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European Commission Grants Orphan Drug Designation to Soliris® (Eculizumab) for the Prevention of Graft Rejection Following Solid Organ Transplantation

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (Nasdaq:ALXN) today announced that the European Commission has granted an orphan drug designation (ODD) to Soliris® (eculizumab), a first-in-class terminal complement inhibitor, for the prevention of graft rejection following solid organ transplantation. Graft rejection can cause severe injury to the transplanted organ and is a significant barrier to successful transplantation.

Soliris 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. Alexion is investigating Soliris for the prevention of acute antibody-mediated rejection (AMR) in kidney transplant recipients, and for the prevention of delayed graft function (DGF) in patients receiving deceased donor kidney transplants. Soliris is not approved in any country to prevent or treat rejection following kidney or other solid organ transplantation.

"Rejection after transplantation is a severe and potentially devastating occurrence for patients undergoing organ transplantation due to the very real risk of losing the transplanted organ," said Martin Mackay, Ph.D., Executive Vice President, Global Head of R&D at Alexion. "By specifically inhibiting the terminal complement pathway, Soliris has the potential to lower the risk of rejection, a benefit that could lead to improved clinical outcomes for these patients."

The European Commission grants orphan medicinal product status to provide incentives to develop medicinal products to treat, prevent or diagnose diseases or conditions that affect no more than five in 10,000 persons in the EU. The orphan medicinal product status designation would provide Alexion with certain benefits and incentives, including a period of marketing exclusivity if Soliris is approved in the EU for the orphan therapeutic indication of acute antibody-mediated rejection (AMR).

About AMR

Acute antibody-mediated rejection (AMR) is a severe and potentially life-threatening condition that can lead to severe allograft damage resulting in rapid loss of function and possible loss of the transplanted organ.[1] Patients who are sensitized (have high levels of donor-specific-antibodies [DSAs]) are at high risk for developing acute AMR,[1,2] may have difficulty finding a donor to who they are not sensitized, and therefore may never have the opportunity to have a life-saving transplant. The development of acute AMR is believed to be primarily a result of uncontrolled complement activation caused by DSAs.[1,2] Currently, there are no approved therapies for the prevention of acute AMR.

About Soliris

Soliris® (eculizumab) 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), a debilitating, ultra-rare and life-threatening blood disorder, characterized by complement-mediated hemolysis (destruction of red blood cells). Soliris is indicated to reduce hemolysis. 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), a debilitating, ultra-rare and life-threatening genetic disorder characterized by complement-mediated thrombotic microangiopathy, or TMA (blood clots in small vessels). Soliris is not approved to prevent or treat rejection following kidney or other solid organ transplantation.

Alexion's breakthrough approach in terminal complement inhibition has received 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, including the full 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. 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 Serious Meningococcal Infections (5.1) for additional guidance on the management 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. 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 hypertension, upper respiratory tract infection, diarrhea, headache, anemia, vomiting, nausea, urinary tract infection, and leukopenia. 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 the United States, European Union, Japan and other 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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Safe Harbor Statement

This news release contains forward-looking statements, including statements related to potential medical benefits of Soliris® (eculizumab) for the prevention of rejection after solid organ transplant. 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 rejection after solid organ transplant and the timing of such decisions, delays in arranging satisfactory manufacturing capabilities and establishing commercial infrastructure for Soliris for rejection after solid organ transplant, the possibility that results of clinical trials are not predictive of safety and efficacy results of Soliris for rejection after solid organ transplant, the risk that third party payors (including governmental agencies) will not reimburse for the use of Soliris for rejection after solid organ transplant (if approved) at acceptable rates or at all, the risk that estimates regarding the number of patients with Soliris for rejection after solid organ transplant and observations regarding the natural history of patients with Soliris for rejection after solid organ transplant 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 Annual Report on Form 10-K for the period ended Dec. 31, 2013. 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. Takemoto SK, Zeevi A, Feng S, et al. National conference to assess antibody-mediated rejection in solid organ transplantation. Am J Transplant. 2004; 4(7):1033-41.

2. Collins AB, Schneeberger EE, Pascual MA, et al. Complement activation in acute humoral renal allograft rejection: diagnostic significance of C4d deposits in peritubular capillaries. J Am Soc Nephrol. 1999;10(10):2208-14.

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