

Rare disease

CMD24
CAPITAL MARKETS DAY

7 MARCH



Ludovic Helfgott
EVP Rare disease



Martin Holst Lange
EVP Development

VICTOR DA SILVIA MELCUNAS
Victor lives with severe haemophilia A
Brazil

Forward-looking statements

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Unless required by law, Novo Nordisk has no duty and undertakes no obligation to update or revise any forward-looking statement after the distribution of the Annual Report 2023, whether as a result of new information, future events, or otherwise.

Important drug information

Victoza® and Ozempic® are approved for the management of type 2 diabetes only
Saxenda® and Wegovy® are approved for the treatment of obesity only

Strategic aspirations 2025



Purpose and sustainability (ESG)

- Progress towards zero environmental impact
- Being respected for adding value to society
- Being recognised as a sustainable employer



Innovation and therapeutic focus

- Further raise the innovation-bar for diabetes treatment
- Develop a leading portfolio of superior treatment solutions for obesity
- **Strengthen and progress the Rare disease pipeline**
- Establish presence in Cardiovascular & emerging therapy areas



Commercial execution

- Strengthen Diabetes leadership - aim at global value market share of more than 1/3
- More than 25 billion DKK in Obesity sales by 2025
- **Secure a sustained growth outlook for Rare disease**



Financials

- Deliver solid sales and operating profit growth
- Drive operational efficiencies across the value chain to enable investments in future growth assets
- Deliver free cash flow to enable attractive capital allocation to shareholders

RareD constitutes an attractive opportunity for Novo Nordisk

Addressing the unmet needs

Patient burdens¹

- Reduced life-expectancy
- Severe co-morbidities and impaired quality of life
- Long diagnostic lead-times
- Broken continuum of care and strong inequalities

A longstanding legacy

Since 1970s in growth disorders

norditropin[®]
somatotropin (rDNA origin) injection

Since 1980s in haemophilia

NovoSeven[®]
Recombinant Factor VIIa

refixia[®]
nonacog beta pegol

esperoct[®]
turoctocog alfa pegol

The Rare disease opportunity for Novo Nordisk

A strategic portfolio play in specialty care



Few patients, high unmet need



Specialised healthcare base



Specialised scientific and commercial teams

A platform to spearhead new trends

Integrated therapeutic solutions
adding diagnostics, digital, data, device and drug (5D)

Innovative access pathways

New operating models




An integrated unit

From research to commercial, RareD is operating as an **integrated unit** within Novo Nordisk, with dedicated resources, to provide agility and flexibility

¹Editorial, The Lancet Diabetes & Endocrinology. 2019; 7(2)75
Note: RareD is Novo Nordisk's rare disease unit

Executing on new strategy since 2019 with near-term focus on next generation launches

The Rare disease strategy

 <p>Serve more</p>	<p>Build on expertise to expand selectively:</p> <ul style="list-style-type: none"> • From haemophilia to rare blood disorders • From growth disorders to rare endocrine disorders
 <p>Find more</p>	<p>Accelerate the pipeline with internal and external innovation building on all platforms available within Novo Nordisk</p>
 <p>Grow more</p>	<p>Further increase commercial competitiveness and launch speed across geographies</p>

Focus on succeeding with launches from the core

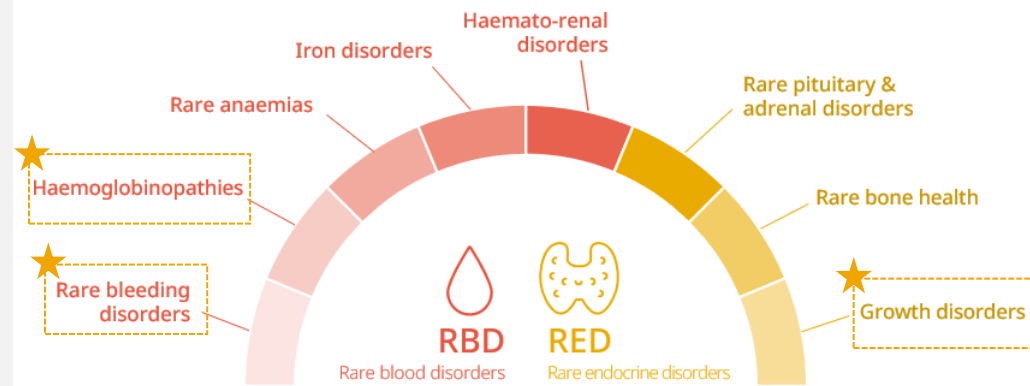


PPx: Prophylaxis; RBD: Rare blood disorders; RED: Rare endocrine disorders; sPPH: Severe postpartum haemorrhage
 Note: Alhemo® is the brand name for concizumab

