

# Clinical meaningfulness of improvements in quality of life observed with garadacimab treatment in patients living with hereditary angioedema: Results from the pivotal Phase 3 (VANGUARD) study

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## CONCLUSIONS

- Patients with hereditary angioedema (HAE) were more likely to experience a clinically meaningful improvement in Angioedema Quality of Life Questionnaire (AE-QoL) scores when receiving garadacimab compared to placebo at meaningful score difference (MSD) thresholds of 6 and 15, irrespective of the method used

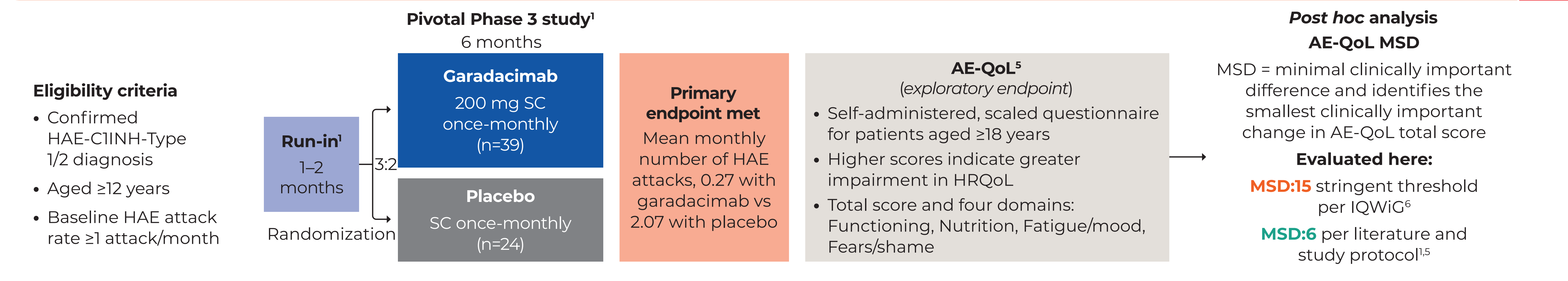
## BACKGROUND

- HAE is a rare genetic disorder that causes recurrent, unpredictable, debilitating, and potentially life-threatening attacks of angioedema, which substantially impair health-related quality of life (HRQoL)<sup>1-3</sup>
- Recent US Food and Drug Administration (FDA) patient-focused drug development guidance suggests using MSD to identify clinically meaningful changes to HRQoL
- Garadacimab is a first-in-class, fully human monoclonal antibody targeting activated factor XII, the key initiator of the kallikrein-kinin system<sup>1</sup>
- Garadacimab demonstrated efficacy versus placebo with a favorable safety profile in the 6-month pivotal Phase 3 (VANGUARD) study for long-term prophylaxis of HAE attacks and was associated with HRQoL improvements<sup>1</sup>

## OBJECTIVE

- To further evaluate the impact of garadacimab versus placebo on HRQoL in a *post hoc* analysis of the Phase 3 study<sup>1</sup> using AE-QoL data analyzed and interpreted according to the recent FDA guidance on evaluating the meaningfulness of treatment benefit<sup>4</sup>

