

partnering with us

We have a strong track record of leadership in navigating a complex rare disease market to develop and provide access to critical treatments, and we are actively seeking new products for our specialty care portfolio.

We are
committed to
providing a diverse
and inclusive workplace
for our
1,300+
people around
the world.



As we advance our R&D pipeline and scale up operations in North America and globally, we look forward to continued growth and partnership opportunities that help us strengthen our impact for the rare disease community.



our team

Our highly experienced, passionate team brings a cumulative 180+ years of pharmaceutical industry experience, which spans all key development and commercialization functions.

contact us

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we care about rare disease patients





driven to **serve** **unmet needs** for patients

In the U.S., orphan diseases are defined as those that affect fewer than 200,000 people. There are approximately 7,000 known rare diseases, yet only about 700 rare disease treatments have been approved since 1983.

At Sobi, we focus on ultra-rare diseases, which can affect just hundreds or even dozens of patients. These patients have many critical unmet needs yet are frequently overlooked by the health care industry.

Sobi is dedicated to providing access to innovative treatments that make a significant difference in the lives of individuals with rare and ultra-rare diseases. We work closely with patients, caregivers and advocacy organizations to understand and address the challenges they face throughout their journey, from infancy to adulthood. And we keep innovating to meet their evolving needs, serving as a trusted partner for life.

Nearly **50%** of rare diseases **begin in childhood**; and it's a difficult journey no one should have to make alone.

our **focus**

Sobi markets more than **35 products** across over **70 countries** and has a **robust pipeline** of investigational therapies based on expertise in genetics/metabolism, inflammation and recombinant protein engineering.

We have built a 30+ year legacy in rare diseases. We were one of the original pioneers in this space, and our vision is to become a global leader. As we move towards that vision, we are growing our strong presence in North America.

Our currently **approved indications** in North America include:

- **Primary Haemophagocytic Lymphohistiocytosis (HLH)**, a rare, life-threatening genetic condition that most commonly impacts infants and young children. Primary HLH is a syndrome of extreme immune activation that is characterized by fevers and the abnormal enlargement of organs, among other symptoms, which is often fatal without treatment.
- **Hereditary tyrosinemia type 1 (HT-1)**, a rare genetic disorder that may cause liver, kidney and neurological issues as well as shorter life-expectancy if not treated.
- **Moderate to severe active Rheumatoid Arthritis (RA)** in adults, a chronic inflammatory disorder affecting the lining of the joints.
- **Neonatal-Onset Multisystem Inflammatory Disease (NOMID)**, a rare genetic disorder that may cause rashes, fevers and other symptoms, over time resulting in chronic inflammation that can lead to developmental delay, physical disability and intellectual disability.
- **Oral Mucositis in Hematologic Malignancies**, a painful inflammation commonly associated with cancer chemotherapy treatment given with stem cell transplant.
- **Respiratory Syncytial Virus (RSV)**, an easily spread virus that can cause severe lung disease in babies who are born either prematurely or during RSV season. Almost all children catch it at least once by the time they turn two.
- **Chronic immune thrombocytopenia (ITP)**, a rare autoimmune bleeding disorder characterized by low number of platelets.
- **Chronic liver disease (CLD)**, a chronic disorder marked by the gradual destruction of liver tissue over time. One consequence is low platelet counts.

Sobi also has a robust pipeline of investigational programs targeting multiple rare diseases in various stages of research and clinical development.

Sobi in **north america**

Sobi's North American business is headquartered in the greater Boston area, with key additional locations in the Toronto, Ontario and Durham, North Carolina areas. Our team in North America is growing as we work to bring patients and caregivers not only important medicines, but also patient support programs including services such as nurse support, help navigating reimbursement, refill reminders, door-to-door medicine delivery, educational disease information, peer to peer mentoring, and more.

