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New Data Presented at ASH Annual Meeting Enhance Clinical Knowledge of aHUS and PNH and Underscore the Effectiveness of Soliris® (eculizumab) Treatment

- New Analyses Report Clinical Benefits of Ongoing Soliris Therapy in Children and Adults with aHUS With or Without Identified Genetic Mutations -

- Observational Data Highlight Importance of High Sensitivity Flow Cytometry to Enable Reliable Detection of PNH Cells -

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced that researchers presented data that continue to advance the understanding of atypical hemolytic uremic syndrome (aHUS) and paroxysmal nocturnal hemoglobinuria (PNH) and provide further insight into optimal care for patients with these devastating diseases. These data, which were presented at the 56th Annual Meeting of the American Society of Hematology (ASH) in San Francisco, include post-hoc analyses of Soliris® (eculizumab) treatment in adult and pediatric patients with aHUS with and without an identified genetic mutation, an update from the Global aHUS Registry, and interim results from an observational clinical study in Japan (OPTIMA) that highlights the importance of high sensitivity flow cytometry to enable the reliable detection of PNH cells in different patient groups.

Soliris, a first-in-class terminal complement inhibitor, is approved in nearly 50 countries as a treatment for patients with PNH, a debilitating, ultra-rare and life-threatening blood disorder characterized by complement-mediated hemolysis (destruction of red blood cells). Soliris is also approved in nearly 40 countries as a treatment for patients with aHUS, a genetic, chronic and ultra-rare disease associated with vital organ failure and premature death caused by permanent, uncontrolled activation of the complement system, resulting in systemic thrombotic microangiopathy (TMA).

"We are pleased that the data presented at ASH continue to expand our understanding of aHUS and PNH so that we can optimize care for patients with these life-threatening and ultra-rare disorders," said Leonard Bell, M.D., Chairman and Chief Executive Officer of Alexion. "Importantly, significant improvements in hematologic and renal outcomes were observed in pediatric and adult patients with aHUS, both with and without identified genetic mutations, supporting the early initiation of Soliris treatment regardless of mutation status."

Eculizumab is an Effective Treatment for Atypical Hemolytic Uremic Syndrome in Pediatric and Adult Patients with or without Identified Genetic Complement Mutations or Complement Factor H Autoantibodies (Abstract 2789)

Spero R. Cataland M.D., of Ohio State University Medical Center, presented results from two post-hoc sub-analyses from two open-label single-arm trials of Soliris in pediatric (Study C10-003, N=22) and adult (Study C10-004, N=41) patients with aHUS. The 26-week analyses evaluated the safety and efficacy of Soliris in patients with aHUS with or without identified genetic mutations at baseline. Dr. Cataland reported that platelet count normalization was achieved by 100% (11/11) of pediatric and 100% (21/21) of adult patients with an identified mutation and by 91% (10/11) of pediatric and 95% (19/20) of adult patients without an identified mutation. Mean change from baseline in platelet count was 172 x 10^9/L for pediatric and 101 x 10^9/L for adult patients with an identified mutation and 154 x 10^9/L for pediatric and 179 x 10^9/L for adult patients without an identified mutation. Dr. Cataland also reported mean eGFR improvement from baseline of +71 mL/min/1.73 m^2 for pediatric and +31 mL/min/1.73 m^2 for adult patients with an identified mutation, and +57 mL/min/1.73 m^2 for pediatric and +27.0 mL/min/1.73 m^2 for adult patients without an identified mutation. For patients on dialysis at baseline, 100% (5/5) of pediatric and 78.6% (11/14) of adult patients with an identified mutation discontinued dialysis by 26 weeks, as did 66.7% (4/6) of pediatric and 90% (9/10) of adult patients without an identified mutation. Researchers concluded that Soliris treatment resulted in clinically meaningful improvements in hematologic and renal parameters in adult and pediatric patients with aHUS regardless of the presence of an identified genetic mutation.[1]

There were no unexpected safety signals reported during the analysis period for either study. No meningococcal infections were reported in the C10-003 study. Two patients in the C10-004 study had meningococcal infections during the 26-week study period. One patient discontinued from the study and later recovered; the other continued treatment with no interruption and recovered without sequelae. The most common AEs reported by subgroup in C10-003 were: for patients with an identified mutation, abdominal pain (36.4%), pyrexia (36.4%), and upper respiratory tract infection (36.4%); for patients without an identified mutation, pyrexia (63.3%), vomiting (63.6%), and cough (45.5%). The most common AEs reported by subgroup in C10-004 were: for patients with an identified mutation, diarrhea (47.6%), headache (38.1%), and peripheral edema (33.3%); for patients without an identified mutation, headache (35.0%), pyrexia (25.0%), diarrhea (20.0%), hypotension (20.0%), renal impairment (20.0%), and urinary tract infection (20.0%).

"Given the life-threatening nature of aHUS and the well-established clinical efficacy of Soliris, these data provide additional evidence for initiating treatment with Soliris immediately upon clinical diagnosis of aHUS. This is particularly important since genetic testing can take several months to complete and, to date, genetic complement mutations can only be identified in 50%-70% of patients with aHUS," said Dr. Cataland.

Baseline Demographics and Characteristics of 532 Patients with Atypical Hemolytic Uremic Syndrome in the Global aHUS Registry (Abstract 4204)

Christoph Licht, M.D., FASN, of The Hospital for Sick Children, Toronto, presented baseline demographics from patients enrolled in the global aHUS Registry, which is dedicated to increasing the awareness and understanding of aHUS to help optimize care and improve quality of life for patients. As of September 30, 2014, 532 patients had enrolled in the registry.

