press release

Once-weekly Mim8 is well-tolerated and efficacious in children living with haemophilia A with and without inhibitors

Phase 3 FRONTIER3 interim analysis data presented at EAHAD 2025 showed that 74.3% of children on once-weekly Mim8 prophylaxis experienced zero treated bleeds

Bagsværd, Denmark, 7 February 2025 – Novo Nordisk today announced interim results from the phase 3 FRONTIER3 trial of 70 children (aged 1-11 years old) with haemophilia A with and without inhibitors. The trial initially assessed once-weekly prophylaxis treatment (regular treatment to prevent prolonged and spontaneous bleeding) with investigational Mim8 before giving participants the option to change to once-monthly dosing after 26 weeks. The data were presented at the 18th Annual Congress of the European Association for Haemophilia and Allied Disorders (EAHAD 2025) in Milan, Italy.

The data showed that Mim8 was well-tolerated and efficacious in children with haemophilia A with and without inhibitors. In part one of the FRONTIER3 study, participants with haemophilia A received once-weekly doses of Mim8 administered under the skin for 26 weeks. In part two, participants had the option to continue with once-weekly dosing or move to once-monthly dosing for the remaining 26 weeks. The interim analysis reports on results at the completion of part one of the study, with some preliminary data shared from part two, which continued following this analysis.

For part one of the study in children on once-weekly prophylaxis, the estimated mean (average) annualised bleeding rate (ABR) for treated bleeds was 0.53. The median (middle or central value in the data set) ABR was zero; 74.3% of participants had zero treated bleeds. All children with haemophilia A with inhibitors (n=14) reported zero treated bleeds. After completing the initial 26 weeks of the study, 45% of participants chose to move to once-monthly Mim8, and the rest (55%) remained on the once-weekly dose.

“Managing haemophilia A in young children can be a complex balancing act of delivering ongoing care, minimising time out of school, and ensuring their physical, emotional and social wellbeing is optimised,” said Professor Johnny Mahlangu, lead investigator and Director of the Haemophilia Comprehensive Care Centre at Charlotte Maxeke Johannesburg Hospital at

Novo Nordisk A/S
Global Communication

Novo Alle 1
2880 Bagsværd
Denmark

Tel: +45 4444 8888
www.novonordisk.com

CVR no: 24 25 67 90

University of the Witwatersrand in Johannesburg, South Africa. “The FRONTIER3 interim analysis data are encouraging for families with young children and indicate that Mim8 could offer an efficacious, convenient, flexible dosing option for children, helping to reduce the treatment burden so families can live more normal lives.”

Patient- and caregiver-reported outcomes data from part one of the FRONTIER3 trial with once-weekly Mim8 were also presented, which indicated that Mim8 may reduce treatment burden and improve physical function and quality of life (QoL) in children living with haemophilia A with or without inhibitors. At week 26, 98% of caregivers said they preferred Mim8 to prior treatment, of which 73% said they ‘very strongly’ preferred Mim8 to prior treatment. Additionally, at week 26, positive improvement trends were observed in the mean physical function score (a measure of a child’s ability to perform everyday physical activities such as running) and mean QoL total score, compared to baseline.

“We see the daily challenges faced by children living with haemophilia A and their caregivers. The FRONTIER3 data represent another step forward in our ambition to provide treatment options that place equal focus on safety and efficacy without requiring a compromise on treatment administration and quality of life,” said Ludovic Helfgott, executive vice president for Rare Disease at Novo Nordisk. “Mim8 is designed with the aim to offer treatment flexibility based on individual lifestyles, so it is encouraging to see that patients and caregivers in these analyses prefer Mim8 over their previous treatment. At Novo Nordisk, our commitment to the rare bleeding disorders community compels us to reduce limitations in the lives of children with haemophilia and their families.”

Novo Nordisk expects Mim8 regulatory submission during 2025. Data from the ongoing phase 3 FRONTIER programme will be disclosed at upcoming congresses and in publications in 2025 and 2026.

About haemophilia
Haemophilia is a rare inherited bleeding disorder that impairs the body’s ability to make blood clots, a process needed to stop bleeding. It is estimated to affect approximately 1,125,000 people worldwide[1]. Due to the nature of haemophilia being an x-linked recessive disorder, it often presents differently in males compared to females, with ~88% of people diagnosed with haemophilia worldwide being male[2,3]. There are different types of haemophilia, which are characterised by the type of clotting factor protein that is defective or missing. Haemophilia A is caused by a missing or defective clotting Factor VIII (FVIII).

About Mim8
Mim8 is an investigational Factor VIIIa (FVIIIa) mimetic bispecific antibody designed with the aim to deliver sustained haemostasis for once-weekly, once every two weeks or once-monthly prophylaxis for people living with haemophilia A with and without inhibitors. Administered

under the skin, Mim8 bridges Factor IXa and Factor X. This action replaces Factor VIII, which restores the body's thrombin generation capacity, helping blood to clot. The use of Mim8 in people living with haemophilia A is investigational and not approved by regulatory authorities or available anywhere in the world.

About the FRONTIER3 trial

FRONTIER3 is a phase 3 clinical trial that evaluated the efficacy and safety profile of Mim8 as a prophylaxis treatment in 70 children (aged 1-11 years old) with haemophilia A with inhibitors (n=14) and without inhibitors (n=56)[4]. The study consisted of two parts:

- Part one: All participants received once-weekly Mim8 prophylaxis under the skin for 26 weeks[4,5]
- Part two: For a further 26 weeks, participants chose between receiving Mim8 prophylaxis once-monthly or continuing to receive Mim8 prophylaxis once-weekly[4,5]

In the FRONTIER3 trial, no major safety concerns (deaths, thromboembolic events or severe treatment-emergent adverse events) or clinical evidence of neutralising anti-drug antibodies (ADAs) were observed with Mim8, in line with previous trials. Less than 1% of all injections were reported to have injection site reactions (ISRs).

The FRONTIER clinical programme investigates Mim8 as a prophylaxis treatment for people with haemophilia A with or without inhibitors. The phase 3 programme includes FRONTIER2[6], FRONTIER3[4], FRONTIER4[7], and FRONTIER5[8].

About Novo Nordisk

Novo Nordisk is a leading global healthcare company founded in 1923 and headquartered in Denmark. Our purpose is to drive change to defeat serious chronic diseases, built upon our heritage in diabetes. We do so by pioneering scientific breakthroughs, expanding access to our medicines, and working to prevent and ultimately cure disease. Novo Nordisk employs about 76,300 people in 80 countries and markets its products in around 170 countries. For more information, visit novonordisk.com, Facebook, Instagram, X, LinkedIn and YouTube.

Contacts for further information

Media:

Ambre James-Brown
+45 3079 9289
abmo@novonordisk.com

Liz Skrbkova (US)
+1 609 917 0632
lzsk@novonordisk.com

Investors:

Jacob Martin Wiborg Rode
+45 3075 5956
jrde@novonordisk.com

David Heiberg Landsted
+45 3077 6915
dhel@novonordisk.com

Sina Meyer
+45 3079 6656
azey@novonordisk.com

Frederik Taylor Pitter
+1 609 613 0568
fptr@novonordisk.com

Ida Schaap Melvold
+45 3077 5649
idmg@novonordisk.com

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