

Attack-free probability modeling of garadacimab long-term prophylaxis for hereditary angioedema

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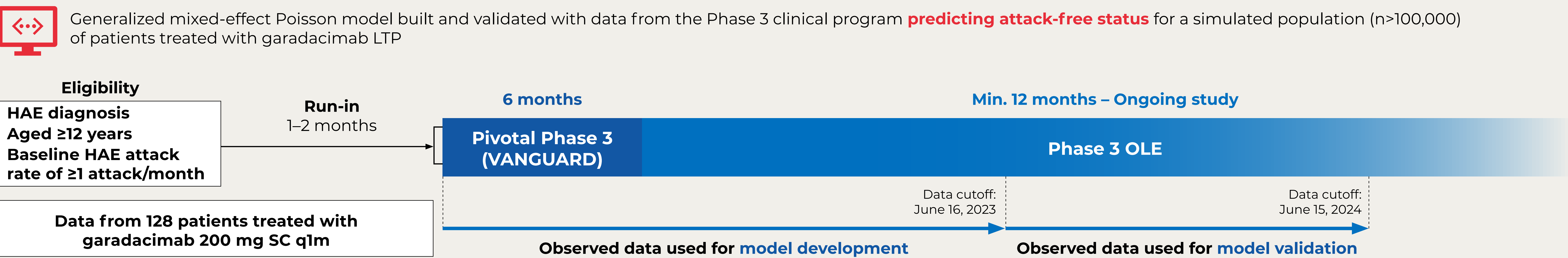
BACKGROUND

- HAE is a genetic disorder characterized by recurrent, unpredictable and potentially life-threatening attacks of angioedema^{1,2}
- The WAO/EAACI treatment goals of total disease control and normalization of life can only be achieved with LTP²

Garadacimab

- First-in-class, fully human mAb inhibiting FXIIa approved for routine prevention of recurrent HAE attacks in patients aged ≥12 years in Australia, Europe, Japan, Switzerland, and the UK^{3–8}
- Early onset and durable efficacy with a favorable long-term safety profile in the pivotal Phase 3 (VANGUARD) and ongoing Phase 3 OLE (NCT04739059) studies^{9–11}
- Clinical prediction models in rare diseases may be useful to aid diagnosis, appropriate management and decision-making^{12,13}

