

ABOUT ALEXION



SERVING PATIENTS IN
50
COUNTRIES

FOUNDED
1992

30
YEARS OF LEADERSHIP
IN RARE DISEASE



Alexion is 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

KOSELUGO®
(SELUMETINIB)
The first and only FDA-approved treatment for children 2 years of age and older with:
NEUROFIBROMATOSIS TYPE 1
(NF1) PLEXIFORM NEUROFIBROMAS **(PN)**



OUR VALUES



**We Follow
the Science**



**We Put
Patients First**



**We Play
to Win**



**We Do the
Right Thing**



**We are
Entrepreneurial**

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
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 PHASE 3 PROGRAMS INCLUDE:

SOLIRIS® (eculizumab), a first-in-class C5 complement inhibitor, for Guillain-Barre syndrome (GBS).

ULTOMIRIS® (ravulizumab-cwvz), a long-acting C5 inhibitor, for:

- Subcutaneous QW for AhuS & PNH
- generalized Myasthenia Gravis (gMG)
- Neuromyelitis Optica Spectrum Disorder (NMOSD)
- Hematopoietic Stem Cell Transplant-Associated Thrombotic Microangiopathy (HSCT-TMA)
- Complement Mediated Thrombotic Microangiopathy (CM-TMA)

KOSELUGO® (selumetinib), a kinase inhibitor, for Neurofibromatosis Type 1 (NF1), Plexiform Neurofibromas (PN) in adults.

ALXN1840, an investigational, oral, targeted de-coppering therapy, for Wilson disease.

CAEL-101, an investigational first-in-class amyloid fibril targeted therapy, for AL Amyloidosis.

Acoramidis (ALXN2060) is an investigational, oral, small molecule. Alexion holds an exclusive license to develop and commercialize acoramidis in Japan, for Transthyretin Amyloidosis (ATTR).

Danicopan (ALXN2040), an investigational, oral, factor D inhibitor, for PNH with extravascular hemolysis (EVH).

**ALEXION HAS
ADDITIONAL R&D PROGRAMS
IN COLLABORATION WITH:
DICERNA
NEURIMMUNE
ZEALAND PHARMA**