press release

Novo Nordisk to present new data from sickle cell disease and haemophilia trials at the 66th American Society of Hematology (ASH) annual meeting

- HIBISCUS phase 2 data evaluating the safety profile and efficacy of etavopivat in adult and adolescent patients with sickle cell disease
- FRONTIER4 interim phase 3 results evaluating safety profile and efficacy of Mim8 prophylaxis administered once every two weeks for patients with haemophilia A with or without inhibitors
- Explorer7 phase 3 study results evaluating the safety profile and efficacy of concizumab in patients with haemophilia A or B with inhibitors, with or without target joints at baseline

Bagsværd, Denmark, 3 December 2024 – Novo Nordisk today announced the presentation of 13 abstracts, three of which will be presented in oral sessions, at the upcoming 66th Annual Meeting and Exposition of the American Society of Hematology (ASH), which will take place from 7 to 10 December 2024 in San Diego, California.

In an oral presentation on 7 December, 52-week results from the phase 2 part of the ongoing phase 2/3 HIBISCUS trial of etavopivat will be presented. These results have determined the dose for the phase 3 part of the trial, as well as examined the safety profile and efficacy of etavopivat, including the incidence of vaso-occlusive crises (severe pain caused when blood vessels are blocked and deprive tissues of oxygen[1]) in patients with sickle cell disease. The results from HIBISCUS have been chosen to be highlighted in the ASH Annual Meeting Press Program session 'Reading Up on the Classics: Treating Not-So-Benign Hematology Conditions' on 7 December at 08.30 PST.

From Novo Nordisk's haemophilia portfolio, there are two oral presentations of note: first on the efficacy and safety results of an interim analysis from FRONTIER4, a phase 3 open-label, multi-centre extension study of Mim8 in people living with haemophilia A with and without inhibitors (HA/HAwI). Additionally, an oral presentation of data from the phase 3 explorer7 study assessing the efficacy of concizumab in people with haemophilia A or B with inhibitors (HAwI/HBwI), with or without recurring bleeds into the same joint (or target joints) at baseline.

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“There is a significant unmet need for novel treatment options that have the potential to transform care for people with rare blood disorders globally," said Martin Holst Lange, executive vice president and head of Development at Novo Nordisk. “I am particularly excited that we at the ASH congress will present new data from our pipeline in sickle cell disease, a first for Novo Nordisk. Sickle cell disease affects approximately eight million people worldwide with big consequences for the individual patient, yet there are few treatment options available. Our sickle cell research builds on our legacy in haemophilia, where we continue to advance research to address the unmet needs of patients.”

Summary of presentations
Accepted data at the 66th ASH annual meeting include the following poster and oral presentations. Accepted abstracts include preliminary data that may be subject to change in final manuscripts, which will be published in the journal *Blood* following the congress. Dates and times of the presentations can be found on the ASH website.

Sickle cell disease

- Etavopivat reduces incidence of vaso-occlusive crises in patients with sickle cell disease: HIBISCUS trial phase 2 results through 52 weeks (179-O)
- Etavopivat increases arterial haemoglobin-oxygen saturation during moderate and severe hypoxia: a mechanistic phase 1 trial in healthy volunteers (2461-P)
- Large scale analysis of the real-world association between fetal hemoglobin and vaso-occlusive crises in sickle cell disease (1124-P)
- Characterizing People with sickle cell disease who share attitudes regarding clinical trial participation: findings from the global LISTEN survey (1124-P)
- How people with sickle cell disease rate motivators is associated with the likelihood of wanting to participate in a clinical trial: findings from the global LISTEN survey (1135-P)
- Motivators and barriers for people with sickle cell disease participating in clinical trials: United States findings from the LISTEN survey (1135-P)
- Noninvasive, accessible smartphone app for at-home hemoglobin monitoring in sickle cell disease (2248-P)

Haemophilia

- Safety and efficacy of Mim8 prophylaxis administered once every two weeks for patients with hemophilia A with or without inhibitors: interim analysis of the FRONTIER4 open-label extension study (718-O)
- Mim8 prophylaxis beyond bleeding: investigating multifaceted, patient-reported outcomes for hemophilia A in the FRONTIER2 study (1212-P)
- Annualized bleeding rates in patients with hemophilia A or B and inhibitors with and without target joints at baseline: results from the concizumab phase 3 explorer7 study (715-O)
- Burden of treatment on people with hemophilia: global real-world data (5077-P)

- Physical and psychological burden on people with hemophilia: global real-world data (2318-P)
- Unmet needs of patients with hemophilia A/B with or without inhibitors: real-world end-of-study results from the explorer6 non-interventional study (2585-P)

About sickle cell disease
Sickle cell disease is a debilitating, life-threatening group of rare, inherited red blood cell disorders caused by a mutation in the haemoglobin gene within red blood cells[2,3]. This mutation causes red blood cells to become stiff and half-moon or 'sickle' shaped[2]. Sickle cells are less effective at carrying oxygen, do not last as long as healthy cells and risk getting stuck in blood vessels, leading to blockages known as vaso-occlusion[1,4–7]. Sickle cell disease is characterised by acute and chronic pain, anaemia and fatigue alongside vaso-occlusive crises (VOCs), which can require hospitalisation and can lead to complications, including organ damage[8]. Globally, almost 8 million people are living with sickle cell disease[9].

About etavopivat
Etavopivat is an investigational, oral, small-molecule activator of erythrocyte pyruvate kinase (PKR) activator in development for the treatment of sickle cell disease and other haemoglobinopathies[10]. Etavopivat-mediated activation of PKR lowers levels of 2,3-diphosphoglycerate (2,3-DPG) and raises adenosine triphosphate (ATP) levels, which has the potential to improve oxygen bind to haemoglobin (i.e. increase oxygen affinity), reduce haemolysis and decrease VOCs by improving red blood cell health[10].

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[11]. 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[12,13]. 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 Mim8
Mim8 is an investigational Factor VIIIa (FVIIIa) mimetic bispecific antibody designed with the potential to deliver sustained haemostasis for once-monthly, once every two weeks or once-weekly 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 anywhere in the world.

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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, called TFPI. By blocking TFPI, Alhemo® encourages the production of thrombin, which helps to clot the blood and prevent bleeding[14]. Alhemo® is currently approved in Australia[15] and Switzerland[16] for the treatment of adolescents and adults (12 years or older) with haemophilia A or B with inhibitors; in Japan, Alhemo® is currently approved for the treatment of adolescents and adults (12 years or older) with haemophilia A or B with and without inhibitors[17]. In all approved countries, it is indicated for routine prophylaxis to prevent or reduce the frequency of bleeding episodes. Alhemo® received a positive opinion from the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) on 17 October 2024[18].

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 72,000 people in 80 countries and markets its products in around 170 countries. For more information, visit novonordisk.com, Facebook, Instagram, X, LinkedIn and YouTube.

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