Patients in the global aHUS Registry had a mean age of 26.5 years at Registry enrollment. Of the 532 patients enrolled, 320 were ≥18 years old at Registry enrollment, 60 were between ≥12 to < 18 years, 81 were between ≥5 to < 12 years, 35 were between ≥2 to < 5 years and 36 patients were < 2 years old at Registry enrollment. In terms of baseline clinical characteristics, 104 patients (19.5%) had a prior kidney transplant, 310 patients (58.3%) had prior dialysis, 317 patients (59.6%) had prior plasma exchange/infusion, and mean baseline eGFR of patients was 50.6 (N=185).[2]

The Interim Analysis of the OPTIMA (Observation of GPI-Anchored Protein-Deficient [PNH-type] Cells in Japanese Patients with Bone Marrow Failure Syndrome and in those Suspected of Having PNH) Study (Abstract 1595)

Hideyoshi Noji, M.D., Ph.D., of Fukushima Medical University, Japan, presented interim data from a multi-center observational study (OPTIMA) to determine the prevalence and clinical significance of PNH-type cells in patients with various bone marrow failure (BMF) syndromes and those with suspected PNH. By implementing a uniform flow-cytometry protocol in six laboratories across the country, investigators enabled reliable detection of PNH-type cells through high-sensitivity flow cytometry.

Out of 1,739 samples examined, 607 (34.9%) were positive for PNH cells with 172 (9.9%) ≥1% PNH cells. Of these 607 patients, LDH levels ≥1.5 x upper limits of normal, which is a risk factor for serious complications, were seen in 37% of patients with 1%-10% PNH cells and in 97% of patients with ≥10% PNH cells. Researchers concluded that high-resolution flow cytometry is a helpful diagnostic tool in BMF syndromes and useful in understanding the pathophysiology of these disorders.[3]

About aHUS

aHUS is a chronic, ultra-rare, and life-threatening disease in which a life-long and permanent genetic deficiency in one or more complement regulatory genes causes chronic, uncontrolled complement activation, resulting in complement-mediated thrombotic microangiopathy (TMA), the formation of blood clots in small blood vessels throughout the body.[4,5] Permanent, uncontrolled complement activation in aHUS causes a life-long risk for TMA, which leads to sudden, catastrophic, and life-threatening damage to the kidney, brain, heart, and other vital organs, and premature death.[4,6] Seventy-nine percent of all patients with aHUS die, require kidney dialysis or have permanent kidney damage within three years after diagnosis despite plasma exchange or plasma infusion (PE/PI).[7] Moreover, 33 to 40 percent of patients die or progress to end-stage renal disease with the first clinical manifestation of aHUS despite PE/PI.[7,8] The majority of patients with aHUS who receive a kidney transplant commonly experience subsequent systemic TMA, resulting in a 90 percent transplant failure rate in these TMA patients.[9]

aHUS affects both children and adults. Complement-mediated TMA also causes reduction in platelet count (thrombocytopenia) and red blood cell destruction (hemolysis). While mutations have been identified in at least ten different complement regulatory genes, mutations are not identified in 30-50 percent of patients with a confirmed diagnosis of aHUS.[7]

About PNH

PNH is an ultra-rare blood disorder in which chronic, uncontrolled activation of complement, a component of the normal immune system, results in hemolysis (destruction of the patient's red blood cells). PNH strikes people of all ages, with an average age of onset in the early 30s.[10] Approximately 10% of all patients first develop symptoms at 21 years of age or younger.[11] 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 ranging from one to more than 10 years.[12] In the period of time before Soliris was available, it had been estimated that approximately one-third of patients with PNH did not survive more than five years from the time of diagnosis.[10] PNH has been identified more commonly among patients with disorders of the bone marrow, including aplastic anemia (AA) and myelodysplastic syndromes (MDS).[13,14,15] In patients with thrombosis of unknown origin, PNH may be an underlying cause.[10]

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) to reduce hemolysis. PNH is a debilitating, ultra-rare and life-threatening blood disorder, characterized by complement-mediated hemolysis (destruction of red blood cells). 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) to inhibit complement-mediated thrombotic microangiopathy, or TMA (blood clots in small vessels). aHUS is a debilitating, ultra-rare and life-threatening genetic disorder characterized by complement-mediated TMA. Soliris is not indicated for the treatment of patients with Shiga-toxin *E. coli*-related hemolytic uremic syndrome (STEC-HUS). For the breakthrough medical innovation in complement inhibition, Alexion and Soliris have received some of the pharmaceutical industry's highest honors: the Prix Galien USA (2008, Best Biotechnology Product) and France (2009, Rare Disease Treatment).

More information including the full U.S. 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 [see Warnings and Precautions (5.1)]. 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 Warnings and Precautions (5.1) for additional guidance on the management of the risk 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 [see Warnings and Precautions (5.2)]. 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 headache, diarrhea, hypertension, upper respiratory infection, abdominal pain, vomiting, nasopharyngitis, anemia, cough, peripheral edema, nausea, urinary tract infections, and pyrexia. Soliris is not indicated for the treatment of patients with Shiga-toxin *E. coli*-related hemolytic uremic syndrome (STEC-HUS). 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 nearly 40 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, including asfotase alfa, 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 anticipated clinical development, regulatory and commercial milestones and potential health and medical benefits of Soliris® (eculizumab) for the potential treatment of patients with PNH and 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-Q for the period ended September 30, 2014, 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.

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