STUDY DESIGN OF THE PHASE 3 CLINICAL PROGRAM AND DETAILS OF THE ATTACK-FREE PROBABILITY MODEL



FOCUS OF THIS PRESENTATION

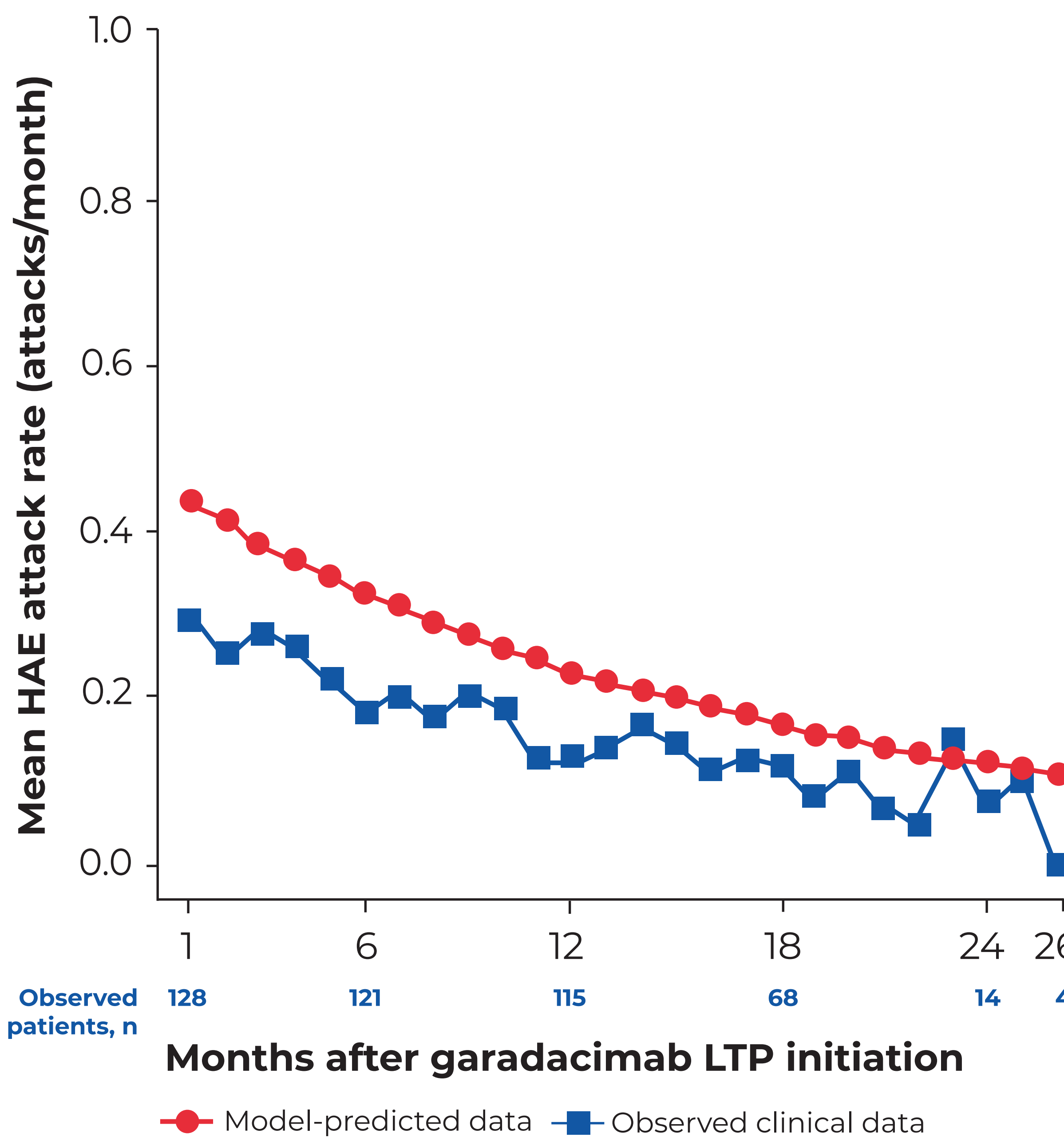
TO PRESENT RESULTS FROM A PROBABILITY MODEL PREDICTING THE ATTACK-FREE STATUS IN PATIENTS WITH HAE TREATED WITH GARADACIMAB LTP

Predicted variables:

