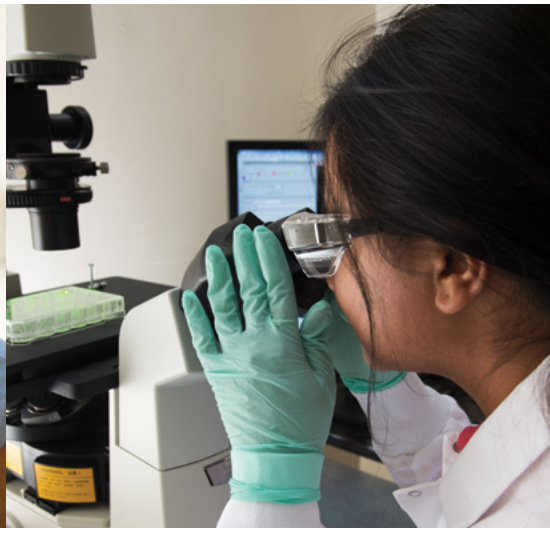
ABOUT ALEXION



COMPANY OVERVIEW

Alexion Pharmaceuticals, Inc. is a global biopharmaceutical company focused on serving patients and families affected by rare diseases through the discovery, development, and commercialization of life-changing therapies. For more than 25 years, Alexion has given hope to people who had none and shined a light on those who felt isolated and alone.

Through the transformative power of our science, Alexion has delivered solutions to challenges that once seemed impossible for patients with devastating complement-mediated and metabolic disorders. As the global leader in complement biology, Alexion developed Soliris® (eculizumab), the world's first and only approved complement inhibitor, for the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH), atypical hemolytic uremic syndrome (aHUS), and anti-acetylcholine receptor (AchR) antibody-positive generalized myasthenia gravis (gMG).

Alexion also has two highly innovative enzyme replacement therapies for patients with life-threatening and ultra-rare metabolic disorders: Strensiq® (asfotase alfa) for patients with hypophosphatasia (HPP) and Kanuma® (sebelipase alfa) for patients with lysosomal acid lipase deficiency (LAL-D).

Consistently recognized for our innovation, Alexion has been named on the Forbes List of World's Most Innovative Companies for the last seven years.

COMPANY INFORMATION

1992 FOUNDED

ALXN NASDAQ

ALEXION.COM

20+ YEARS OF LEADERSHIP IN COMPLEMENT BIOLOGY

3 APPROVED THERAPIES FOR PATIENTS WITH 5 DEVASTATING RARE DISEASES

2,500 TALENTED EMPLOYEES

50 SERVING PATIENTS IN 50 COUNTRIES

BOSTON
HEADQUARTERS IN BOSTON, MASS.

NEW HAVEN
COMPLEMENT RESEARCH CENTER OF EXCELLENCE IN NEW HAVEN, CONN.

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Alexion Corporate Communications. Updated October 2018.

ALEXION®

RESEARCH & DEVELOPMENT

At Alexion, we know that fighting rare diseases requires persistence, dedication, and a relentless pursuit of the highest levels of medical innovation. Through our research and development programs, we aim to deliver life-changing therapies to patients suffering from rare diseases. Alexion's research efforts are focused on leveraging our 20+ years of experience in complement biology to pursue novel molecules and targets in the complement cascade. Our development efforts focus on the core therapeutic areas of hematology, nephrology, neurology, and metabolic disorders, including expanding Soliris® into new indications. We are also pursuing disciplined business development opportunities to build a leading rare disease pipeline that is diversified by development stage and risk.

Alexion's lead R&D programs include:

- **Soliris® for NMOSD**—Alexion is evaluating Soliris® as a potential treatment for patients with neuromyelitis optica spectrum disorder (NMOSD), a life-threatening, rare neurological disease, in the Phase 3 PREVENT study.
- **ALXN1210 IV for PNH**—Alexion is evaluating ALXN1210, an investigational, long-acting C5 inhibitor, administered intravenously every eight weeks in patients with PNH. Alexion has completed two Phase 3 studies in adults with PNH—one in complement inhibitor treatment-naïve patients and one in patients who were stable on Soliris®. A Phase 3 study in children and adolescents who have PNH is currently underway.
- **ALXN1210 IV for aHUS**—Alexion is evaluating ALXN1210 administered intravenously every eight weeks in Phase 3 trials in complement inhibitor treatment-naive adolescent and adult patients with aHUS, as well as in pediatric patients with aHUS.
- **WTX101 for Wilson Disease**—Alexion is evaluating WTX101, a first-in-class copper-binding agent with a unique mechanism of action, as a novel therapy for Wilson disease, a rare, chronic, genetic, and potentially life-threatening liver disorder of impaired copper transport. A Phase 3 study is underway in patients with Wilson disease ages 18 and older.
- **ALXN1210 Subcutaneous**—Alexion plans to initiate a single, PK-based Phase 3 study of ALXN1210 delivered subcutaneously once per week in patients with PNH and aHUS. The Company is also exploring a next-generation subcutaneous formulation of ALXN1210 to potentially extend the dosing interval to once every two weeks or once every four weeks.
- **ALXN1210 IV for Generalized Myasthenia Gravis (gMG)**—Alexion plans to initiate a study with ALXN1210 for the treatment of gMG, a debilitating, chronic and progressive autoimmune neuromuscular disease.
- **ALXN1210 IV for IgA Nephropathy (IgAN)**—Alexion plans to initiate a study with ALXN1210 for the treatment of IgAN, a form of immune complex-mediated glomerulonephritis characterized by granular deposits of IgA and C3, in 2018.
- **SYNT001 for rare IgG-mediated diseases**—Alexion is currently evaluating anti-FcRn antibody SYNT001 in Phase 1b/2a studies in patients with warm autoimmune hemolytic anemia (WAIHA) and in patients with pemphigus vulgaris (PV) or pemphigus foliaceus (PF). SYNT001, a humanized monoclonal antibody that inhibits the interaction of FcRn with Immunoglobulin G (IgG) and IgG immune complexes, has demonstrated clinical proof of mechanism—a rapid reduction in IgG—and has the potential to treat a number of rare IgG-mediated diseases.

SERVING PATIENTS

Serving patients and their families is Alexion's unwavering mission. Every day, we are inspired to continue to find answers that will transform the lives of patients and redefine what it means to live with a rare disease.

Today, Alexion has 2,500 talented colleagues and global operations in place to serve patients in 50 countries. Our approach to serving patients is driven by education and a deep passion for understanding and meeting the unique needs of patients and families suffering from rare diseases. This includes disease education programs to raise awareness among physicians, and diagnostic initiatives to reduce the multi-year delays that patients with rare diseases often face.

Alexion works collaboratively with private healthcare organizations, policymakers, and governments so that patients with rare diseases have access to the therapies they need. In the United States and Canada, we offer OneSource™, a personalized program that provides education, assistance with access, and support for patients and their caregivers.

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

OUR FUTURE

As we look ahead, Alexion's ambition is to be the global leader in rare diseases. Our legacy will be measured in the families we serve today and the generations that follow. We will give patients hope and more. We will give them a future.

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