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Soliris® (eculizumab) Granted Orphan Drug Designation in Japan for the Treatment of Patients with Neuromyelitis Optica

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced that Soliris® (eculizumab) has been granted orphan drug designation (ODD) by Japan's Ministry of Health, Labour and Welfare (MHLW) for the treatment of patients with neuromyelitis optica (NMO), a life-threatening, ultra-rare neurologic disorder. In patients with NMO, chronic, uncontrolled complement activation results in severe damage to the central nervous system (CNS), predominantly impacting the optic nerve and spinal cord.[1-3] This devastating disease is characterized by severe weakness, paralysis, respiratory failure, loss of bowel and bladder function, blindness and premature death.[4-6]

"The orphan drug designation for eculizumab for NMO highlights the significant need for an effective and innovative treatment option for patients in Japan suffering from this debilitating and life-threatening disease," said Martin Mackay, Ph.D., executive vice president and global head of R&D at Alexion. "By specifically inhibiting the terminal complement pathway, eculizumab has the potential to improve outcomes for patients with NMO. We look forward to evaluating the clinical benefits of eculizumab in NMO in our registration study, known as PREVENT, which is currently enrolling patients."

The MHLW, based on the opinion of the Pharmaceutical Affairs and Food Sanitation Council, grants orphan status to drugs and medical devices that treat serious diseases of high unmet medical need that affect fewer than 50,000 patients in Japan. ODD provides drug developers with certain benefits and incentives, including priority review for marketing authorization and a period of 10 years of market exclusivity if regulatory approval is received for the designated indication.

Alexion is enrolling patients in a multinational, placebo-controlled registration trial of eculizumab in patients with relapsing NMO, known as the PREVENT (**P**revention of **R**elapses and **EV**aluation of **E**culizumab in **N**MO **T**reatment) study. More information on this trial is available at www.clinicaltrials.gov under the identifier NCT01892345.

Soliris is a first-in-class terminal complement inhibitor and is currently approved in the United States, European Union, Japan and other countries for the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS), two debilitating, ultra-rare and life-threatening disorders caused by chronic uncontrolled complement activation. Soliris is not approved in any country for the treatment of NMO. In 2013, Soliris was granted ODD in both the U.S. and EU for the treatment of NMO.

About Neuromyelitis Optica

In patients with neuromyelitis optica (NMO), binding of NMO-IgG antibody to astrocytes results in uncontrolled complement activation and destruction of myelin-producing cells, leading to severe damage to the central nervous system and predominantly impacting the spinal cord and optic nerve.[1-3] The disease is characterized by severe weakness, paralysis, respiratory failure, loss of bowel and bladder function, blindness and premature death.[4-6] Patients with NMO have a life-long exposure to the uncontrolled terminal complement activation due to chronic autoimmune attack, and most patients experience an unpredictable, relapsing course of disease with cumulative disability, as each attack adds to the neurologic disability.[5,7,8] Fifty percent of relapsing NMO patients have been reported to sustain permanent severe disability, including paralysis and blindness, within five years of disease onset.[9] Most NMO-related deaths result from respiratory complications from NMO attacks.[9,10] The disease primarily affects women, with a female to male ratio as high as a 9:1.[11] Currently, there are no approved treatments for NMO.

About Soliris® (eculizumab)

Soliris is a first-in-class terminal complement inhibitor developed from the laboratory through regulatory approval and commercialization by Alexion. Soliris is approved in the U.S. (2007), European Union (2007), Japan (2010) and other countries as the first and only treatment for patients with paroxysmal nocturnal hemoglobinuria (PNH) to reduce hemolysis. PNH is a debilitating, ultra-rare and life-threatening blood disorder, characterized by complement-mediated hemolysis (destruction of red blood cells). Soliris is also approved in the U.S. (2011), the European Union (2011), Japan (2013) and other countries as the first and only treatment for patients with atypical hemolytic uremic syndrome (aHUS) to inhibit complement-mediated thrombotic microangiopathy, or TMA (blood clots in small vessels). aHUS is a debilitating, ultra-rare and life-threatening genetic disorder characterized by complement-mediated TMA. Soliris is not indicated for the treatment of patients with Shiga-toxin *E. coli*-related hemolytic uremic syndrome (STEC-HUS). For the breakthrough medical innovation in complement inhibition, Alexion and Soliris

have received some of the pharmaceutical industry's highest honors: the Prix Galien USA (2008, Best Biotechnology Product) and France (2009, Rare Disease Treatment).

More information including the full U.S. prescribing information on Soliris is available at www.soliris.net.

Important Safety Information

The U.S. product label for Soliris includes a boxed warning: "Life-threatening and fatal meningococcal infections have occurred in patients treated with Soliris. Meningococcal infection may become rapidly life-threatening or fatal if not recognized and treated early [see Warnings and Precautions (5.1)]. Comply with the most current Advisory Committee on Immunization Practices (ACIP) recommendations for meningococcal vaccination in patients with complement deficiencies. Immunize patients with a meningococcal vaccine at least two weeks prior to administering the first dose of Soliris, unless the risks of delaying Soliris therapy outweigh the risk of developing a meningococcal infection. [See Warnings and Precautions (5.1) for additional guidance on the management of the risk of meningococcal infection]. Monitor patients for early signs of meningococcal infections and evaluate immediately if infection is suspected. Soliris is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS). Under the Soliris REMS, prescribers must enroll in the program [see Warnings and Precautions (5.2)]. Enrollment in the Soliris REMS program and additional information are available by telephone: 1-888-SOLIRIS (1-888-765-4747)."