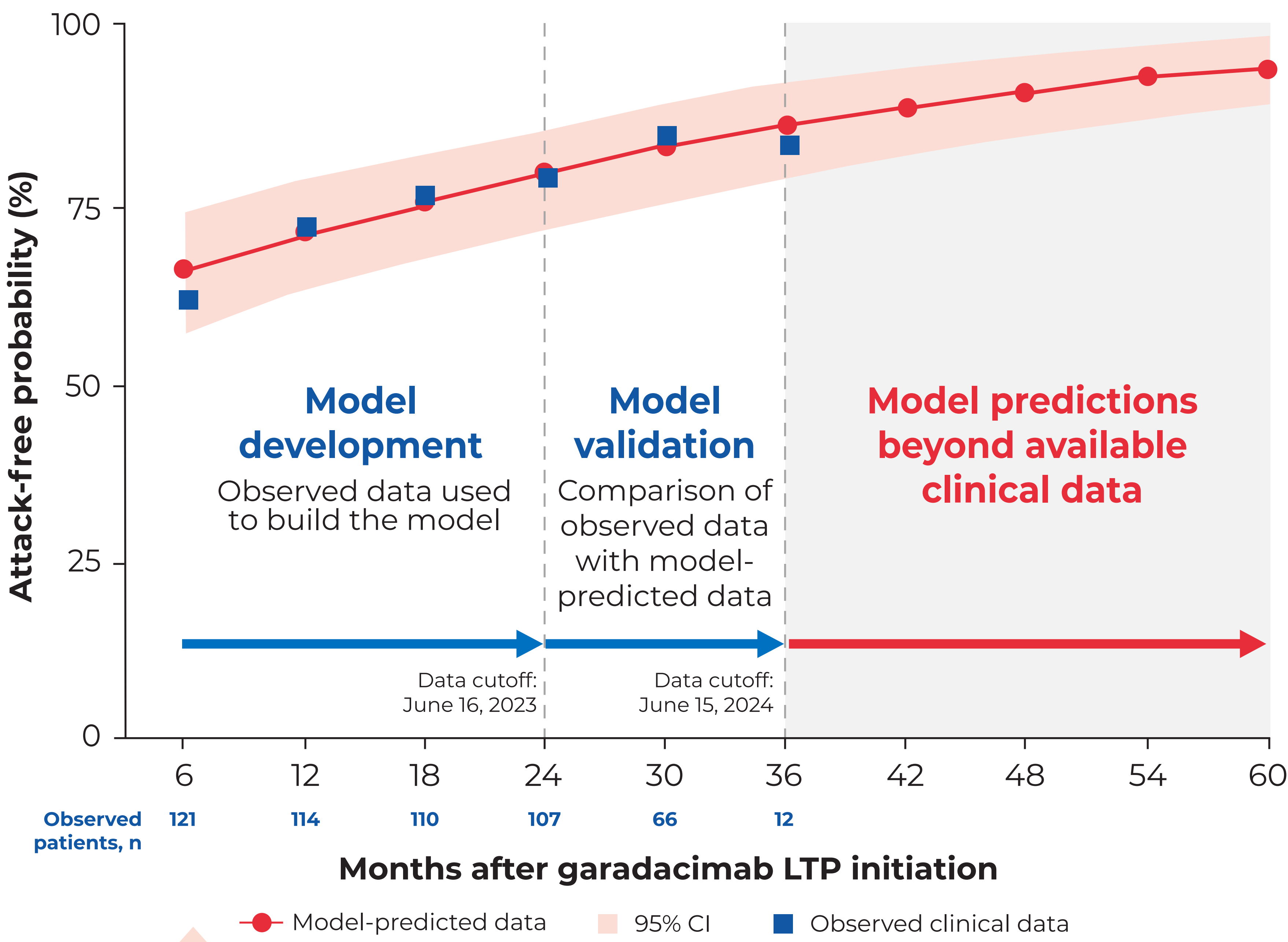
- Monthly HAE attack rates
- Attack-free probabilities by discrete 6-month windows

