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European Commission Grants Orphan Drug Designation to Soliris® (Eculizumab) for Prevention of Delayed Graft Function (DGF) after Solid Organ Transplantation

CHESHIRE, Conn.--(BUSINESS WIRE)-- **Alexion Pharmaceuticals (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 delayed graft function (DGF) after solid organ transplantation. DGF is an early and serious complication of organ transplantation that is characterized by the failure of a transplanted organ to function normally immediately following transplantation. In patients undergoing kidney transplantation, patients who develop DGF require dialysis in order to survive. [1-3]

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. Soliris is not approved in any country to prevent or treat DGF following kidney or other solid organ transplantation. Last month the U.S. Food and Drug Administration (FDA) also granted an orphan drug designation to Soliris for the prevention of DGF in renal transplant patients.

"Delayed graft function after transplantation is a debilitating and life-threatening condition because of the 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, which is believed to play a critical role in the development of DGF, Soliris has the potential to lower the risk of DGF, a benefit that may have positive implications for improved clinical outcomes for transplant 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 market exclusivity if Soliris is approved in the EU to prevent or treat DGF.

Alexion plans to initiate a single multinational DGF registration study in kidney transplant patients with Soliris in 2014 to gather the clinical evidence needed to support approval for this indication.

About Delayed Graft Function (DGF)

DGF is an early and serious complication of organ transplantation that is characterized by the failure of a transplanted organ to function normally immediately following transplantation. When DGF occurs in the setting of kidney transplantation, the patient requires dialysis after the transplant procedure. [1-3] Most often, DGF results from organ injury caused by severe inflammation and complement activation associated with the normal processes for removal and transplantation of the donor organ.[1-4] DGF has a substantial negative impact on graft function both in the short and long term, which can result in premature graft loss, prolonged hospitalization or patient death.[5,6] In addition, as donor organs are in short supply, reducing the risk of DGF for organs that are at higher risk to develop DGF may allow more donor organs to be transplanted. With specific regard to kidney transplantation, 15-20 percent of donor kidneys are reportedly never used and thus discarded each year in the U.S. and Europe due to the risk of poor outcomes associated with DGF[7,8] denying many patients the benefit of transplantation.

About Soliris

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), 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 indicated to inhibit complement-mediated TMA. The effectiveness of Soliris in aHUS is based on its effects on TMA and renal function. Prospective clinical trials in additional patients, the preliminary results of which were reported at international nephrology and hematology conferences in 2013, are ongoing to confirm the benefit of Soliris in patients with aHUS. Soliris is not indicated for the treatment of patients with Shiga-toxin E. coli-related hemolytic uremic syndrome (STEC-HUS). For the breakthrough innovation in complement inhibition, Alexion and Soliris have 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 in Europe is available at:
http://www.ema.europa.eu/docs/en_GB/document_library/EPAR_-_Product_Information/human/000791/WC500054208.pdf

Important Safety Information

In Europe, the Summary of Product Characteristics (SmPC) for Soliris includes a special warning and precaution for use: Due to its mechanism of action, the use of Soliris increases the patient's susceptibility to meningococcal infection (Neisseria meningitidis). These patients might be at risk of disease by uncommon serogroups (particularly Y, W135 and X), although meningococcal disease due to any serogroup may occur. To reduce the risk of infection, all patients must be vaccinated at least 2 weeks prior to receiving Soliris. aHUS patients who are treated with Soliris less than 2 weeks after receiving a meningococcal vaccine must receive treatment with appropriate prophylactic antibiotics until 2 weeks after vaccination. Patients must be re-vaccinated according to current medical guidelines for vaccination use. Tetravalent vaccines against serotypes A, C, Y and W135 are strongly recommended, preferably conjugated ones. Vaccination may not be sufficient to prevent meningococcal infection. Consideration should be given to official guidance on the appropriate use of antibacterial agents.

The most common or serious adverse reactions are headache (occurred mostly in the initial phase), leukopenia and meningococcal infection. Soliris treatment should not alter anticoagulant management. Please see Summary of Product Characteristics for full prescribing information for Soliris, including all special warnings and precautions.

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 delayed graft function (DGF) in renal transplant patients. 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 DGF, delays in arranging satisfactory manufacturing capabilities and establishing commercial infrastructure for Soliris for DGF, the possibility that results of clinical trials are not predictive of safety and efficacy results of Soliris for DGF in broader or different patient populations, the risk that third party payors (including governmental agencies) will not reimburse for the use of Soliris for DGF (if approved) at acceptable rates or at all, the risk that estimates regarding the number of patients with Soliris for DGF and observations regarding the natural history of patients with Soliris for DGF 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.

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