

ABOUT ALEXION



COMPANY OVERVIEW

Alexion Pharmaceuticals, Inc. is a global biopharmaceutical company focused on developing and delivering life-transforming therapies for patients with devastating and rare diseases that have no effective treatment options.

Alexion's diversified business includes three products for four devastating disorders. We are the global leader in complement inhibition and developed and commercialize the first and only approved complement inhibitor to treat two severe and ultra-rare complement-mediated disorders. Our metabolic franchise includes two highly innovative enzyme replacement therapies for patients with life-threatening and ultra-rare disorders metabolic disorders.

Alexion is advancing the most robust rare disease pipeline in the biotech industry, which includes novel product candidates across multiple therapeutic areas, including severe and life-threatening metabolic and complement-mediated disorders. As we progress our complement and metabolic programs, we continue to evaluate additional development programs not based on disease area or modality, but instead on the devastating and rare nature of a disease and the life-transforming benefit a product candidate can potentially provide.

DRUG DEVELOPMENT

Alexion has significant experience in ultra-orphan drug discovery, regulatory affairs, and clinical trial design and recruitment. The company has a strong preclinical development infrastructure, including a proprietary antibody discovery platform, a robust

COMPANY INFORMATION

- Founded: 1992
- NASDAQ: ALXN
- Website: alexion.com

GLOBAL PRESENCE

More than 3,000 dedicated employees worldwide:

- Global headquarters in New Haven, CT, U.S.
- Manufacturing operations in Smithfield, RI, U.S.
- Translational medicine group in Lexington, MA, U.S.
- EMEA headquarters in Zürich, Switzerland
- Global supply chain, distribution and manufacturing operations in Dublin, Ireland
- Japan headquarters in Tokyo, Japan
- Asia-Pacific regional headquarters in Sydney, Australia
- Latin America regional headquarters in Miami, FL, U.S.
- Middle East operations center in Dubai, United Arab Emirates
- Local operations throughout Europe, Asia and the Americas

AWARDS AND RECOGNITION



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translational medicine group and global experience in regulatory complexities involving therapies for pediatric and adult patients.

MANUFACTURING

Alexion recognizes the vital importance of ensuring continuity of treatment for patients with severe and rare diseases. Alexion's manufacturing facilities produce commercial and clinical quantities of Alexion therapies to ensure uninterrupted worldwide supply, and also to support clinical development programs. In line with sound supply chain practices, Alexion also works with third-party providers to manufacture additional Alexion therapies, and with other third-party providers for services such as product filling, packaging and labeling.

Alexion's global supply chain and operations headquarters are located in Ireland, where the Company also has an aseptic vial fill-finish facility and is building a state-of-the-art bulk biologics manufacturing facility. Alexion also has manufacturing sites in the United States.

COMMERCIALIZATION

Alexion's approach to serving patients is driven by education and a passion for understanding and meeting the unique needs of patients and families suffering with rare diseases. These include disease education programs to raise awareness among physicians, and diagnostic initiatives to reduce the multi-year delays that patients with rare diseases often face, even when a safe and effective therapy is available. By expanding the knowledge and awareness of rare diseases globally, our commercial teams help the medical community improve diagnosis and treatment of severe, life-threatening diseases, with the goal of improving patient outcomes.

To ensure the highest level of excellence in these initiatives, Alexion has built its own multinational infrastructure from the ground up. Today, we have operations in place to serve patients in 50 countries.

OUR COMMITMENT TO PATIENTS

Alexion is committed to patients having access to our medicines. We work with private healthcare organizations, policymakers and governments around the world so that patients with rare diseases have access to the therapies they need.

Alexion has several programs in place to help patients:

OneSource™ is a personalized program that provides education, assistance with access, and treatment support for patients and their caregivers.

The Alexion Access Foundation was established to help patients who do not have insurance, access to insurance, or any other means for obtaining Alexion medicines.

Alexion supports Patient Assistance Programs that are administered by independent, non-profit, charitable organizations to help cover disease- and treatment-related costs for eligible patients.

The Company also supports advocacy groups and charitable foundations, as we know they are a critical part of supporting the patient community.

OUR FUTURE

Alexion is dedicated to transforming the lives of patients by developing and delivering innovative therapies for devastating and rare diseases.

As the global leader in complement biology, Alexion continues to focus on novel approaches to best serve patients with severe complement-mediated diseases. The Company has several product candidates in its complement inhibitor portfolio being investigated for the treatment of rare and devastating disorders, including:

- **Eculizumab**—a C5 complement inhibitor being evaluated in:
 - Neuromyelitis optica spectrum disorder (NMOSD)
 - Refractory generalized myasthenia gravis (gMG)
 - Delayed graft function (DGF)
 - Antibody-mediated rejection (AMR)
- **ALXN1210**—a longer-acting complement inhibitor being evaluated for patients with PNH
- **ALXN1007**—a novel anti-inflammatory antibody being investigated in Gastrointestinal Graft-versus-Host Disease (GI-GVHD) and Antiphospholipid Syndrome (APS)

As Alexion continues to serve more patients with its approved enzyme replacement therapies, the Company is building a metabolic rare disease franchise with the development of other potential therapies, including:

- **SBC-103**—an enzyme replacement therapy being investigated for patients with mucopolysaccharidosis (MPS) IIIIB
- **ALXN1101 (cPMP Replacement Therapy)**—an enzyme cofactor replacement therapy being evaluated for patients with molybdenum cofactor deficiency (MoCD) Type A

Beyond the current clinical programs, Alexion is also focused on expanding its preclinical pipeline and has more than 30 diverse preclinical programs across a range of therapeutic modalities.