THE ATTACK-FREE PROBABILITY MODEL ADEQUATELY REFLECTED THE CLINICAL DATA OF PATIENTS TREATED WITH GARADACIMAB LTP

Model-predicted vs observed HAE attack rate

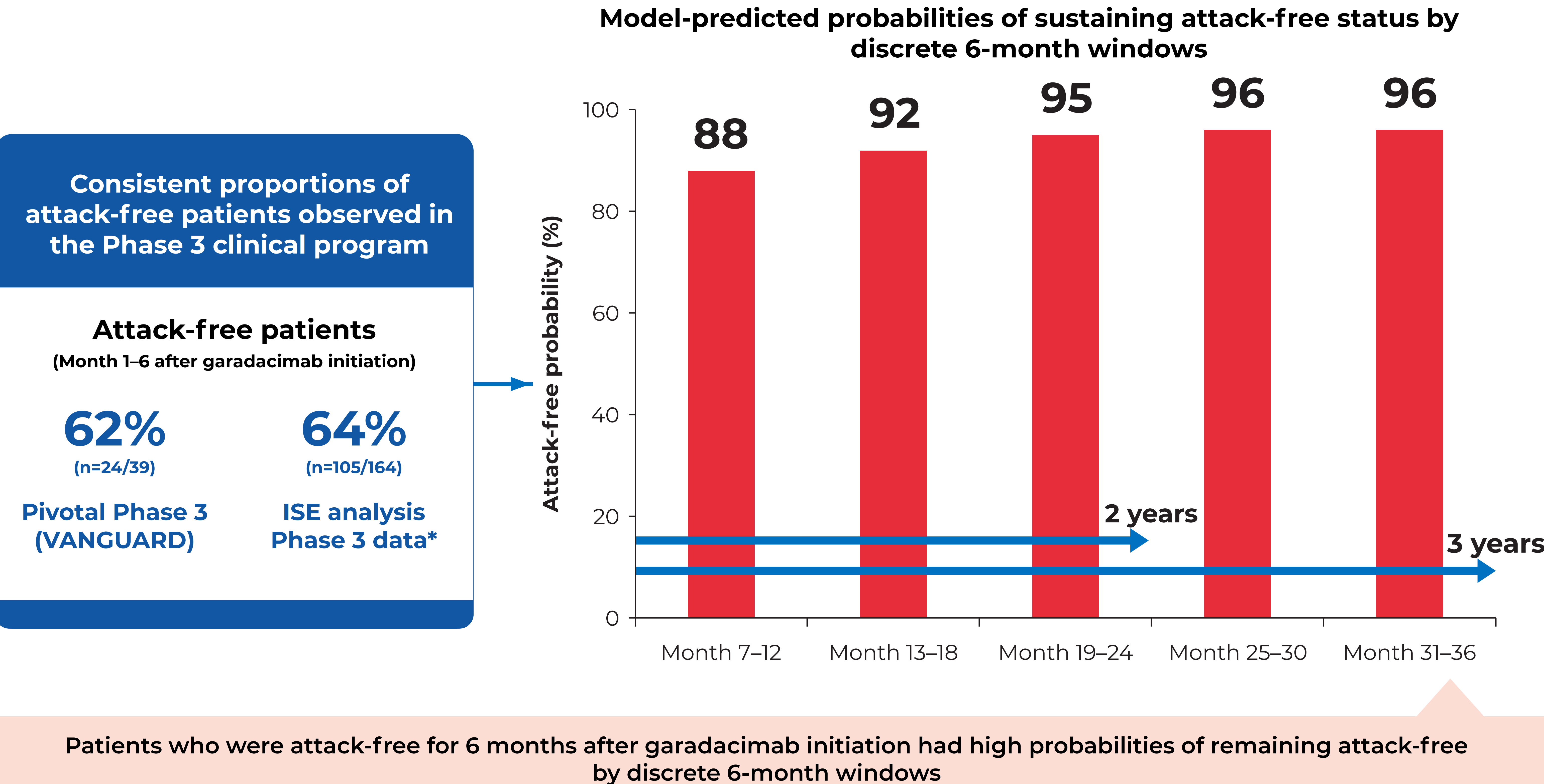


Attack-free probabilities by discrete 6-month windows



The modelled data showed high goodness-of-fit with clinical data: predicted mean monthly HAE attack rates and attack-free probabilities adequately described observed mean monthly HAE attack rates and proportions of attack-free patients

BEING ATTACK-FREE FOR 6 MONTHS AFTER GARADACIMAB LTP INITIATION LED TO A HIGH PROBABILITY OF REMAINING ATTACK-FREE



CONCLUSIONS

- The attack-free probability model adequately described with high goodness-of-fit the clinical data of patients with HAE treated with garadacimab LTP
- High probabilities of sustaining long-term attack-free status were predicted after treatment initiation with garadacimab LTP
- This model adds to the available garadacimab clinical data and could be further explored to predict treatment effect in HAE beyond clinical data

*ISE Month 1–6 data in patients with HAE from the pivotal Phase 3 (VANGUARD) and the Phase 3 OLE (data cutoff: June 16, 2023) studies. CI, confidence interval; FXIIa, activated factor XII; HAE, hereditary angioedema; ISE, Integrated Summary of Efficacy; LTP, long-term prophylaxis; mAb, monoclonal antibody; OLE, open-label extension; q1m, once monthly; SC, subcutaneous.

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Disclosures

This study was funded by CSL Behring LLC. Medical writing support was provided by Anita Toscani, PhD, of Helix, OPEN Health Communications, and was funded by CSL Behring, in accordance with Good Publication Practice guidelines (GPP 2022).

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