Alexion Pipeline

ADVANCED CLINICAL DEVELOPMENT

ULTOMIRIS® (ravulizumab-cwvz) IV for PNH

ULTOMIRIS, a long-acting C5 inhibitor, is approved for the treatment of adults with PNH, a severe and 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). A Phase 3 study in children and adolescents who have PNH is underway.

ULTOMIRIS IV for aHUS

ULTOMIRIS is a long-acting C5 inhibitor being evaluated for the treatment of patients with aHUS, a chronic, ultra-rare, and life-threatening disease. Alexion has completed a Phase 3 trial of ULTOMIRIS administered intravenously every eight weeks in complement inhibitor treatment-naive adults with aHUS. A Phase 3 study in children and adolescents who have aHUS is underway.

ULTOMIRIS IV for Neuromyelitis Optica Spectrum Disorder (NMOSD)

ULTOMIRIS is a long-acting C5 inhibitor. Alexion plans to initiate a Phase 3 study of ULTOMIRIS in NMOSD by the end of 2019, pending regulatory feedback.

ULTOMIRIS IV for Hematopoietic Stem Cell Transplant-Associated Thrombotic Microangiopathy (HSCT-TMA)

ULTOMIRIS is a long-acting C5 inhibitor. Alexion plans to initiate a Phase 3 study of ULTOMIRIS in HSCT-TMA in the first half of 2020, pending regulatory feedback.

SOLIRIS® (eculizumab) for Generalized Myasthenia Gravis (gMG)

SOLIRIS, a first-in-class complement inhibitor, is approved for the treatment of adults with generalized myasthenia gravis (gMG). A Phase 3 study of SOLIRIS in children and adolescents with gMG is underway.

SOLIRIS for Neuromyelitis Optica Spectrum Disorder (NMOSD)

SOLIRIS, a first-in-class complement inhibitor, is approved for the treatment of adults with anti-aquaporin-4 (AQP4) antibody positive neuromyelitis optica spectrum disorder (NMOSD). Alexion plans to initiate a Phase 3 study in children and adolescents with NMOSD by the end of 2019.

ALXN1840 for Wilson Disease

ALXN1840 (bis-choline tetrathiomolybdate) is a novel oral copper-protein binding agent with a unique mechanism of action, under investigation for Wilson disease, a rare, chronic, genetic, and potentially life-threatening liver disorder of impaired copper transport. A Phase 3 study is underway in patients with Wilson disease ages 18 and older.

ULTOMIRIS Subcutaneous QW

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ULTOMIRIS is a long-acting C5 inhibitor. A single, PK-based Phase 3 study of ULTOMIRIS delivered subcutaneously once per week is underway to support registration in PNH and aHUS. Data are expected in early 2020.

ULTOMIRIS IV for Generalized Myasthenia Gravis (gMG)
ULTOMIRIS is a long-acting C5 inhibitor. A Phase 3 study of ULTOMIRIS in gMG is underway.

EARLY CLINICAL DEVELOPMENT

ULTOMIRIS IV for Amyotrophic Lateral Sclerosis (ALS)
ULTOMIRIS is a long-acting C5 inhibitor. Alexion plans to initiate a proof-of-concept study of ULTOMIRIS in ALS in early 2020, pending regulatory feedback.

ULTOMIRIS IV for Primary Progressive Multiple Sclerosis (PPMS)
ULTOMIRIS is a long-acting C5 inhibitor. Alexion plans to initiate an exploratory clinical study of ULTOMIRIS in PPMS.

ALXN1810 Subcutaneous Q2W or Q4W
Alexion has completed a Phase 1 study of subcutaneous ULTOMIRIS co-administered with Halozyme's ENHANZE® drug-delivery technology, recombinant human hyaluronidase enzyme (rHuPH20), a next-generation subcutaneous formulation called ALXN1810.

ALXN1830 for Warm Autoimmune Hemolytic Anemia (WAIHA) and Generalized Myasthenia Gravis (gMG)
ALXN1830 is a humanized monoclonal antibody that inhibits the interaction of neonatal Fc receptor (FcRn) with Immunoglobulin G (IgG) and IgG immune complexes and has the potential to improve treatment in a number of rare IgG-mediated diseases. Alexion plans to initiate a Phase 2/3 study of ALXN1830 (SYNT001) in warm autoimmune hemolytic anemia (WAIHA) in early 2020. In addition, Alexion plans to initiate a Phase 1 study of a subcutaneous formulation of ALXN1830 in healthy volunteers in early 2020. Pending results from the Phase 1 study, Alexion plans to initiate a Phase 2/3 study of subcutaneous ALXN1830 in gMG in 2020.

CAEL-101
Alexion is collaborating with Caelum Biosciences to develop CAEL-101 for light chain (AL) amyloidosis, a rare systemic disorder that causes misfolded immunoglobulin light chain protein to build up in and around tissues, resulting in progressive and widespread organ damage. CAEL-101 is a first-in-class amyloid fibril targeted therapy designed to improve organ function by reducing or eliminating amyloid deposits in patients with AL amyloidosis. Pending regulatory feedback, a Phase 2/3 study investigating CAEL-101 as an add-on to current standard-of-care therapy is planned to begin in 2020.

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ABY-039
Alexion is partnering with Affibody AB to co-develop ABY-039 for rare Immunoglobulin G (IgG)-mediated autoimmune diseases. Currently in Phase 1 development, ABY-039 is a bivalent antibody-mimetic that targets the neonatal Fc receptor (FcRn).

PRECLINICAL

ALXN1720
Alexion plans to initiate a Phase 1 study of ALXN1720, a novel anti-C5 albumin-binding bi-specific mini-body that binds and prevents activation of human C5, in late 2019.

Peptide Therapies
Alexion is collaborating with Zealand Pharma A/S to discover and develop novel peptide therapies for up to four targets in the complement pathway.

GalXC™ RNA interference (RNAi)
Alexion and Dicerna are jointly discovering and developing up to four subcutaneously delivered GalXC™ RNAi candidates for the treatment of complement-mediated diseases.

CP010
Alexion and Complement Pharma are co-developing C6 complement inhibitor CP010 for neurodegenerative disorders.

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