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New Data Presented at ASH Annual Meeting Enhance Understanding of PNH and aHUS to Provide Optimal Care for Patients with These Life-threatening Disorders

— New Biomarker Data Support the Need for Chronic Terminal Complement Blockade with Soliris® (eculizumab) in Patients with aHUS — —

— Data from the Largest Prospective Trial in Expanded Population of Adults with aHUS and First Prospective Trial in Pediatric Patients with aHUS Now Presented to Hematology Community— —

— New Registry Data Highlight Similar Disease Severity in PNH Patients With or Without a History of Transfusion —

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) today announced that researchers presented data from clinical trials demonstrating the clinical benefits of chronic Soliris® (eculizumab) treatment in patients with atypical hemolytic uremic syndrome (aHUS) as well as new patient registry data providing further insight into optimal care for patients with paroxysmal nocturnal hemoglobinuria (PNH) at the American Society of Hematology (ASH) 55th Annual Meeting and Exposition in New Orleans. Data included:

- A new, comprehensive study of key aHUS biomarkers indicating that, prior to initiation of Soliris treatment, aHUS patients have severe ongoing terminal complement activation and inflammation with increased thrombotic risk. The study further indicates that inhibition of terminal complement activation with Soliris should be sustained because loss of terminal complement inhibition could lead to potentially catastrophic clinical consequences.[1]
- Results from the first prospective clinical trial in pediatric patients with aHUS, which confirm previous results from a retrospective pediatric study, and support the use of Soliris as first-line treatment for children with aHUS.[2]
- Safety and efficacy data from the largest prospective aHUS trial which extends results from earlier studies to a broader adult patient population, and support the recent guidelines recommending immediate treatment with Soliris once a diagnosis has been made.[3]
- A prospective PNH registry in Korea showing that non-transfused patients with PNH, similar to patients with a history of transfusion, have hemolysis and clinical symptoms that lead to life- threatening consequences including thrombosis and chronic kidney disease (CKD).[4]
- An observational clinical study (OPTIMA) in Japan that establishes the importance of high sensitivity flow cytometry to enable the reliable detection of PNH cells in different patient groups.[5]
- Findings from a Spanish cohort of the international PNH registry that show the importance of regularly monitoring clone size in patients with PNH to detect possible expansion of pre-existing PNH clones.[6]

Soliris, a first-in-class terminal complement inhibitor, specifically targets uncontrolled complement activation. It is the first and only approved 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 approved in nearly 50 countries for the treatment of PNH, including the United States, European Union and Japan. Soliris is also approved in the United States, European Union, Japan and other countries as the first and only treatment for pediatric and adult patients with aHUS, a genetic, chronic, ultra-rare disease associated with vital organ failure and premature death.

"Extensive clinical data presented at ASH demonstrate a significant and sustained inhibition of complement-mediated TMA with Soliris treatment and support the chronic use of Soliris in pediatric and adult patients with aHUS. New biomarker data also reinforce the need for chronic terminal complement inhibition in patients with aHUS and suggest that a loss of terminal complement inhibition could lead to a marked increase in thrombotic risk with the potential for sudden and catastrophic damage to vital organs," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "Additionally, new findings from the largest PNH registry highlight the importance of understanding risk factors and ongoing monitoring of PNH patients to achieve the best possible outcomes. We are pleased that new data continue to enhance the understanding of PNH and aHUS so that we can optimize care for patients with these life-threatening and ultra-rare disorders."

Biomarkers in Patients with aHUS Treated with Soliris (Abstract 2184)[1]

In a poster session today, researchers presented biomarker data from a prospective open-label trial of adult patients with aHUS treated with Soliris. The data show that at baseline, prior to Soliris treatment initiation, patients with aHUS have significantly elevated levels of all biological measures of complement-mediated TMA compared to normal healthy volunteers. Prior to Soliris treatment, in all aHUS patient groups-- including those receiving PE/PI or those with normal platelets, haptoglobin (Hp) or lactate dehydrogenase (LDH) levels--patients demonstrated significant elevation in terminal complement activation that was 45-305 fold higher than levels in normal healthy volunteers. In addition, patients with aHUS had significant elevation in measures of vascular inflammation, endothelial activation and damage, thrombotic risk, vital organ damage and proximal complement alternative pathway (CAP) activation.

