

## press release

### **Alhemo® recommended for European approval as first once-daily subcutaneous prophylactic treatment for people living with haemophilia A or B with inhibitors**

**Bagsværd, Denmark, 18 October 2024** – Novo Nordisk today announced that the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) has adopted a positive opinion, recommending approval of Alhemo® (concizumab) as the first once-daily subcutaneous prophylactic treatment for people aged 12 years or older living with haemophilia A or B with inhibitors.

Haemophilia is a rare bleeding disorder that impairs the body's ability to make blood clots, a process needed to stop bleeding. Haemophilia is often treated by replacing the missing clotting factor via intravenous infusions, also known as replacement therapy. However, sometimes the body can produce inhibitors as an immune response to the clotting factors in the therapy, which means replacement therapy does not work and limits treatment options overall. Currently, it is estimated that up to 30% of people living with severe haemophilia A develop inhibitors,<sup>1</sup> and 5-10% of those with severe haemophilia B develop inhibitors.<sup>2,3</sup>

Alhemo® is an anti-tissue factor pathway inhibitor (TFPI) monoclonal antibody that, if approved by the European Commission, will offer the first once-daily subcutaneous prophylactic treatment to people with haemophilia A or B with inhibitors.<sup>4</sup> Alhemo® is designed to block a protein called TFPI in the body that stops blood from clotting. By blocking TFPI, Alhemo® ensures the production of thrombin, which helps to clot the blood and prevent bleeding, even when the other clotting factors are missing or deficient. Importantly, this means Alhemo® allows blood clots to happen, even in the presence of inhibitors.<sup>5</sup>

"The positive opinion from the CHMP for Alhemo® is a major milestone for patients," said Stephanie Seremetis, chief medical officer for haemophilia at Novo Nordisk. "If approved, Alhemo® would be an important addition to our growing haemophilia portfolio as it offers the potential of everyday prophylaxis to prevent bleeds for people living with haemophilia who have developed the complication of inhibitors. It could alleviate the physical, emotional, and overall treatment burden for people living with haemophilia, as it is delivered in a pre-filled, multi-use, portable pen that can be stored at room temperature for up to four weeks. This could enable patients to have greater confidence in the pursuit of daily activities, which is especially

important for people living with haemophilia B with inhibitors, who currently have very limited treatment options.”

The CHMP positive opinion is based on data from the phase 3 explorer7 study, which was designed to evaluate the efficacy and safety profile of Alhemo® for people living with haemophilia A or B with inhibitors.<sup>6</sup> If approved, Alhemo® will be provided in a portable, pre-mixed and prefilled pen, enabling quick and easy subcutaneous administration with the aim of easing the treatment burden of regular intravenous infusions.<sup>7</sup> Novo Nordisk expects a final approval by the European Commission within approximately two months.

### **About Alhemo® (concizumab)**

Alhemo® (concizumab) is an anti-tissue factor pathway inhibitor (TFPI) monoclonal antibody designed to block a protein in the body that stops blood from clotting. By blocking TFPI, Alhemo® ensures the production of thrombin, which helps to clot the blood and prevent bleeding.<sup>5</sup> Alhemo® is currently approved in Australia<sup>8</sup> and Switzerland<sup>9</sup> for the treatment of adolescents and adults (12 years or older) with haemophilia A or B with inhibitors. In Japan,<sup>10</sup> Alhemo® is currently approved for the treatment of adolescents and adults (12 years or older) with haemophilia A or B with and without inhibitors and is indicated for routine prophylaxis to prevent or reduce the frequency of bleeding episodes.

### **About the explorer7 study**

In explorer7, 133 males (aged 12 years or older) were randomised 1:2 to either no prophylaxis (arm one; ≥24 weeks) or concizumab prophylaxis (arm two; ≥32 weeks) or assigned to concizumab prophylaxis (arm three and four).<sup>11</sup> The primary analysis compared the number of treated spontaneous and traumatic bleeding episodes, measured as annualised bleeding rate (ABR), between arms one and two. 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.<sup>11</sup> The overall median ABR of concizumab was zero, compared to 9.8 for no prophylaxis. The safety and tolerability profile of concizumab in this study was within the expected range.<sup>11</sup>

### **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.<sup>12</sup> Due to the nature of haemophilia being an x-linked recessive disorder, it often presents differently in males compared to females, with roughly 88% of people diagnosed with haemophilia worldwide being male.<sup>13,14</sup> 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), and haemophilia B is caused by a missing or defective clotting Factor IX (FIX).

## 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 69,000 people in 80 countries and markets its products in around 170 countries. For more information, visit [novonordisk.com](https://www.novonordisk.com), [Facebook](#), [Instagram](#), [X](#), [LinkedIn](#) and [YouTube](#).

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