

press release

Phase 3 data for concizumab show 86% reduction in treated bleeds in haemophilia A or B with inhibitors

Data presented today show a reduction in treated spontaneous and traumatic bleeds and mean annualised bleeding rate (ABR) of 1.7 with concizumab¹

London, UK, 10 July 2022 – Novo Nordisk announced the phase 3 results of the explorer7 study, assessing the efficacy and safety of prophylactic treatment with concizumab in people living with haemophilia A or B with inhibitors.¹ The results from the primary analysis were presented today at the International Society of Thrombosis and Haemostasis Annual Congress (ISTH 2022) in London, UK.

Concizumab is an anti-tissue factor pathway inhibitor (TFPI) antibody in development for once-daily prophylactic treatment (regular treatment to prevent prolonged and spontaneous bleeding) by administration under-the-skin for all types of haemophilia. The use of concizumab is investigational and not approved by regulatory authorities.

The results showed an 86% reduction in treated spontaneous and traumatic bleeds when on concizumab prophylaxis, with an estimated mean ABR of 1.7 compared to 11.8 with no prophylaxis, fulfilling the primary objective.¹ The overall median ABR of concizumab was zero, compared to 9.8 for no prophylaxis. Twenty-one (63.6%) people on concizumab experienced no treated bleeds, compared to two (10.5%) on no prophylaxis.¹ The safety and tolerability profile of concizumab in this study was within the expected range, with no thromboembolic events reported after treatment restart.¹

“One of the most critical complications in the treatment of haemophilia is the development of inhibitors, as they render standard replacement therapy ineffective and severely limit treatment options for haemophilia B,” said explorer7 lead investigator Dr Victor Jiménez-Yuste, MD, Haematology Department, La Paz University Hospital, Madrid, Spain. “Based on the results of the explorer7 study, there is a potential for concizumab to become a new treatment option for people living with haemophilia A or B with inhibitors.”

“The treatment of haemophilia is complex and no one treatment fits all,” said Martin Lange, executive vice president and head of Development at Novo Nordisk. “Concizumab offers the potential for everyday protection for people living with haemophilia and provides an important potential addition to our haemophilia offering, especially in the haemophilia B with inhibitor population who currently have limited treatment options.”

Novo Nordisk expects to submit concizumab for regulatory approval for the prophylactic treatment of haemophilia A or B with inhibitors in the second half of 2022 in the US and Japan, and in 2023 in the EU and the UK.

About the explorer7 study

Explorer7 is part of the ongoing explorer clinical trial programme for concizumab, which aims to evaluate the efficacy and safety of concizumab for people living with haemophilia A or B with or without inhibitors. It is designed as a once-daily prophylactic treatment by administration under-the-skin with a ready-to-use, prefilled pen.^{2,3,4,5,6,7,8} In explorer7, 133 males (aged 12 years and over) were randomised 1:2 to either a no prophylaxis (arm one; ≥ 24 weeks) or concizumab prophylaxis (arm two; ≥ 32 weeks) or assigned to concizumab prophylaxis (arm three and four). The primary analysis compared number of treated spontaneous and traumatic bleeding episodes, measured as ABR, between arms one and two.¹

About haemophilia

Haemophilia is a rare disease that impairs the body's ability to make blood clots, a process needed to stop bleeding after a traumatic event.⁹ It is estimated to affect approximately 1,125,000 people worldwide.¹⁰ Haemophilia A and B are more common in males than in females, with ~ 88% of people diagnosed with haemophilia worldwide being male.^{9,11} Some people with haemophilia may also develop inhibitors, which are an immune system response to the clotting factors in replacement therapy that cause the treatment to stop working.⁹ Currently, it is estimated that 30% of people living with haemophilia A and 1-3% of people living with haemophilia B have inhibitors.¹²

About concizumab

Concizumab is an anti-TFPI monoclonal antibody, designed to block a protein in the body that stops blood from clotting, called TFPI. By blocking TFPI, concizumab encourages the production of a blood clotting protein called thrombin, which helps to clot the blood and prevent bleeding.^{13,14} The pivotal explorer7 (haemophilia A or B with inhibitors) and explorer8 (haemophilia A or B without inhibitors) studies are currently ongoing.⁸ The use of concizumab in people with haemophilia A or B with or without inhibitors is investigational and not yet approved by regulatory authorities. Concizumab is also being evaluated in children living with haemophilia A and B, with and without inhibitors, in the investigational explorer10 paediatric study expected to complete in 2026.¹⁵

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 diabetes and other serious chronic diseases such as obesity and rare blood and endocrine disorders. We do so by pioneering scientific breakthroughs, expanding access to our medicines, and working to prevent and ultimately cure disease. Novo Nordisk employs about 49,300 people in 80 countries and markets its products in around 170 countries. Novo Nordisk's B shares are listed on Nasdaq Copenhagen (Novo-B). Its ADRs are listed on the New York Stock Exchange (NVO). For more information, visit novonordisk.com, [Facebook](#), [Twitter](#), [LinkedIn](#) and [YouTube](#).

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