Sustained Soliris treatment significantly reduced and normalized highly elevated measures of terminal complement activation, and resulted in:

- Significant and sustained reduction in measures of vascular inflammation by up to 94%
- Significant inhibition in measures of endothelial activation by up to 60%
- Significant and sustained reduction in measures of endothelial damage by up to 77% to near normal levels, demonstrating a strong relationship between terminal complement activation and endothelial damage
- Significant and marked reduction by up to 99% in measures of thrombotic risk

Sustained inhibition of terminal complement activation with Soliris dramatically reduced and normalized measures of vital organ damage, and also significantly reduced measures of proximal CAP activation. The study authors concluded that a loss of terminal complement inhibition in aHUS would be expected to lead to an increase in underlying subclinical endothelial activation, significant acceleration of endothelial damage, marked increase in thrombotic risk, and an early, ongoing risk of catastrophic vascular ischemia and vital organ damage. Further, in clinical trials of patients with aHUS, severe TMA complications have been observed in patients following a missed dose of Soliris.[7]

"The biomarker data in this study suggest that chronic terminal complement blockade with Soliris is necessary to protect patients from the potentially catastrophic consequences of aHUS, including vital organ failure and premature death," said Camille L. Bedrosian, M.D., Chief Medical Officer of Alexion Pharmaceuticals. "These data point to the chronic nature of aHUS and the need for continuous treatment in order to achieve optimal outcomes for patients with this genetic and life-long disease."

Soliris in Pediatric and Adult Patients with aHUS

- In a poster presentation (Abstract 2191)[2], researchers presented positive results from the first prospective trial of Soliris in pediatric patients with aHUS. This open-label, prospective, single-arm, multinational trial enrolled a heterogeneous population of patients who were > 1 month old and < 18 years of age. It included 22 pediatric patients with aHUS, of whom 16 (73%) were newly diagnosed. Prior plasma exchange or infusion (PE/PI) was not required for inclusion in the study. Most patients enrolled in the study (12/22, 55%) received Soliris as their first aHUS-specific treatment and had not received PE/PI prior to Soliris therapy.

  In the study, 19 patients (86%) completed the initial 26 weeks of Soliris therapy, and 14 of 22 patients (64%) achieved the study's primary endpoint of complete TMA response at 26 weeks, which required significant improvement in renal function (≥25% decrease in creatinine). Platelet count normalization was achieved in 21 of the 22 patients (95%); the median time to platelet count normalization was seven days and the mean improvement in platelet count from baseline was 164 $x10^9$/L ($p < 0.0001$). In terms of renal parameters, the mean estimated glomerular filtration rate (eGFR) increase from baseline was 64 mL/min/1.73m^2 ($P < 0.001$) and 19 patients (86%) achieved an improvement in eGFR from baseline of at least 15 mL/min/1.73m^2 at 26 weeks. By Week 26, 16 patients (73%) had experienced at least a 25% decrease from baseline in serum creatinine. Importantly, 9 of 11 patients (82%) who were on dialysis at baseline discontinued dialysis for the duration of the study and all 12 patients who were not on dialysis at baseline continued dialysis-free through 26 weeks. Results from this study were also presented at the 2013 American Society of Nephrology (ASN) meeting.[8]

  "This trial was the first prospective study of pediatric patients with aHUS, and demonstrated that chronic Soliris treatment led to rapid and sustained improvement in platelet counts and significant improvement in kidney function, including discontinuation of dialysis," said Larry Greenbaum, M.D., Ph.D., Director of Pediatric Nephrology at Emory University and Children's Healthcare of Atlanta.[9] "The safety and efficacy demonstrated in this prospective trial confirm the results observed in the previous retrospective pediatric study as well as the published Phase 2 prospective adult trials, and support the recommendation of Soliris as a first-line treatment in children with aHUS."

