Sobi
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Leading the way in rare diseases

Sobi is an integrated biopharmaceutical company with capabilities throughout the value chain and a strong track record of commercializing medications in complex markets. While Sobi continues to develop a sustainable research and development base, its proven value-driven platform for launching products for rare diseases offers potential partners strengths in the field. Sobi is looking for opportunities to inlicense late-stage biologics.

Biotechnology companies face distinct challenges when commercializing products for rare diseases. The usual strategies do not apply when working with small populations of patients, and thus a thorough understanding of local regulatory environments, as well as the drivers among payers and health-care providers, is crucial.

Swedish Orphan Biovitrum AB (publ) (Sobi), an international specialty health-care company, aims to be recognized as a global leader in providing access to innovative treatments that transform the lives of people with rare diseases. With a product portfolio including treatments for hemophilia, autoimmune, autoinflammatory and genetic metabolic diseases, Sobi has expertise in developing and commercializing rare disease treatments across Europe, the Middle East, North Africa and North America.

"At Sobi we have played a leading role in rare diseases for many years," said Daniel Rankin, head of business development. "We have a deep understanding of how to bring a rare disease medicine to market for patients. Through our integrated patient access approach, we work with multiple stakeholders to cocreate a sustainable pathway for patients to access our groundbreaking treatments. We deliver realistic pricing that meets the affordability needs of payers to deliver long-term value. For us, collaborations with all stakeholders are essential for engaging with rare-disease patient populations and for market success. We strive to maintain a critical, class-leading relationship with the patient organizations which have a pivotal and powerful role."

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Formed in 2010 by the merger of Swedish Orphan and Biovitrum, and with more than 30 years of experience in hemophilia, Sobi has capabilities across the value chain, from research and development (R&D) and manufacturing to patient access, and an expanding global commercial platform. Sobi's partnering model of collaborating from the development stage through to commercialization has powered its successful hemophilia franchise, as well as numerous other launches over the years.

Research and development activities at the Sobi laboratories.

Efficient path to market

Sobi's integrated, value-driven platform leads the way in making medications available to patients in complex European markets. For example, through a partnership with Bioverativ (a Sanofi company) and its predecessor that began in 2006, Sobi has successfully commercialized a generation of extended-half-life products for hemophilia in Europe, most of the Middle East and in North Africa.

Key to this success was Sobi's work to understand the needs of regulators and payers, creating powerful value propositions and product validations. Sobi was able to achieve reimbursement across its European markets by an average of 4.9 months faster than is standard.

With a similar understanding of the market in the US, Sobi is now expanding in the region and is strengthening its immunology franchise. In 2018, Sobi acquired the global rights to emapalumab, the first and only treatment for primary hemophagocytic lymphohistiocytosis (HLH), recently approved for the treatment of primary HLH in the US. Sobi also announced agreements to acquire the rights to palivizumab in the US from AstraZeneca, along with participation in the US financial rights to follow-on product MEDI8897. Palivizumab is the only approved prophylactic treatment for respiratory syncytial virus infection in high-risk infants. The acquisitions are expected to accelerate the buildup of Sobi's US commercialization platform and enhance Sobi's financial capacity to invest in future products.

Rare diseases approach

Medical teams play a central role in Sobi's approach to commercialization by working with rare disease communities. "This goes hand in hand with working in rare diseases—it is a key to enabling Sobi to bring products to patients so effectively," said Rankin.

The company also applies a complete life cycle management approach using post-launch R&D to identify opportunities for indication expansion and new formulations and to optimize real-world data. For example, since Sobi acquired the interleukin-1 (IL-1) receptor antagonist anakinra in 2009, the product has achieved double-digit growth, has been successfully registered for the treatment of the autoinflammatory disease cryopyrin-associated periodic syndrome (CAPS) and has shown potential for indication expansion to other autoinflammatory diseases involving IL-1.

In 2018, the European Medicines Agency approved an extension to the existing indications of rheumatoid arthritis and CAPS to include Still's disease. In the US, anakinra is approved for the treatment of neonatal-onset multisystem inflammatory disease (NOMID), and a study for its use in Still's disease is ongoing. A clinical trial program is also underway to evaluate the use of anakinra in patients with acute gout.

"We have grown anakinra in current indications by improving our understanding of patient and market needs, and using that insight to approach the medical profession in a different way," said Rankin.

Partnering opportunities

Sobi is seeking opportunities to acquire or inlicense late-stage products for rare diseases in Europe, the Middle East, North Africa and North America. The company is interested in products that complement its targeted treatments for rare diseases.

Potential partners may have a product entering or completing phase 3 trials, or a drug that is already commercially available. Sobi is also open to partners that need help taking a product from phase 2 to phase 3 trials.

"Through early involvement and engagement with a product, we help partners navigate an efficient path to market," said Rankin. "We understand clinical development, regulatory and market dynamics, so we can ensure clinical trials are crafted to answer the questions that payers will be asking, which helps enable patient access once products get regulatory approval."

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