

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 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 and pediatric patients one month of age and older with

aHUS

to inhibit complement-mediated thrombotic microangiopathy (TMA)

PNH

Adults with
AchR+ gMG

STRENSIQ® (ASFOTASE ALFA)

For the treatment of patients with:

HPP HYPOPHOSPHATASIA

KANUMA® (SEBELIPASE ALFA)

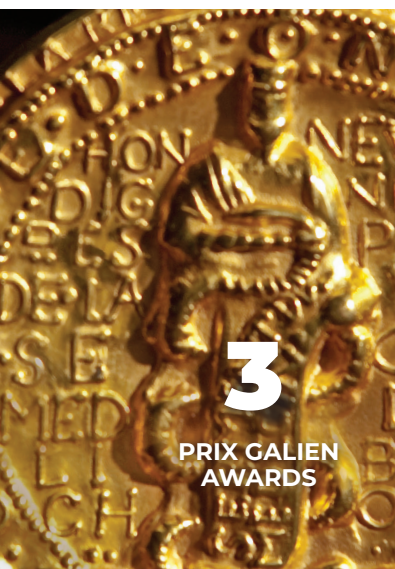
For the treatment of patients with:

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:

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

- Subcutaneous QW for aHUS & PNH
- Neuromyelitis Optica Spectrum Disorder (NMOSD)
- Hematopoietic Stem Cell Transplant-Associated Thrombotic Microangiopathy (HSCT-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.

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

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

Acoramidis, an investigational, oral, small molecule for Transthyretin Amyloid Cardiomyopathy (ATTR-CM). Alexion holds an exclusive license to develop and commercialize acoramidis in Japan.

Gefurulimab, an investigational, anti-C5 albumin-binding humanized bispecific V_HH antibody optimized for subcutaneous delivery.

**ALEXION HAS
ADDITIONAL R&D PROGRAMS
IN COLLABORATION WITH:**

**DICERNA
MERCK
NEURIMMUNE
ZEALAND PHARMA**