

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 and devastating diseases through the development and delivery of valued innovative medicines.



4 APPROVED THERAPIES FOR PATIENTS WITH **6** DEVASTATING RARE DISEASES

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



3
PRIX GALIEN
AWARDS

OUR VALUES

- SERVE PATIENTS
- EMPOWER PEOPLE
- INNOVATE FOR SOLUTIONS
- ACT WITH INTEGRITY



CSR-S.T.A.R

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



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 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 subcutaneous study

ALEXION PLANS TO INITIATE:

- Phase 3 study in **Amyotrophic Lateral Sclerosis (ALS)**
- Phase 3 study in **Hematopoietic Stem Cell Transplant-Associated Thrombotic Microangiopathy (HSCT-TMA)**
- Phase 3 study in **Complement Mediated Thrombotic Microangiopathy (CM-TMA)**

ALXN1830 is a humanized monoclonal antibody that has the potential to improve treatment in a number of rare IgG-mediated diseases. Alexion plans to initiate a Phase 2 study in **warm autoimmune hemolytic anemia (WAIHA)** and a Phase 2 SC trial in **gMG**.

ALXN1840 is a novel oral copper-protein-binding agent with a unique mechanism of action, under investigation as a novel therapy for **Wilson Disease**. Alexion has initiated a Phase 3 superiority trial and is in the process of completing enrollment.

CAEL-101 is a first-in-class amyloid fibril targeted therapy for **light chain (AL) amyloidosis**. Alexion plans to initiate a pivotal Phase 3 study.

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