FDA Grants Priority Review for Alexion's sBLA for Soliris® (eculizumab) as a Treatment for Patients with Atypical Hemolytic Uremic Syndrome (aHUS)

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) announced today that the U.S. Food and Drug Administration (FDA) has granted the Company's request for Priority Review of its supplemental Biologics License Application (sBLA) for Soliris® (eculizumab) as a treatment for patients with atypical Hemolytic Uremic Syndrome (aHUS). A Priority Review designation is given to drugs that may offer major advances in treatment, or provide a treatment where no adequate therapy exists.

If approval is granted, Alexion anticipates that Soliris would be available for U.S. patients with aHUS in the fourth quarter of 2011. The European Medicines Agency (EMA) is also reviewing a marketing application for Soliris as a treatment for patients with aHUS in the European Union.

Both the US and EU sBLA submissions include the positive data from the two 26-week Phase 2 studies of Soliris as a treatment for adult and adolescent patients with aHUS. Primary endpoints in both studies were achieved with statistical significance. Final data from these studies will be presented at the European Hematology Association (EHA) Congress, to be held in London on June 9-12, 2011.[1]

About aHUS

aHUS is a chronic, ultra-rare disease characterized by thrombotic microangiopathy (TMA), the formation of blood clots in small blood vessels throughout the body, causing a reduction in platelet count and life-threatening damage to the kidney, brain, heart and other vital organs.[2-4] Approximately 60 percent of patients with aHUS require dialysis or a kidney transplant or die within a year of diagnosis.[5] The majority of patients with aHUS who receive a kidney transplant experience severe complications of the disease, and more than 90 percent of these patients experience failure of the donor kidney.[6]

aHUS is a progressive disease caused by life-long uncontrolled activation of the complement system due to deficiencies in complement regulatory genes. With genetic deficiency of naturally occurring complement inhibitors, patients experience chronic uncontrolled activation of the complement system, causing ongoing inflammation and blood clots in vital organs.[7,8] In patients with aHUS, uncontrolled complement activation results in an ongoing risk of sudden and catastrophic life-threatening complications. Currently, mutations have been identified in at least ten different genes; however, in approximately one-half of patients diagnosed with aHUS, the specific genetic deficiency cannot currently be identified.

About Soliris

Soliris is a first-in-class terminal complement inhibitor developed from the laboratory through regulatory approval and commercialization by Alexion. Soliris has been approved in the U.S., European Union, Japan and other territories as the first treatment for patients with PNH, an ultra-rare, debilitating and life-threatening blood disorder defined by chronic uncontrolled complement activation which causes chronic red blood cell destruction (hemolysis), leading to blood clots, organ failure, and shortened survival. Prior to these approvals, there were no therapies specifically available for the treatment of patients with PNH. Soliris (eculizumab) is not approved for the treatment of aHUS or other indications other than PNH. Alexion's breakthrough approach to complement inhibition has received some of the pharmaceutical industry's highest honors: the 2008 Prix Galien USA Award for Best Biotechnology Product with broad implications for future biomedical research and the 2009 Prix Galien France Award in the category of Drugs for Rare Diseases. More information on Soliris is available at www.soliris.net.

Important Safety Information

Soliris is generally well tolerated in patients with PNH. The most frequent adverse events observed in clinical studies of patients with PNH were headache, nasopharyngitis (runny nose), back pain and nausea. Treatment with Soliris should not alter anticoagulant management because the effect of withdrawal of anticoagulant therapy during Soliris treatment has not been established.

The U.S. product label for Soliris also includes a boxed warning: "Soliris increases the risk of meningococcal infections. Meningococcal infection may become rapidly life-threatening or fatal if not recognized and treated early. Vaccinate patients with a meningococcal vaccine at least two weeks prior to receiving the first dose of Soliris; revaccinate according to current medical guidelines for vaccine use. Monitor patients for early signs of meningococcal infections, evaluate immediately if infection is

suspected, and treat with antibiotics if necessary." During PNH clinical studies, two out of 196 vaccinated PNH patients treated with Soliris experienced a serious meningococcal infection. Prior to beginning Soliris therapy, all patients and their prescribing physicians are encouraged to enroll in the PNH Registry, which is part of a special risk-management program that involves initial and continuing education and long-term monitoring for detection of new safety findings.

About Alexion

Alexion Pharmaceuticals, Inc. is a biopharmaceutical company focused on serving patients with severe and ultra-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, a debilitating, ultra-rare and life-threatening blood disorder. Soliris is approved in more than 35 countries. Alexion is evaluating other potential indications for Soliris and is pursuing development of other innovative biotechnology product candidates in early stages of development. This press release and further information about Alexion Pharmaceuticals, Inc. can be found at: www.alexionpharma.com.

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Safe Harbor Statement

This news release contains forward-looking statements, including statements related to anticipated clinical development, regulatory and commercial milestones and potential health and medical benefits of Soliris® (eculizumab) for the potential treatment of patients with aHUS. 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 its current or potential new indications, 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 March 31, 2011, and in Alexion's other filings with the Securities and Exchange Commission. 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) Alexion Pharmaceuticals Corporate Website. Press release available from URL: http://www.alxn.com/News/article.aspx?relid=580263

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Alexion Pharmaceuticals, Inc.
Irving Adler, 203-271-8210
Senior Director, Corporate Communications and Public Policy
or
Media:
Makovsky & Company

Mark Marmur, 609-354-8135
or
Investors:
Rx Communications
Rhonda Chiger, 917-322-2569

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