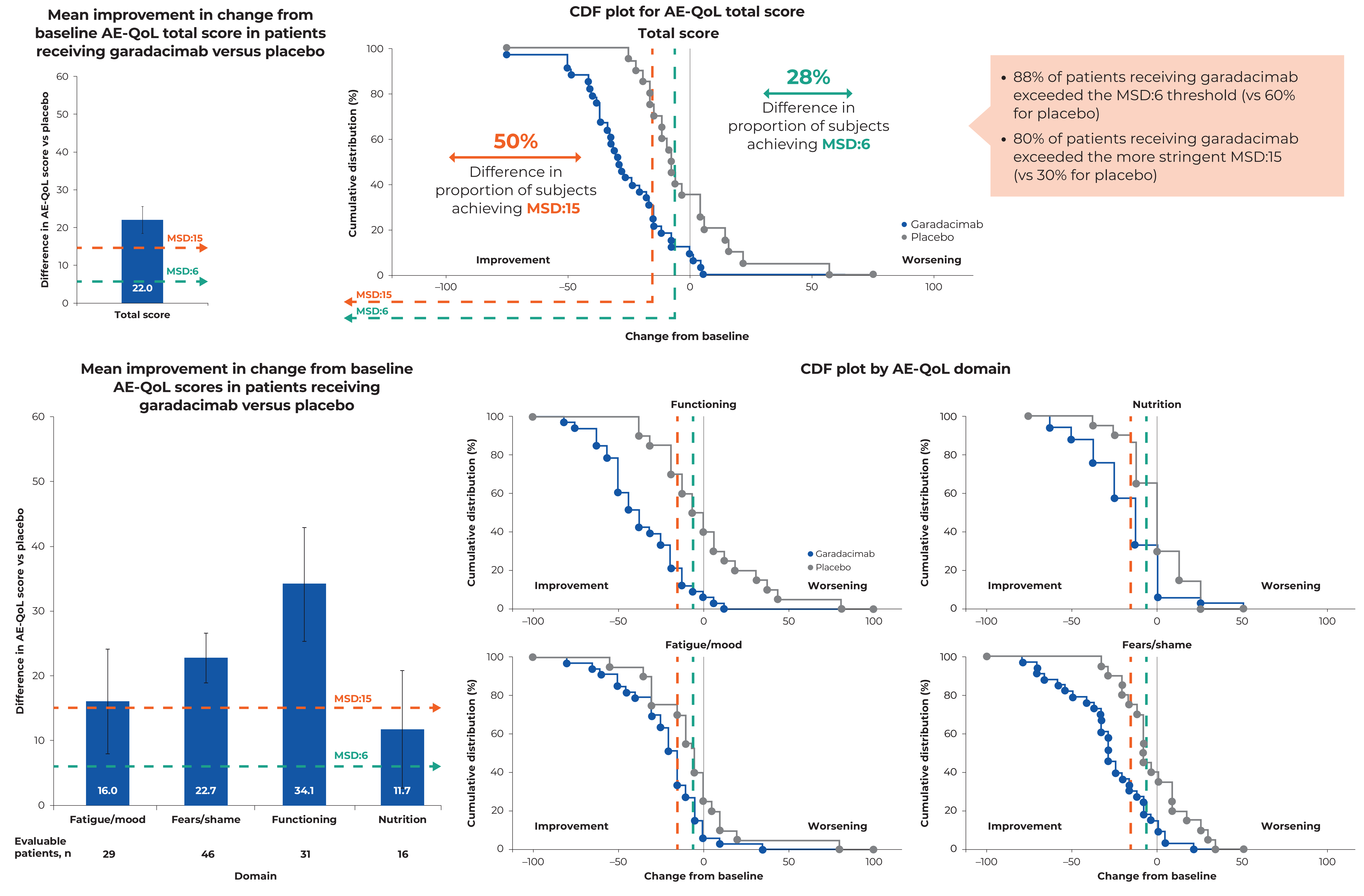
## PIVOTAL PHASE 3 STUDY DESIGN AND POST HOC ANALYSIS



AE-QoL, angioedema quality of life questionnaire; HAE, hereditary angioedema; HAE-C1INH, HAE with C1 inhibitor deficiency/dysfunction; HRQoL, health-related quality of life; IQWiG, Institute for Quality and Efficiency in Health Care; MSD, meaningful score difference; SC, subcutaneous.

## RESULTS

### Garadacimab elicited clinically meaningful improvements in AE-QoL scores versus placebo



AE-QoL, angioedema quality of life questionnaire; CDF, cumulative distribution frequency; MSD, meaningful score difference.

## REFERENCES

1. Craig TJ et al. *Lancet* 2023;401:1079–1090; 2. Bork K et al. *Allergy Asthma Clin Immunol* 2021;17:40; 3. Maurer M et al. *Allergy* 2022;77:1961–1990; 4. IQWiG general methods v7.0. Available at: [https://www.iqwig.de/methoden/general-methods\\_version-7-0.pdf](https://www.iqwig.de/methoden/general-methods_version-7-0.pdf) (accessed October 2024); 5. Weller K et al. *Allergy* 2016;71:1203–1209; 6. FDA PFDD G4 draft guidance document. Available at: <https://www.fda.gov/regulatory-information/search-fda-guidance-documents/patient-focused-drug-development-incorporating-clinical-outcome-assessments-endpoints-regulatory> (accessed October 2024).

## DISCLOSURES

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