RareD has carefully selected focus areas within rare blood and rare endocrine disorders

The RareD strategy

Strategic focus areas



Out of the 350 million+ rare disease patients globally¹, RareD focuses on a total addressable pool of 20 million (6% of total) today

Focus on three rare disease areas



Rare bleeding disorders (eg Haemophilia, GT)

- Persisting unmet needs despite multiple therapeutic options
- Novo Nordisk is present across all haemophilia segments and other bleeding disorders
- **Global market size:** 13 bUSD (Haemophilia, 2023)



Haemoglobinopathies (eg Sickle cell disease, Thalassemia)

- Reduced life expectancy, severe morbidity & organ damage
- Future growth driven by innovation and geographic expansions
- **Global market size:** ~2 bUSD (2023)



Growth disorders

- Persisting unmet needs in growth hormone diseases
- High complementarity with rare bone and adrenal disorders
- **Global market size:** 2.2 bUSD (2022, before LAGH products)

¹Findacure, rare diseases (<https://www.findacure.org.uk/rare-diseases/>)
 GT: Glanzmann's thrombasthenia; LAGH: Long-acting growth hormone
 Sources: Evalute Ltd., Long-Term Outlook (2022-2028) Report (Accessed Feb 2024)

Focus to strengthen the rare disease portfolio with internal and external innovation

