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
ATYPICAL HEMOLYTIC UREMIC SYNDROME
to inhibit complement-mediated thrombotic microangiopathy (TMA)

PNH

Adults with

AchR+ gMG

NMOSD

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.

COLLABORATING TO SPEED 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:

- Hematopoietic Stem Cell Transplant-Associated Thrombotic Microangiopathy (HSCT-TMA)
- Cardiac surgery-associated acute kidney injury (CSA-AKI)

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

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

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

Danicopan for PNH with extravascular hemolysis (EVH)

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