Introduction to Sobi and our current research in Cold Agglutinin Disease (CAD)

rare **strength**

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Sobi at a glance

Biopharmaceutical company with a heritage in rare diseases since the 1930s

Therapeutic areas:

- Hematology
- Immunology
- Specialty Care

Present in more than 30 countries, delivering treatments to patients in over 70 countries around the world

Head office in Stockholm, Sweden
US office in Waltham, MA

Appr. 1,500 employees

We are dedicated to providing **access** to innovative **treatments** that **transform life** for **people with rare diseases**.

Dedicated to **rare diseases**

We specialize in rare diseases because it is where we can make the greatest difference to people's lives.

There are an estimated 6,000 distinct rare diseases, most untreated today.

It is a hugely under-served area with great unmet medical needs unlike any other in medicine.

More than
300 million
People around the world are affected by rare diseases.

95%
of rare diseases currently have no approved treatment.

Therapeutic areas & late-stage pipeline

Sobi therapeutic areas

Hematology

- Hemophilia
- Thrombocytopenia
- Paroxysmal nocturnal hemoglobinuria

Immunology

- Interleukin-1 and autoinflammatory diseases
- Interferon gamma and HLH
- Respiratory syncytial virus (RSV)

Specialty Care portfolio

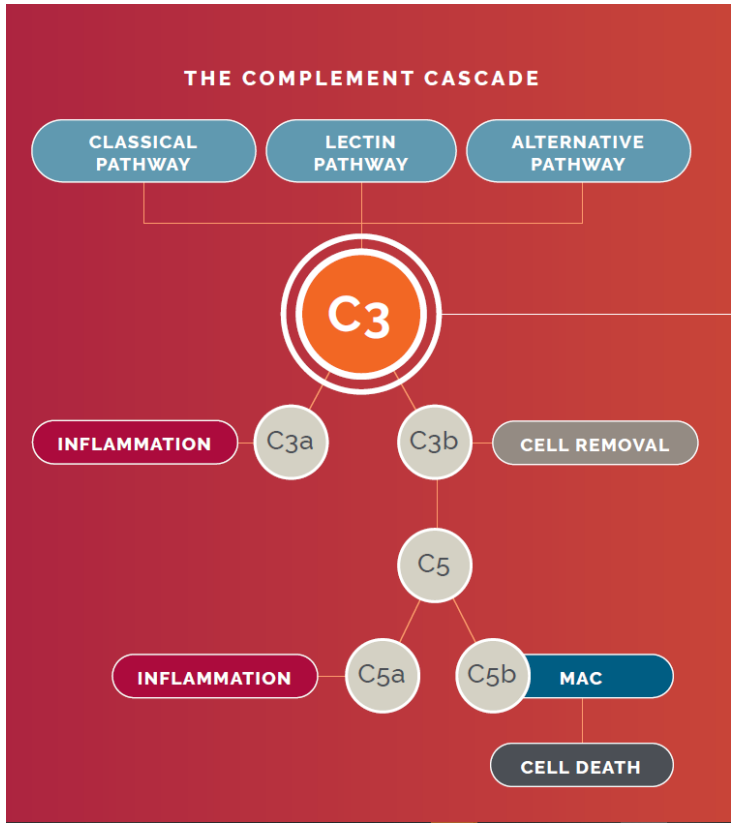
- Genetic and metabolic diseases (such as hereditary tyrosinemia type 1, HT-1), as well as a number of specialist indications

Sobi late-stage product pipeline

Phase 2	Phase 3
Secondary HLH adults	
Secondary HLH/MAS children	Chronic refractory gout
Acute graft failure (aGF)	RSV prevention
	Hemophilia A
HSCT-TMA	**CAD**
ALS	IC-MPGN and C3G

*Research targeting the **complement cascade**, a part of the immune system that eliminates damaged or diseased cells in the body as one of its functions*