Strengthen and progress pipeline

Our key focus areas

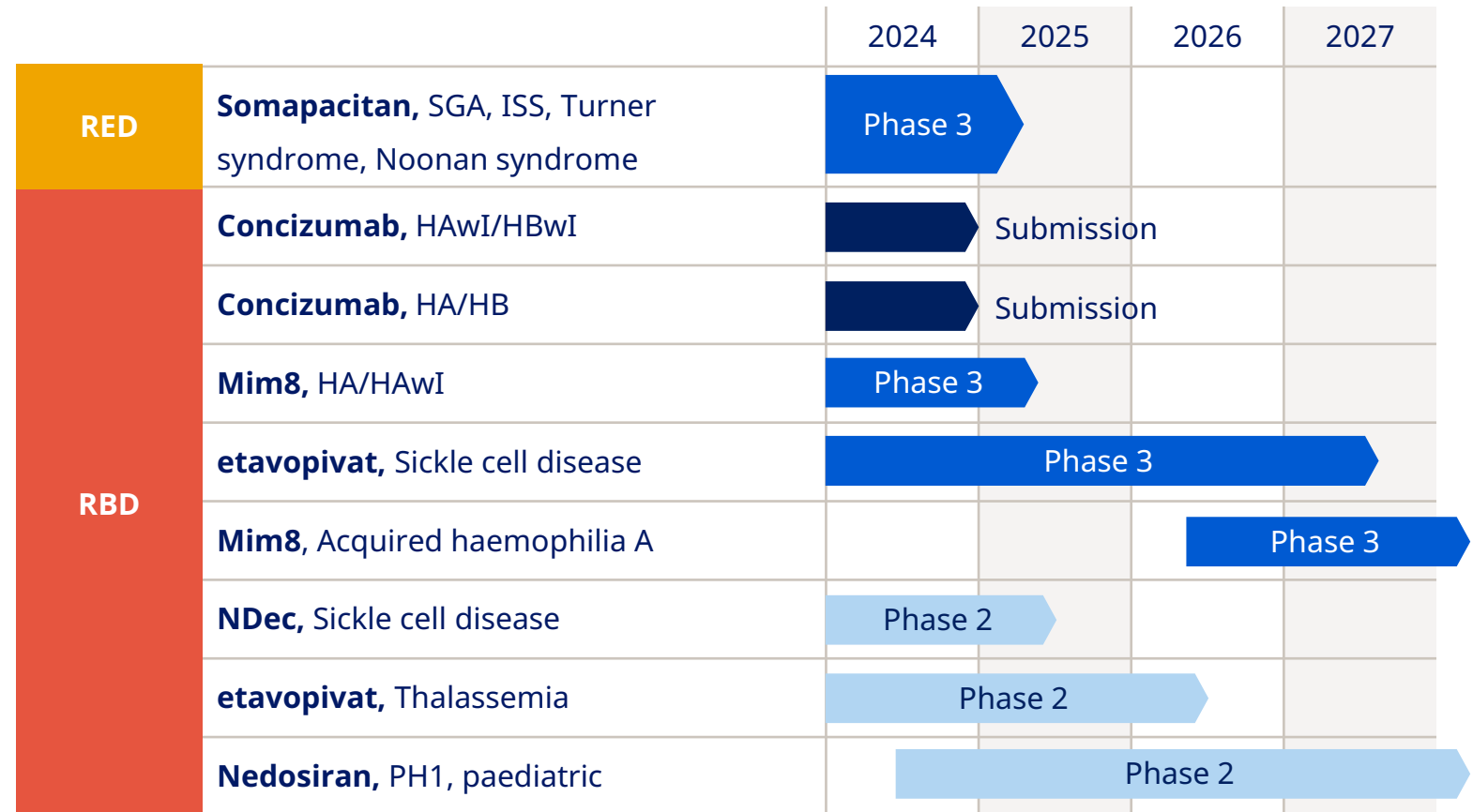
In Research and early development

- All Novo Nordisk technology platforms explored such as monoclonal antibodies, peptides, siRNA, small molecules and gene editing
- External collaborations intensified eg with 2seventy bio™ and Forma Therapeutics

In Development

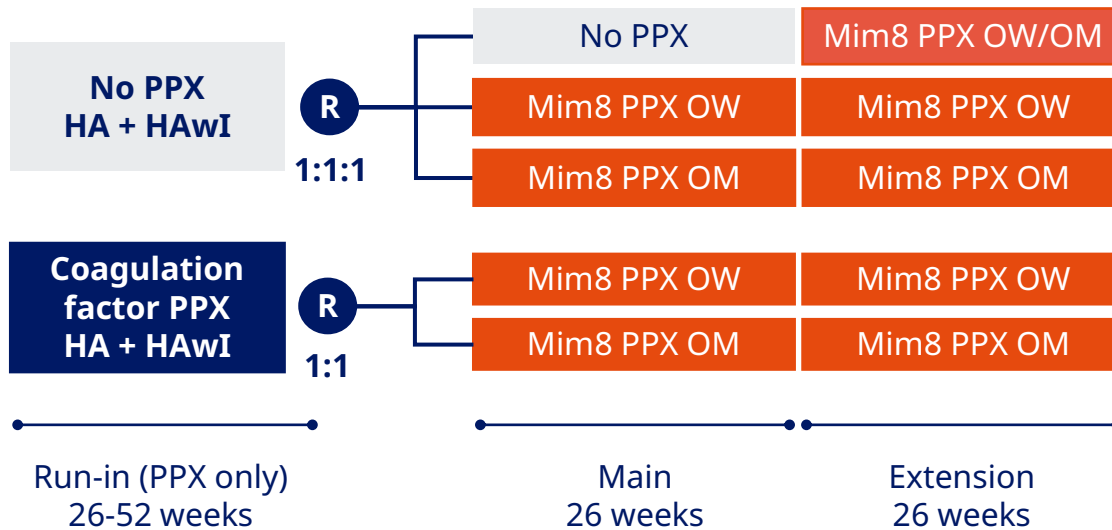
- Faster global patient recruitment with improved collaboration with local sites
- Life cycle management from the start
- An integrated “beyond the drug” approach utilising eg AI algorithms

Development pipeline



Phase 3 trial FRONTIER 2 with Mim8 in haemophilia A is expected to read out during the first half of 2024

FRONTIER 2 trial in >250 adults & adolescents with haemophilia A



About Mim8 and the phase 3 trial programme

Potential differentiators for Mim8



Phase 2: Median ABR of 0, exploratory analysis implied 70% had no bleeds at 12 weeks



Low injection site reaction (high potency allows low volume)



