News Release

Phase I/II Study in Hemophilia A Suggests NXT007 May Have the Potential to Provide Hemostatic Normalization

- Results from The NXTAGE study part B for people with hemophilia A without factor VIII inhibitors showed that plasma concentration of participants in high dose cohorts reached the predicted normal range of FVIII–equivalent activity, with no treated bleeds observed in the study in those cohorts
- NXT007 was well tolerated in the part B of this study
- NXT007 is a Chugai originated project currently under development for hemophilia A. Three Phase III studies to be initiated in 2026

TOKYO, June 23, 2025 -- Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced today that the first clinical data in people with hemophilia A from a Phase I/II study (NXTAGE) of NXT007 was presented at the International Society on Thrombosis and Haemostasis 2025 Congress in Washington, D.C., United States. NXT007 is a next-generation bispecific antibody for subcutaneous injection, based on Hemlibra (generic name: emicizumab) which is widely used to treat hemophilia A. It is currently under development for hemophilia A.

“We are very excited that this first data in people with hemophilia A, suggests that NXT007 has the potential to provide hemostatic normalization. Based on these data, we plan to initiate three Phase III studies of NXT007 next year, including a head-to-head study with Hemlibra. Treatment in hemophilia A has drastically evolved in recent years, and there are now expectations for treatments with even higher efficacy and even more convenient dosing form. Together with Roche, we will focus on the clinical development of NXT007, aiming to deliver it as a new treatment option as soon as possible to people who need it,” said Dr. Osamu Okuda, Chugai’s President and CEO.

NXTAGE study is a multicenter, Phase I/II clinical trial to evaluate the safety, pharmacokinetics, pharmacodynamics, and efficacy of NXT007. The presented data was from Part B of the study, which was the multiple ascending dose part including Hemlibra-naïve people with severe hemophilia A without FVIII inhibitors aged ≥12 years and <65 years. Participants were included in 4 cohorts, and following 4- to 6-week loading doses, they received subcutaneous maintenance doses of NXT007 at different dosage levels every 4 weeks. The primary analysis was conducted when at least 6 participants had completed 16 weeks of NXT007 treatment in all Part B cohorts.

In Part B, dose dependent increase of NXT007 plasma concentration was observed. Plasma concentrations in the high dose cohorts B-3 and B-4 reached the predicted normal range of FVIII–equivalent activity based on preclinical data[1]. No treated bleeds were

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observed in cohorts B-3 and B-4, in the maintenance dose period. Annualized bleed rates of all cohorts were as follows.

NXTAGE Study Part B Annualized Bleed Rate for Treated Bleeds*

Cohort	Before Enrollment	NXT007 Maintenance Dose Period
B-1	12.83	1.20
B-2	2.17	0.28
B-3	5.44	0
B-4	2.72	0

*Bleeding information before study was collected from 24 weeks before the study in a retrospective manner. ABR was calculated by annualizing the number of bleeding episodes observed during the evaluation period

NXT007 was well tolerated in part B. The number of adverse events (AEs) were not dose-dependent, and AEs leading to treatment withdrawal and serious AEs were not related to NXT007. No thromboembolic events were observed, and the safety profile was tolerable.

[Reference]
Non-Clinical Research Results of Chugai's NXT007 Published in Journal of Thrombosis and Haemostasis (News release issued on November 7, 2023)
https://www.chugai-pharm.co.jp/english/news/detail/20231107160000_1021.html

About NXT007
NXT007 is a bispecific antibody originated from Chugai, designed with the goal of achieving coagulation activities at a level comparable to individuals without hemophilia, while optimizing administration convenience. NXT007 is designed to bind factor IXa and factor X, to provide the cofactor function of factor VIII in people with hemophilia A, who either lack or have impaired coagulation function of factor VIII. By applying Chugai's proprietary antibody engineering technology, FAST-Ig™[2] for the first time, the variable region of Hemlibra has been optimized, aiming to further enhance efficacy. ACT-Ig®[3] was also applied, aiming to further improve antibody pharmacokinetics. Roche decided to in-license the investigational drug in August 2022. Phase I/II clinical trials for hemophilia A are currently ongoing, and three Phase III clinical trials are to be initiated in 2026.

Sources:
1. Yoneyama et.al., Blood (2022) 140 (Supplement 1): 11295–11296
2. Hikaru Koga et al. Efficient production of bispecific antibody by FAST-IgTM and its application to NXT007 for the treatment of hemophilia A, mAbs, 15:1

3. Atsuhiko Maeda et al. Identification of human IgG1 variant with enhanced FcRn binding and without increased binding to rheumatoid factor autoantibody, mAbs, 9:5, 844-853

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