Investigating the role of C3



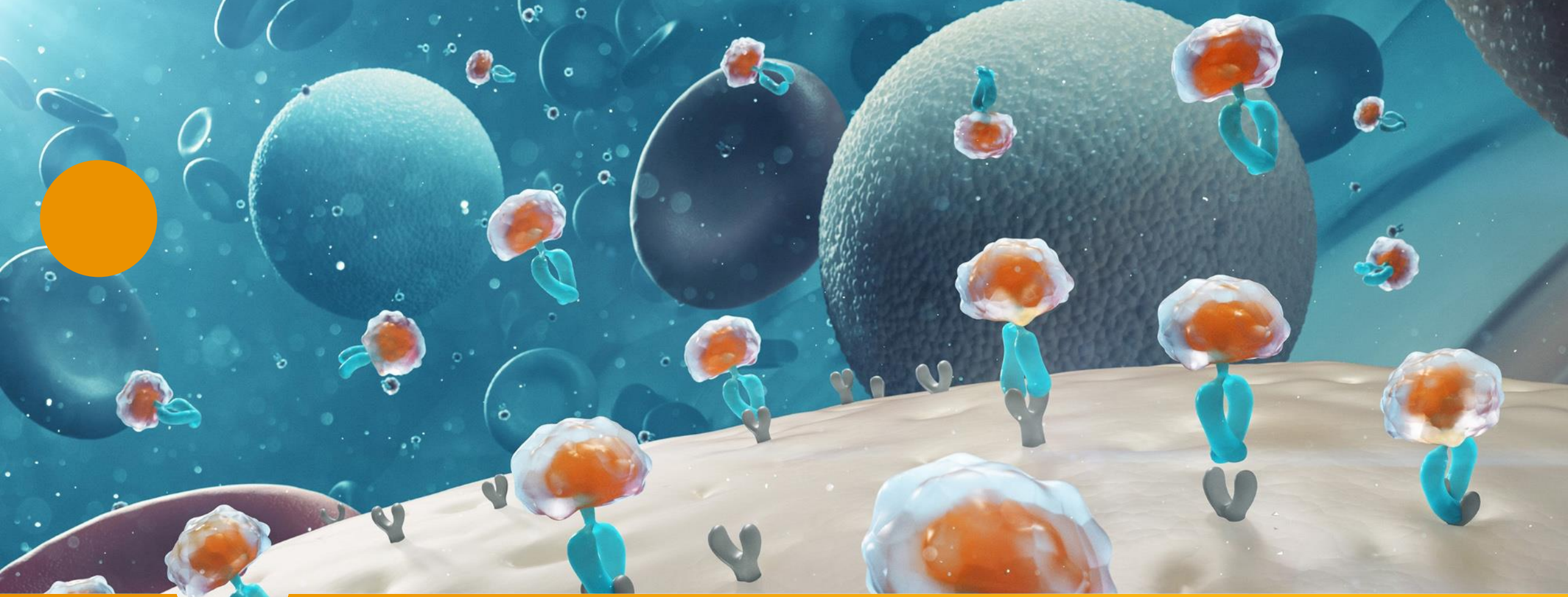
- Sobi, in collaboration with Apellis, is conducting **clinical trials** with an investigational drug, pegcetacoplan,* to evaluate its potential to treat a range of **complement-driven diseases**

- This **investigational therapy binds to C3**, a protein that plays a very important role in the early stage of the complement cascade. Uncontrolled complement activation is thought to drive the onset of many diseases.

- Pegcetacoplan has been **approved by the FDA for the treatment of adult patients with paroxysmal nocturnal hemoglobinuria (PNH),** a rare blood disorder.

- Pegcetacoplan* is under investigation as a potential treatment for CAD

**Pegcetacoplan is an investigational compound that has not been approved by any regulatory authority for CAD*



For further trial information please visit:

A Phase 3, Randomized, Double-blind, Placebo-controlled Multicenter Study to Evaluate the Efficacy and Safety of Pegcetacoplan in Patients With Cold Agglutinin Disease (CAD)

https://www.clinicaltrials.gov/ct2/show/NCT05096403

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