

ABOUT ALEXION



COMPANY OVERVIEW

Alexion Pharmaceuticals, Inc. is a global biopharmaceutical company focused on serving patients and families affected by rare diseases through the innovation, 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 six 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.
OPENING SUMMER 2018

NEW HAVEN

COMPLEMENT RESEARCH CENTER OF EXCELLENCE IN NEW HAVEN, CONN.

US/ALL-ALL/16/0002b

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:

- **ALXN1210 IV for PNH**—Alexion is evaluating ALXN1210, an innovative, long-acting C5 inhibitor, administered intravenously every eight weeks in Phase 3 trials in patients with PNH.
- **ALXN1210 IV for aHUS**—Alexion is evaluating ALXN1210 administered intravenously every eight weeks in Phase 3 trials in patients with aHUS.
- **ALXN1210 Subcutaneous**—Alexion is progressing the development of a subcutaneous formulation of ALXN1210 and 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 further extend the dosing interval to once every two weeks or once per month.
- **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.

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.