Alexion's Soliris® Receives 2008 Prix Galien USA Award for Best Biotechnology Product

Release Date:
Thursday, September 25, 2008 8:08 am EDT

Terms:
Company News

Recognizes Scientific Accomplishment in Complement Inhibition and Impact of Soliris on Patients Living with PNH

Alexion Pharmaceuticals, Inc. (NASDAQ: ALXN) has received a Prix Galien USA 2008 Award for Best Biotechnology Product for Soliris® (eculizumab). The Award recognizes the scientific innovation represented by the complement-inhibition technology of Soliris, and the impact the drug is having on the lives of patients with paroxysmal nocturnal hemoglobinuria (PNH), a rare, debilitating and life-threatening blood disorder.

Soliris is a first-in-class complement inhibitor that selectively blocks the formation of terminal complement, a component of the normal immune system. Patients with PNH lack naturally occurring proteins that ordinarily prevent terminal complement from causing the red blood cell destruction (hemolysis) that is central to the serious morbidities and mortality associated with PNH.

"We are living in the midst of a biological revolution and the breakthrough agents honored by the Prix Galien USA illustrate the substantial research and development necessary to bring the fruits of that revolution in molecular medicine to the clinic," said Gerald Weissmann, M.D., Prix Galien USA committee chair, New York University professor emeritus and editor-in-chief of The FASEB Journal.

"We deeply appreciate this honor, which recognizes more than 15 years of dedicated complement-based research. The Prix Galien award is especially gratifying for the scientists, physicians, patients and advocates involved in the discovery and development of Soliris," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "We are committed to making sure that every patient with PNH who can benefit from Soliris will have access to Soliris. We are building on the success of Soliris by increasing our understanding of PNH and by evaluating the promise of complement inhibition for the treatment of other rare and life-threatening kidney, blood, transplant and neurologic disorders."

About the Prix Galien USA Awards

The Prix Galien Award (http://www.prix-galien-usa.com) recognizes the technical, scientific and clinical research skills necessary to develop innovative medicines, and is considered the industry's highest accolade for pharmaceutical research and development -- equivalent to the Nobel Prize.

Prix Galien was first established in 1970 by French pharmacist Roland Mehl and was inaugurated in the United States in September 2007. The Prix Galien USA awards committee of 11 individuals includes seven Nobel Laureates, founders of major biotechnology companies and editors of world-renowned biology journals. The candidates for this year's awards were selected from drugs approved during the past five years.

About PNH

PNH is a rare blood disease that affects an estimated 8,000 to 10,000 people in North America and Europe and, using similar prevalence estimates, potentially 1,000 to 2,000 patients in Japan. (1) PNH often strikes people in the prime of their lives, with an average age of onset in the early 30's. (2) Approximately ten percent of all patients first develop symptoms at 21 years of age or younger. (3) PNH develops without warning and can occur in men and women of all races, backgrounds and ages. PNH often goes unrecognized, with delays in diagnosis often ranging from one to more than 10 years. (4) The estimated median survival for PNH patients is between 10 and 15 years from the time of diagnosis. (2,4)

PNH has been identified more commonly among patients with disorders of the bone marrow, including aplastic anemia (AA) and myelodysplastic syndrome (MDS). (5,6,7,8,9) In patients with thrombosis of unknown origin, PNH may be an underlying cause. (3,9)

Prior to approval of Soliris, there were no therapies specifically available for the treatment of PNH. PNH treatment was limited to symptom management through periodic blood transfusions, non-specific immunosuppressive therapy and, infrequently, bone marrow transplantations -- a procedure that carries considerable mortality risk. (3,9)

About Soliris

Soliris was approved in March 2007 by the U.S. Food and Drug Administration (FDA) as the first treatment for PNH, a rare, debilitating and life-threatening blood disorder defined by hemolysis. In June 2007, the European Commission (EC) also approved the use of Soliris for the treatment of patients with PNH. Soliris is the first drug therapy ever approved under

ALXN2020
IPR2023-01070

Priority Review in the U.S. and through the Accelerated Assessment procedure in Europe.

Important Safety Information

Soliris is generally well tolerated. The most frequent adverse events observed in clinical studies were headache, nasopharyngitis (a 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. 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 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 enrolled in the Soliris Safety 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.

Please see full prescribing information at www.soliris.net.

About Alexion

Alexion Pharmaceuticals, Inc. is a biopharmaceutical company working to develop and deliver life-changing drug therapies for patients with serious and life-threatening medical conditions. Alexion is engaged in the discovery, development and commercialization of therapeutic products aimed at treating patients with a wide array of severe disease states, including hematologic diseases, cancer, and autoimmune disorders. In March 2007, the FDA granted marketing approval for Alexion's first product, Soliris, for all patients with PNH and Alexion began commercial sale of Soliris in the U.S. during April 2007. In June 2007, the EC granted marketing approval for Soliris in the European Union for all patients with PNH. Alexion is evaluating other potential indications for Soliris as well as other formulations of eculizumab for additional clinical indications, and is pursuing development of other antibody product candidates in early stages of development. This press release and further information about Alexion Pharmaceuticals, Inc. can be found at: www.alexionpharm.com.

[ALXN-G]

(1) Hill A, Platts PJ, Smith A et al. The incidence and prevalence of paroxysmal nocturnal hemoglobinuria (PNH) and survival of patients in Yorkshire [abstract]. Blood. 2006;108(11):985.

(2) Socie G, Mary J Yves, de Gramont A, et al. Paroxysmal nocturnal haemoglobinuria: long-term follow-up and prognostic factors. Lancet 1996; 348:573-577.

(3) Parker C, Omine M, Richards S, et al. Diagnosis and management of paroxysmal nocturnal hemoglobinuria. Blood. 2005;106:3699-3709.

(4) Hillmen P. Lewis SM, Bessler M, Luzzatto L, Dacie JV. Natural history of paroxysmal nocturnal hemoglobinuria. N Engl J Med 1995; 333:1253-1258.

(5) Johnson RJ, Hillmen P. Paroxysmal nocturnal hemoglobinuria: Nature's gene therapy? Mol Pathol. 2002;55(3):145-52.

(6) Wang, et al. Clinical significance of a minor population of paroxysmal nocturnal hemoglobinuria-type cells in bone marrow failure syndrome. Blood 2002;100:3897-3902.

(7) Iwanga, et al. Paroxysmal nocturnal haemoglobinuria clones in patients with myelodysplastic syndromes. Brit J Haem. 1998; 102:465-474.

(8) Maciejewski, et al. Relationship between bone marrow failure syndromes and the presence of glycophosphatidyl inositol-anchored protein-deficient clones. Brit J Haem. 2001;115:1015-1022.

(9) Hill A, Richards S, Hillmen P. Recent developments in the understanding and management of paroxysmal nocturnal haemoglobinuria. Brit J Haem. 2007; 137:3, 181-192.

Please see full Prescribing Information and Important Safety Information for Soliris® (eculizumab).

Source URL: https://news.alexion.com/press-release/company-news/alexions-soliris-receives-2008-prix-galien-usa-award-best-biotechnology-p