

# ABOUT ALEXION



JESSE  
LIVING WITH gMG

ALXN  
NASDAQ

FOUNDED  
1992

25+  
YEARS OF  
LEADERSHIP  
IN RARE  
DISEASE



ANNA  
LIVING WITH HPP

SERVING  
PATIENTS IN 50  
COUNTRIES



HEADQUARTERS  
BOSTON, MA



R&D CENTER  
OF EXCELLENCE IN  
NEW HAVEN, CT



3,000+  
TALENTED EMPLOYEES

Alexion Pharmaceuticals, Inc. is a global biopharmaceutical company focused on transforming the lives of people living with rare diseases and devastating conditions through the development and delivery of valued innovative medicines.



5  
APPROVED  
MEDICINES FOR  
7  
RARE DISEASES  
AND DEVASTATING  
CONDITIONS

**SOLIRIS®**  
(ECULIZUMAB)  
**The world's first approved complement inhibitor for the treatment of patients with:**  
**NMOSD**  
ANTI-AQUAPORIN-4 ANTIBODY POSITIVE NEUROMYELITIS  
OPTICA SPECTRUM DISORDER  
**PNH**  
PAROXYSMAL NOCTURNAL HEMOGLOBINURIA  
**aHUS**  
ATYPICAL HEMOLYTIC UREMIC SYNDROME  
**AchR+ gMG**  
ANTI-ACETYLCHOLINE RECEPTOR ANTIBODY-POSITIVE GENERALIZED MYASTHENIA GRAVIS

**ULTOMIRIS®**  
(RAVULIZUMAB-CWVZ)  
**The first and only long-acting C5 inhibitor for the treatment of:**  
**Adults with**  
**PNH**  
PAROXYSMAL NOCTURNAL HEMOGLOBINURIA  
**Adults and pediatric patients one month of age and older with**  
**aHUS**  
ATYPICAL HEMOLYTIC UREMIC SYNDROME  
*to inhibit complement-mediated thrombotic microangiopathy (TMA)*

**STRENSIQ®**  
(ASFOTASE ALFA)  
**For the treatment of patients with:**  
**HPP**  
HYPOPHOSPHATASIA

**KANUMA®**  
(SEBELIPASE ALFA)  
**For the treatment of patients with:**  
**LAL-D**  
LYSOSOMAL ACID LIPASE DEFICIENCY

**ANDEXXA®**  
(COAGULATION FACTOR XA [RECOMBINANT], INACTIVATED-ZHZO)  
**For the treatment of patients with:**  
**life-threatening bleeds**

**OUR VALUES**  
**SERVE PATIENTS**  
**EMPOWER PEOPLE**  
**INNOVATE FOR SOLUTIONS**  
**ACT WITH INTEGRITY**



SUPPORTING OUR MISSION TO TRANSFORM THE LIVES OF PEOPLE AFFECTED BY RARE AND DEVASTATING DISEASE WHILE CREATING VALUE FOR ALL OUR STAKEHOLDERS.

- SERVE** COMMUNITIES AND SUSTAIN OUR PLANET
- TRANSFORM** PATIENT LIVES
- ADVANCE** OUR PEOPLE AND OUR COMPANY
- REDEFINE** WHAT IT MEANS TO LIVE WITH A RARE DISEASE

**ETHICS & COMPLIANCE: OUR FOUNDATION**



**8,500+**  
 VOLUNTEER HOURS  
 BY  
**1,700+**  
 EMPLOYEES DURING 2019 GLOBAL DAY OF SERVICE

Our innovation begins with understanding people living with rare diseases, which fuels all of our efforts, beginning with our own medicine discovery efforts, as well as collaboration with external partners.

**WE ARE SPEEDING RARE DISEASE DIAGNOSES WITH:**

RADY CHILDREN'S SEMA4  
 BOSTON CHILDREN'S

At Alexion, our passion drives us to continuously innovate and create meaningful value in all we do. In doing so, we change lives for the better – ours, people living with rare diseases and devastating conditions, and the communities we serve. Every day.

**ALEXION'S LEAD R&D PROGRAMS INCLUDE:**

**ULTOMIRIS® (ravulizumab-cwvz)** is a long-acting C5 inhibitor. It is being evaluated for the treatment of a number of hematologic, nephrologic and neurologic disorders.

ALEXION HAS INITIATED:

- Phase 3 study in **gMG**
- Phase 3 study in **NMOSD**
- Phase 3 study in **Amyotrophic Lateral Sclerosis (ALS)**
- Phase 3 study in **Severe COVID-19**
- Phase 3 study in **Hematopoietic Stem Cell Transplant-Associated Thrombotic Microangiopathy (HSCT-TMA)**
- Phase 3 subcutaneous study

ALEXION PLANS TO INITIATE:

- Phase 3 study in **Complement Mediated Thrombotic Microangiopathy (CM-TMA)**
- Proof-of-concept trial in patients with **IgA nephropathy** and **lupus nephritis** in 2020

**ALXN1840** is a novel oral copper-protein-binding agent with a unique mechanism of action, under investigation as a novel therapy for **Wilson disease**. Enrollment is complete in a Phase 3 study of ALXN1840 in Wilson disease. Study results are expected in the first half of 2021.

**CAEL-101** is a first-in-class amyloid fibril targeted therapy for **light chain (AL) amyloidosis**. Enrollment is underway in two parallel Phase 3 studies - one in patients with Mayo stage IIIa disease and one in patients with Mayo stage IIIb disease.

**ALXN2060 (AG10) - Eidos** Alexion holds an exclusive license to develop and commercialize ALXN2060 (AG10) in Japan. Eidos is currently evaluating AG10 in a Phase 3 study in the U.S. and Europe for **ATTR cardiomyopathy (ATTR-CM)** and plans to begin a Phase 3 study in **ATTR polyneuropathy (ATTR-PN)** in 2020. Alexion plans to initiate a Phase 3 bridging study of ALXN2060 for patients with ATTR-CM in Japan by the end of 2020.

**ALEXION HAS ADDITIONAL R&D PROGRAMS IN COLLABORATION WITH: COMPLEMENT PHARMA DICERNA ZEALAND PHARMA HALOZYME THERAPEUTICS**