Monthly dosing frequency

FRONTIER phase 3 trial programme

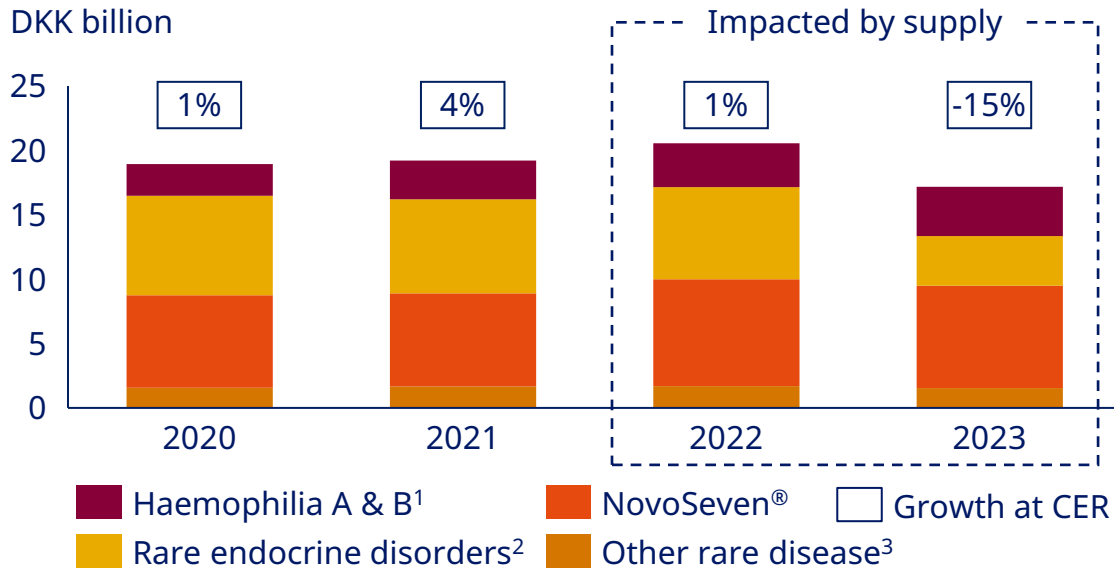
- FRONTIER 3: Paediatric trial
- FRONTIER 4: Long-term safety (open label extension)
- FRONTIER 5: Switch study (from emicizumab)

Key trial endpoints

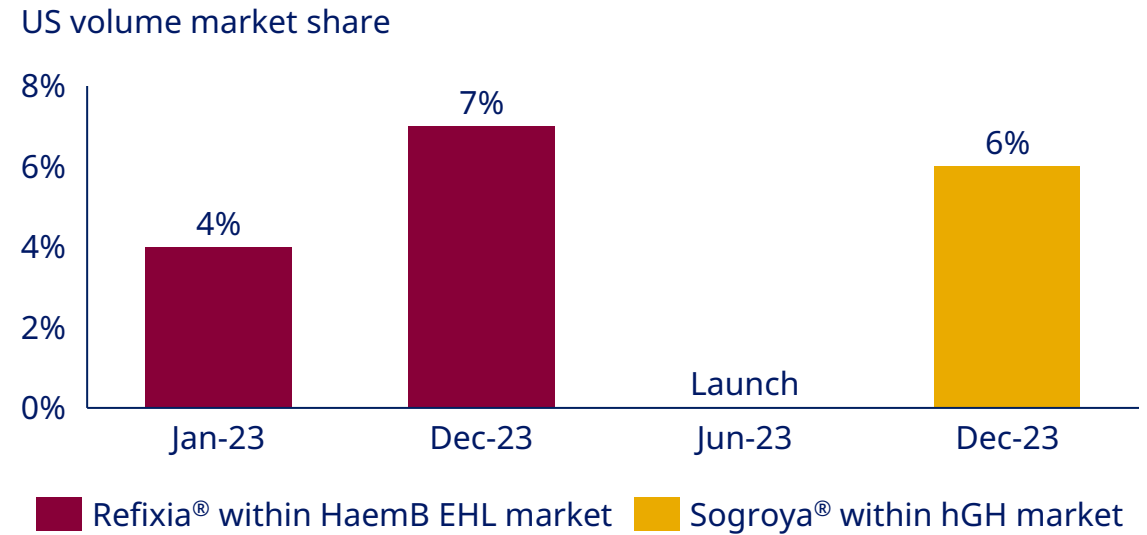
- Annual bleeding rate for treated bleeds over 26 weeks of treatment
- Overall safety of Mim8 prophylaxis including occurrence of anti-Mim8 antibodies and injection site reactions

Focus to move beyond recent supply constraints to leverage launch capabilities

Rare disease performance was impacted by supply constraints



Market share increases with Refixia® and Sogroya® in the US



- Growth impacted by a reduction in manufacturing output starting in H2 2022 due to a planned facility upgrade
- The supply situation is expected to gradually stabilise during 2024

- RBD: Growth accelerates, and market share increases with the latest launch products supported by commercial execution and launch capabilities
- RED: Sogroya® is the 3rd entrant and already #1 in the US within the long-acting growth hormone segment

¹Also comprises NovoThirteen®; ²Primarily Norditropin®; ³Other Rare disease primarily consists of Vagifem® and Actively®
 CER: Constant exchange rates; EHL: Extended half-life; HaemB: Haemophilia B; hGH: Human growth hormone; RBD: Rare bleeding disorders; RED: Rare endocrine disorders
 Note: Company reported sales; ADIVO data, Dec 2023

Closing remarks

Strategy unchanged with focus areas within rare blood and rare endocrine disorders

A dedicated Rare disease unit with an early and late-stage pipeline and launch capabilities to support growth

Mim8 phase 3 results expected in the first half 2024