

Eligibility

Inclusion criteria
- Diagnosed with HAE
- Aged ≥12 years
- Receiving on-label treatment with garadacimab
- Able to use an electronic device for data collection

Exclusion criteria
- Concomitant diagnosis with other forms of angioedema
- Participation in another ongoing interventional clinical study

Retrospective data collection
12 months prior to initiation

Treatment (24 months)

Garadacimab 200 mg SC
q1m*

Follow-up (30 days after discontinuation)†

*Initial loading dose of 400 mg administered SC as two 200 mg injections on the first day of treatment followed by a monthly dose of 200 mg; †Includes any discontinuation during the study and following completion of 24 months.
HAE, hereditary angioedema; q1m, once monthly; SC, subcutaneous.

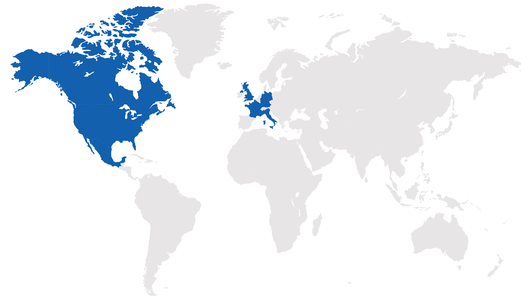
Study objectives

The GREAT study will evaluate:
- Characteristics of the population of patients with HAE receiving garadacimab in a real-world setting
- HAE attack rate while on-treatment with garadacimab vs pre-enrollment baseline
- How quickly control of HAE attacks is achieved with garadacimab
- The proportion of patients achieving attack-free status and the duration for which they remain attack-free
- Real-world safety of garadacimab
- The real-world impact of garadacimab on HRQoL, productivity, and use of on-demand therapy
- HCRU
- Treatment adherence and patient satisfaction
- Patient preference
- Treatment patterns (including retrospectively from medical records)
- Economic impact of garadacimab LTP treatment

Target enrollment is 200 patients with HAE

The study is estimated to be completed in 2029.

Global recruitment is planned across 30 centers



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