In patients with PNH, the most frequently reported adverse events observed with Soliris treatment in clinical studies were headache, nasopharyngitis (runny nose), back pain and nausea. Soliris treatment of patients with PNH should not alter anticoagulant management because the effect of withdrawal of anticoagulant therapy during Soliris treatment has not been established. In patients with aHUS, the most frequently reported adverse events observed with Soliris treatment in clinical studies were headache, diarrhea, hypertension, upper respiratory infection, abdominal pain, vomiting, nasopharyngitis, anemia, cough, peripheral edema, nausea, urinary tract infections, pyrexia. Soliris is not indicated for the treatment of patients with Shiga-toxin *E. coli*-related hemolytic uremic syndrome (STEC-HUS). Please see full prescribing information for Soliris, including BOXED WARNING regarding risk of serious meningococcal infection.

About Alexion

Alexion is a biopharmaceutical company focused on serving patients with severe and rare disorders through the innovation, development and commercialization of life-transforming therapeutic products. Alexion is the global leader in complement inhibition and has developed and markets Soliris® (eculizumab) as a treatment for patients with PNH and aHUS, two debilitating, ultra-rare and life-threatening disorders caused by chronic uncontrolled complement activation. Soliris is currently approved in nearly 50 countries for the treatment of PNH, and in nearly 40 countries for the treatment of aHUS. Alexion is evaluating other potential indications for Soliris in additional severe and ultra-rare disorders beyond PNH and aHUS, and is developing other highly innovative biotechnology product candidates across multiple therapeutic areas. This press release and further information about Alexion can be found at www.alexionpharma.com.

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This news release contains forward-looking statements, including statements related to potential medical benefits of Soliris® (eculizumab) for the treatment of neuromyelitis optica (NMO). Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ from those expected, including, for example, decisions of regulatory authorities regarding marketing approval or material limitations on the marketing of Soliris for NMO, delays in arranging satisfactory manufacturing capabilities, the possibility that results of clinical trials are not predictive of safety and efficacy results of Soliris for NMO in broader or different patient populations, decisions of regulatory authorities to require additional testing, the risk that estimates regarding the number of patients with NMO and observations regarding the natural history of patients with NMO are inaccurate, and a variety of other risks set forth from time to time in Alexion's filings with the Securities and Exchange Commission, including but not limited to the risks discussed in Alexion's Quarterly Report on Form 10-Q for the period ended September 30, 2014. Alexion does not intend to update any of these forward-looking statements to reflect events or circumstances after the date hereof, except when a duty arises under law.

References

1. Jarius S, Wildemann B. AQP4 antibodies in neuromyelitis optica: diagnostic and pathogenetic relevance. Nat Rev Neuro. 2010;6:383-92.

2. Hinson SR, Romero MF, Popescu BFG, et al. Molecular outcomes of neuromyelitis optica (NMO)-IgG binding to aquaporin-4 in astrocytes. Proc Nat Acad Sci 2012;109(4):1245-50.

3. Hinson SR, Pittock SJ, Lucchinetti CF, et al. Pathogenic potential of IgG binding to water channel extracellular domain in neuromyelitis optica. Neurology 2007;69:2221-31.

4. Wingerchuk DM, Lennon VA, Lucchinetti CF, Pittock SJ, Weinshenker BG. The spectrum of neuromyelitis optica. Lancet Neuro. 2007;6(9):805-15.

5. Wingerchuk DM. Diagnosis and treatment of neuromyelitis optica. Neurologis. 2007;13(1):2-11.

6. Wingerchuk DM, Weinshenker BG. Neuromyelitis optica. Curr Treat Options Neurol 2008;10(1):55-66.

7. Tuzun E, Kurtuncu M, Turkoglu R, et al. Enhanced complement consumption in neuromyelitis optica and Behcet's disease patients. J Neuroimmunol 2011;233(1-2):211-5.

8. Kuroda H, Fujihara K, Takano R, et al. Increase of complement fragment C5a in cerebrospinal fluid during exacerbation of neuromyelitis optica. J Neuroimmunol 2013;254(1-2):178-82.

9. Wingerchuk DM, Hogancamp WF,O'Brien PC, Weinshenker BG. The clinical course of neuromyelitis optica (Devic's syndrome). Neurology 1999;53(5):1107-14.

10. Kitley J, Leite MI, Nakashima I, et al. Prognostic factors and disease course in aquaporin-4 antibody-positive patients with neuromyelitis optica spectrum disorder from the United Kingdom and Japan. Brain 2012;135(Pt 6):1834-49.

11. Wingerchuk DM. Neuromyelitis optica. Int MS J 2006;13(2):42-50.

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