Alexion Submits Applications for Soliris® (Eculizumab) as a Treatment for Patients with Atypical Hemolytic Uremic Syndrome (aHUS) in the United States and European Union

2011 Revenue Guidance Revised Upward

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) announced today that the company has submitted marketing applications to the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) for Soliris® (eculizumab) as a treatment for patients with atypical Hemolytic Uremic Syndrome (aHUS).

Both the US and EU filings include the positive data from the two 26-week Phase 2 studies of Soliris as a treatment for adult and adolescent patients with aHUS. Preliminary data from these two studies were presented at the American Society of Nephrology (ASN) annual meeting in November 2010. [1-3] Primary endpoints in both studies were achieved with statistical significance. aHUS is an ultra-rare, chronic and life-threatening disease in which uncontrolled complement activation causes blood clots in small blood vessels throughout the body (thrombotic microangiopathy, or TMA) leading to kidney failure, stroke, heart attack and death.

"The US and EU regulatory submissions put us one step closer toward accomplishing our goal to transform the lives of patients suffering with aHUS," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "We recognize that patients with aHUS, a life-threatening and ultra-rare disorder, lack adequate treatment options, and we look forward to working with regulatory authorities as they review our applications."

Updated 2011 Revenue Guidance

Alexion is updating its 2011 revenue guidance, from the previously announced range of $715- $735 million, now to the higher range of $720 to $740 million. Other items of previously announced 2011 guidance are being reiterated at this time.

The upward revision in revenue guidance takes into account continued global growth of Soliris for PNH, and the potential for an earlier than previously expected US launch of Soliris for aHUS. The earlier launch could occur if the US regulatory submission is accepted for priority review by the FDA and if a positive decision is then received, making a launch possible late in the fourth quarter of 2011. Further, the upward revision takes into account the potential for disruptions in treatment for individual patients in Japan associated with the recent natural disasters in that country.

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.[4-6] Approximately 60 percent of patients with aHUS require dialysis or a kidney transplant or die within a year of diagnosis.[7] 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.[8]

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.[9,10] 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, a debilitating, ultra-rare 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 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-K for the period ended December 31, 2010, 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) Abstract 1338 entitled "Safety and Efficacy of Eculizumab in aHUS Patients Resistant to Plasma Therapy: Interim Analysis from a Phase II Trial," presented in an oral presentation at the American Society of Nephrology (ASN) Annual Meeting on November 20, 2010 by Dr. Christophe Legendre.

(2) Alexion Pharmaceuticals Corporate Website. Press release available from URL: http://www.alxn.com/News/article.aspx?relid=532082.

(3) Abstract 157 entitled "Safety and Efficacy of Eculizumab in aHUS Patients on Chronic Plasma Therapy: Interim Analysis of a Phase II Trial," presented in a poster presentation at the American Society of Nephrology (ASN) Annual Meeting on November 19, 2010 by Dr. Petra Muus.

(4) Alexion Pharmaceuticals. Soliris in Other Kidney Disorders. Alexion Pharmaceuticals Corporate Website 2010 September 24 Available from: URL: http://www.alexionpharma.com/RandD/Soliris%20In%20Other/kidney%20disorders.aspx#A1.

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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

Source: Alexion Pharmaceuticals, Inc.

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