Alexion Receives Three New U.S. Patents for Soliris® (eculizumab), Extending Patent Protection Into 2027

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NEW HAVEN, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) announced today that the United States Patent and Trademark Office (USPTO) has issued U.S. Patents No. 9,732,149; 9,718,880; and 9,725,504, which are directed to the composition of matter of eculizumab (Soliris®), pharmaceutical formulations of eculizumab, and methods of treating paroxysmal nocturnal hemoglobinuria (PNH) with eculizumab, respectively, and which will expire in 2027.

"Alexion is the leader in complement biology and has invested decades of ground-breaking research and development in Soliris. We continue to strengthen our patent portfolio around this unique complement inhibitor," said Ludwig Hantson, Chief Executive Officer of Alexion. "These new patents work in concert with other patents and regulatory exclusivities to protect Soliris in all indications."

The new composition of matter patent is directed to the full-length amino acid sequence of eculizumab and covers molecules that contain the same sequence. The new formulation patent is directed to pharmaceutical compositions that contain eculizumab, independent of their intended use. The new method of use patent is directed to treating PNH with eculizumab, and supplements other patents that are directed to treating atypical hemolytic uremic syndrome (aHUS) and other complement-mediated diseases with eculizumab.

Alexion is pursuing corresponding patent applications in other regions and countries, including Europe and Japan. In addition, Alexion is pursuing patent applications for pending additional indications of Soliris, such as for the treatment of refractory generalized myasthenia gravis (gMG).

About Soliris® (eculizumab)

Soliris® is a first-in-class complement inhibitor that works by inhibiting the terminal part of the complement cascade, a part of the immune system that, when activated in an uncontrolled manner, plays a role in serious ultra-rare disorders like paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS).

Soliris is approved in the U.S., EU, Japan and other countries as the first and only treatment for patients with PNH and aHUS. In addition, Alexion has submitted applications in the U.S., EU and Japan for Soliris as a potential treatment of refractory gMG in patients who are anti-acetylcholine receptor (AChR) antibody-positive. In June 2017, the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) adopted a positive opinion for this new indication. Soliris is not indicated for the treatment of patients with Shiga-toxin E. coli-related hemolytic uremic syndrome (STEC-HUS). Alexion and Soliris have received some of the pharmaceutical industry's highest honors for the medical innovation in complement inhibition: the Prix Galien USA (2008, Best Biotechnology Product) and France (2009, Rare Disease Treatment).

For more information on Soliris, please see full prescribing information for Soliris, including BOXED WARNING regarding risk of serious meningococcal infection, available at www.soliris.net.

Important Soliris Safety Information

The U.S. prescribing information for Soliris includes the following warnings and precautions: 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 Centers for Disease Control (CDC)'s Advisory Committee on Immunization Practices (ACIP) recommendations for meningococcal vaccination in patients with complement deficiencies. Immunize patients with meningococcal vaccines 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. 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) or at **www.solirisrems.com**.

Patients may have increased susceptibility to infections, especially with encapsulated bacteria. Aspergillus infections have occurred in immunocompromised and neutropenic patients. Children treated with Soliris may be at increased risk of developing serious infections due to *Streptococcus pneumoniae* and *Haemophilus influenza* type b (Hib). 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. Administration of Soliris may result in infusion reactions, including anaphylaxis or other hypersensitivity reactions.

In patients with PNH, the most frequently reported adverse events observed with Soliris treatment in clinical studies were headache, nasopharyngitis, back pain and nausea. 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, and pyrexia.

About Alexion

Alexion is a global biopharmaceutical company focused on developing and delivering life-transforming therapies for patients with devastating and rare disorders. Alexion is the global leader in complement inhibition and has developed and commercializes the first and only approved complement inhibitor to treat patients with paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS), two life-threatening ultra-rare disorders. In addition, Alexion has two highly innovative enzyme replacement therapies for patients with life-threatening and ultra-rare metabolic disorders, hypophosphatasia (HPP) and lysosomal acid lipase deficiency (LAL-D). As the leader in complement biology for over 20 years,

Alexion focuses its research efforts on novel molecules and targets in the complement cascade, and its development efforts on the core therapeutic areas of hematology, nephrology, neurology, and metabolic disorders. This press release and further information about Alexion can be found at: www.alexion.com.

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Forward-Looking Statement

This news release contains forward-looking statements, including statements related to Soliris intellectual property, and the strength and scope of Soliris intellectual property protection. Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ from those expected, including for example, the risks and uncertainties of drug development, decisions of regulatory authorities regarding the adequacy of our research, marketing approval or material limitations on the marketing of eculizumab, delays, interruptions or failures in the manufacture and supply of our products and our product candidates, failure to satisfactorily address matters raised by the FDA and other regulatory agencies, the possibility that results of clinical trials are not predictive of safety and efficacy results of our products in broader patient populations, the possibility that clinical trials of our product candidates could be delayed, the adequacy of our pharmacovigilance and drug safety reporting processes, the risk that third party payers (including governmental agencies) will not reimburse or continue to reimburse for the use of our products at acceptable rates or at all, the outcome of challenges and opposition proceedings to our intellectual property, assertion or potential assertion by third parties that the manufacture, use or sale of our products infringes their intellectual property, risks regarding government investigations, including investigations of Alexion by the SEC and DOJ, the risk that anticipated regulatory filings are delayed, the risk that estimates regarding the number of patients with the diseases our products treat are inaccurate, and a variety of other risks set forth from time to time in Alexion's filings with the U.S. 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 June 30, 2017 and in our other filings with the U.S. 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.

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Alexion Pharmaceuticals, Inc.
Media
Arne Naeveke, PhD, 475-230-3774
Executive Director, Product Communications
or
Investors
Elena Ridloff, CFA, 475-230-3601
Vice President, Investor Relations
or
Catherine Hu, 475-230-3599
Director, Investor Relations

Source: Alexion Pharmaceuticals, Inc.