COMPLEMENT FRANCHISE
METABOLIC FRANCHISE

Alexion Pipeline

MARKET

Soliris® (eculizumab) for Paroxysmal Nocturnal Hemoglobinuria (PNH)

Soliris is approved for the treatment of PNH in nearly 50 countries, including the United States, EU and Japan. PNH is an ultra-rare blood disorder in which chronic, uncontrolled activation of complement, a component of the normal immune system, results in hemolysis (destruction of the patient's red blood cells).

Soliris® (eculizumab) for Atypical Hemolytic Uremic Syndrome (aHUS)

Soliris is approved for the treatment of aHUS in nearly 40 countries, including the United States, EU and Japan. aHUS is a chronic, ultra-rare, and life-threatening disease in which a lifelong and permanent genetic deficiency in one or more complement regulatory genes causes chronic uncontrolled complement activation, resulting in complement-mediated thrombotic microangiopathy (TMA), the formation of blood clots in small blood vessels throughout the body.

Strensiq® (asfotase alfa) for Hypophosphatasia (HPP)

Strensiq is approved in the United States, EU, Japan and Canada for the treatment of patients with HPP, a genetic, chronic and progressive ultra-rare metabolic disease characterized by defective bone mineralization. HPP can cause destruction and deformity of bones and other skeletal abnormalities, as well as systemic complications such as profound muscle weakness, seizures, pain, and respiratory failure leading to premature death in infants.

Kanuma® (sebelipase alfa) for Lysosomal Acid Lipase Deficiency (LAL-D)

Kanuma is approved in the United States, EU and Japan for the treatment of patients with LAL-D, a genetic, chronic and rare metabolic disease associated with multi-systemic organ damage including hepatic fibrosis, cirrhosis, liver failure, accelerated atherosclerosis, cardiovascular disease, and other devastating consequences.

REGISTRATION

Eculizumab for Refractory Generalized Myasthenia Gravis (gMG)

Alexion has submitted marketing applications in the U.S., EU and Japan for Soliris for the treatment of patients with refractory gMG who are anti-acetylcholine receptor (AChR) antibody-positive. Refractory gMG patients who are AChR antibody-positive represent an ultra-rare segment of patients with MG—a debilitating, complement-mediated neuromuscular disease—who experience severe morbidities despite currently available MG therapies.

ADVANCED CLINICAL DEVELOPMENT

Eculizumab for Relapsing Neuromyelitis Optica Spectrum Disorder (NMOSD)

Eculizumab is being investigated in relapsing NMOSD, a life-threatening, ultra-rare, autoimmune neurological disorder, in which complement activation by antibodies against aquaporin-4 on astrocyte cell surfaces causes damage in the central nervous system, including the spinal cord and optic nerve. The disease leads to severe weakness, paralysis, respiratory failure, loss of bowel and bladder function, blindness and premature death. Most patients experience an unpredictable, relapsing course of disease where each individual attack adds to cumulative neurologic disability.

ALEXION

COMPLEMENT FRANCHISE
METABOLIC FRANCHISE

Alexion Pipeline

Eculizumab for Antibody Mediated Rejection (AMR)

Eculizumab is being investigated in patients undergoing kidney transplant who are at elevated risk of AMR, a severe and potentially life-threatening condition that can lead to kidney allograft damage, resulting in rapid loss of function and possible loss of the transplanted kidney.

ALXN1210 for PNH

ALXN1210 is a highly innovative, longer-acting anti-C5 antibody being evaluated for the treatment of patients with PNH, a severe and ultra-rare blood disorder. Alexion has completed enrollment in a Phase 3 trial comparing ALXN1210 administered intravenously every eight weeks to Soliris in complement inhibitor treatment-naive patients with PNH. Alexion has also initiated a Phase 3 PNH Switch study of ALXN1210 administered intravenously every eight weeks compared to patients currently treated with Soliris.

ALXN1210 for aHUS

ALXN1210 is a highly innovative, longer-acting anti-C5 antibody being evaluated for the treatment of patients with aHUS, a chronic, ultra-rare, and life-threatening disease. Alexion has a Phase 3 trial under way with ALXN1210 administered intravenously every eight weeks in complement inhibitor treatment-naive adolescent and adult patients with aHUS.

EARLY CLINICAL DEVELOPMENT

ALXN1210–Subcutaneous

ALXN1210 is a highly innovative, longer-acting anti-C5 antibody. The ALXN1210 subcutaneous clinical program is under way in healthy volunteers.

PRECLINICAL

Multiple Complement

Alexion's Research & Development pipeline includes multiple complement inhibitor candidates for the potential treatment of severe and devastating complement-mediated disorders.