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.

Soliris® (eculizumab) for AchR+ Generalized Myasthenia Gravis (gMG)

Soliris® (eculizumab) is approved in the U.S. and Japan for the treatment of adult patients with generalized myasthenia gravis (gMG) who are anti-acetylcholine receptor (AchR) antibody-positive and in the European Union for the treatment of refractory gMG in adult patients who are anti-AchR antibody-positive. MG is a debilitating, chronic and progressive autoimmune neuromuscular disease that typically begins with weakness in the muscles that control the movements of the eyes and eyelids, and often progresses to the more severe and generalized form, known as gMG, with weakness of the head, neck, trunk, limb and respiratory muscles. While most patients with gMG can be managed with current MG therapies, 10% to 15% of patients fail to respond adequately to or cannot tolerate multiple therapies for MG and continue to suffer profound muscle weakness and severe disease symptoms that limit function.

ADVANCED CLINICAL DEVELOPMENT

Soliris® (eculizumab) for Relapsing Neuromyelitis Optica Spectrum Disorder (NMOSD)

Soliris® (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

ALXN1210 IV for PNH

ALXN1210 is an innovative, long-acting C5 inhibitor 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 and in a Phase 3 PNH Switch study of ALXN1210 administered intravenously every eight weeks compared to patients currently treated with Soliris.

ALXN1210 IV for aHUS

ALXN1210 is an innovative, long-acting C5 inhibitor 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, as well as a Phase 3 trial of ALXN1210 in pediatric patients with aHUS.

EARLY CLINICAL DEVELOPMENT

ALXN1210 Subcutaneous QW

ALXN1210 is an innovative, long-acting C5 inhibitor. Initial pharmacokinetic and tolerability data from a Phase I study in healthy volunteers support progressing the development of a subcutaneous formulation of ALXN1210. Based on discussions with regulators, Alexion plans to initiate a single, PK-based Phase 3 study of ALXN1210 delivered subcutaneously once per week to support registration in PNH and aHUS in late 2018.

ALXN1210 IV for Generalized Myasthenia Gravis (gMG)

ALXN1210 is an innovative, long-acting C5 inhibitor. Alexion plans to initiate a study with ALXN1210 for the treatment of gMG, a debilitating, chronic and progressive autoimmune neuromuscular disease, in 2018.

PRECLINICAL

ALXN1210 IV for IgA Nephropathy (IgAN)

ALXN1210 is an innovative, long-acting C5 inhibitor. Alexion plans to initiate a study with ALXN1210 for the treatment of IgAN, a form of immune complex-mediated glomerulonephritis characterized by granular deposits of IgA and C3, in 2018.

ALXN1210 Next-Generation Subcutaneous Q2W or Q4W

ALXN1210 is an innovative, long-acting C5 inhibitor. Alexion plans to utilize the Enhanze® drug-delivery technology to extend dosing of a next-generation of ALXN1210 delivered subcutaneously to either once every two weeks or once every four weeks.

Additional Complement

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

ALEXION