Efficacy And Safety of Garadacimab Via Autoinjector/Pre-Filled Pen For Hereditary Angioedema Long-Term Prophylaxis – Interim Results From A Phase 3 Open-Label Extension Study

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CONCLUSIONS

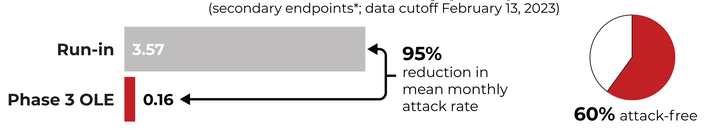
- **Long-term prophylaxis with garadacimab administered via autoinjector/pre-filled pen (AI/PFP) showed durable protection against hereditary angioedema (HAE) attacks with a favorable safety profile**
- **Garadacimab administered via AI/PFP reduced the frequency of HAE attacks and use of on-demand treatment (ODT) versus run-in**

BACKGROUND

- HAE causes recurrent, unpredictable, debilitating, and potentially life-threatening attacks[1-5]
- Garadacimab is a first-in-class, fully human, anti-activated factor XII (FXIIa) monoclonal antibody under evaluation for the long-term prophylaxis of HAE attacks[1,2,5]
- Garadacimab has demonstrated early and durable efficacy and favorable long-term safety profile when administered subcutaneously once-monthly via pre-filled syringe/needle safety device (PFS/NSD) in Phase 2, pivotal Phase 3 (VANGUARD), and Phase 3 open-label extension (OLE) studies[1,2,6]
- Administration by AI/PFP may offer increased convenience allowing rapid, uniform dose delivery and providing ease of use for all patients including those of advanced age or with disabilities affecting strength and dexterity[7-12]

EFFICACY WITH PFS/NSD[2]

Phase 3 OLE study (N=161)
(secondary endpoints*; data cutoff February 13, 2023)

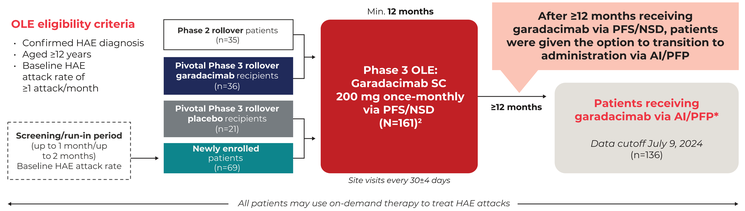


*The primary endpoint was long-term safety.

OBJECTIVE

- To report efficacy and safety data in patients with HAE receiving garadacimab as long-term prophylaxis via AI/PFP from the ongoing Phase 3 OLE (NCT04739059)

STUDY DESIGN[1,2,6]



*At data cutoff of July 9, 2024, not all patients in the Phase 3 OLE had completed 12 months of the study, and therefore not all 161 patients were eligible to transition to AI/PFP.
AI/PFP, autoinjector/pre-filled pen; HAE, hereditary angioedema; OLE, open-label extension; PFS/NSD, pre-filled syringe/needle safety device; SC, subcutaneous.

RESULTS

Baseline characteristics of patients who received garadacimab via AI/PFP

Characteristic	Patients receiving garadacimab via AI/PFP (n=136)
Mean (SD) age, years	42.1 (15.01)
Sex, n (%)	
Female	80 (58.8)
Male	56 (41.2)
Race, n (%)	
White	112 (82.4)
Asian	21 (15.4)
Black	1 (0.7)
Other	2 (1.4)
Mean (SD) BMI	27.7 (5.70)
Median (IQR) duration of AI/PFP use, months	9.43 (7.39–11.01)

94.9% of patients received garadacimab via **AI/PFP** for **≥6 months**

AI/PFP, autoinjector/pre-filled pen; BMI, body mass index; HAE, hereditary angioedema; IQR, interquartile range; SD, standard deviation.

Safety profile of garadacimab administered via AI/PFP

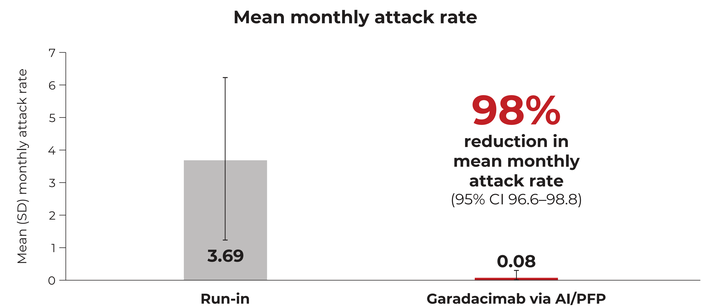
Patients with TEAEs, n (%)	Patients receiving garadacimab via AI/PFP (n=136)
Patients with ≥1 TEAE	58 (42.6)
Related to study treatment	3 (2.2)
Leading to discontinuation	0 (0)
Leading to death	0 (0)
Investigator-identified AESIs per protocol*	1 (0.7)
Related to garadacimab	0
SAEs†	3 (2.2)
Related to garadacimab	0
By severity	
Mild	32 (23.5)
Moderate	37 (27.2)
Severe	5 (3.7)

Garadacimab-related TEAEs were ISRs, all mild in severity

No AESIs per protocol* or SAEs were considered garadacimab-related

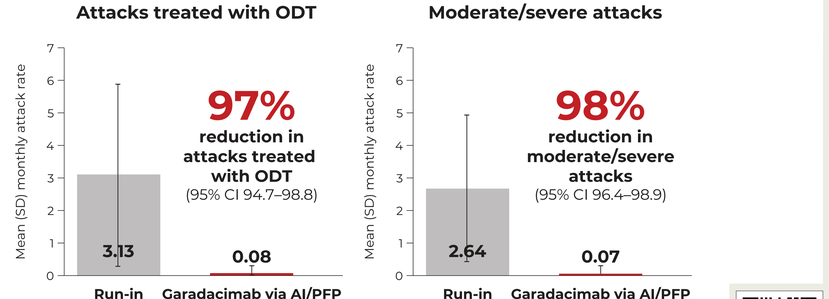
*Allergic reaction to food, not related to study treatment; this was incorrectly reported as an AESI (AESI per protocol defined as thromboembolic or abnormal bleeding events, severe hypersensitivity, or anaphylaxis) and was subsequently correctly counted as an SAE; †Allergic reaction to food, fractured fingers, fractured skull, n=1 each, none related to garadacimab.
AESI, adverse event of special interest; AI/PFP, autoinjector/pre-filled pen; ISR, injection-site reaction; SAE, serious adverse event; TEAE, treatment-related adverse event.

Garadacimab treatment via AI/PFP reduced time-normalized mean monthly HAE attack rate versus run-in



AI/PFP, autoinjector/pre-filled pen; CI, confidence interval; HAE, hereditary angioedema; SD, standard deviation.

Garadacimab treatment via AI/PFP reduced time-normalized number of attacks treated with ODT (left) and moderate/severe attacks versus run-in (right)



AI/PFP, autoinjector/pre-filled pen; CI, confidence interval; ODT, on-demand treatment; SD, standard deviation.

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DISCLOSURES

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