  Soliris was generally well tolerated in the study. The most common adverse events (AEs) were fever (50%) and cough (36%). One patient had a human anti-human antibody response, and continued chronic Soliris treatment without

apparent adverse effect.[2]

- In a separate poster presentation (Abstract 2179)[3], researchers presented positive data from the largest prospective trial of Soliris in adult patients with aHUS. This open-label, single-arm, multinational trial enrolled 41 adult patients with aHUS representing a broad patient population. Prior PE/PI was not required for inclusion in the study, and patients had a short duration from the onset of aHUS symptoms to the first dose of Soliris treatment (median of 2 weeks). Thirty of 41 patients (73%) in the study were newly diagnosed, six patients (15%) had no PE/PI during the current clinical manifestation, 24 patients (59%) were on dialysis at baseline, nine patients (22%) had a prior kidney transplant, and 21 patients (51%) had no identified genetic mutation.

  The study met its primary endpoint, with 30 of 41 patients (73%) achieving a complete TMA response at 26 weeks, as measured by platelet count normalization, LDH normalization and preservation of renal function (< 25% increase in serum creatinine from baseline. Soliris significantly improved renal function with a mean increase in eGFR from baseline of 29 mL/min/1.73m^2 ($P < 0.0001$). Most importantly, of the 24 patients who were on dialysis at baseline, 20 patients (83%) discontinued dialysis by Week 26. Results from this study were also presented at the 2013 ASN meeting.[8]

  "This trial is the largest study in aHUS and extends the results from earlier prospective trials, in which ongoing Soliris treatment led to sustained inhibition of complement-mediated TMA, rapid and sustained improvements in hematological parameters, and continued, on-going improvement in renal function in adult patients with aHUS," said Fadi Fakhouri, M.D., Ph.D., Centre Hospitalier Universitaire de Nantes, Nantes, France.[10] "Due to high morbidities and premature mortality in aHUS despite PE/PI, results from this study also support the recent guidelines recommending immediate treatment with Soliris in adults with aHUS once an unequivocal diagnosis has been made."

  Soliris was generally well tolerated in the study. The most common AEs were headache (37%), diarrhea (32%) and peripheral edema (22%). There were two cases of meningococcal infections; both patients recovered, with one patient continuing on Soliris therapy and one discontinuing therapy with subsequent deterioration of renal function that necessitated dialysis support. There were no deaths in the study.[3]

- Separately, researchers presented data on hematologic and renal improvements in patients with aHUS and long disease duration and chronic kidney disease previously managed with PE/PI who were treated with Soliris (Abstract 2186).[11] In the study, patients had immediate improvements in hematologic values with Soliris treatment, followed by time-dependent improvements in renal function. The analysis showed that the majority of patients demonstrated improvements in platelet count and LDH at 1 month, with 90 and 95% of patients, respectively, meeting the normalization threshold at 3 months. Improvements in renal function began later than improvements in hematologic outcomes and increased gradually throughout the study period. By the end of the 2-year study period, the majority of patients met the thresholds for creatinine (≥25%) and CKD (≥1 stage) improvement. The authors concluded that these data demonstrate that improvement in renal function may be achieved over time in patients with long-standing aHUS and pre-existing CKD, and underscore the importance of ongoing and consistent treatment with Soliris. Throughout the study period, Soliris was well-tolerated; one patient died of complications that were deemed unrelated to Soliris.

- In another poster presentation (Abstract 3426)[12], researchers presented data on the time to hematologic and renal improvements in aHUS patients with progressing thrombotic microangiopathy (TMA) treated with Soliris for two years. In the study, statistically significant mean improvements from baseline were achieved as early as 1 week for platelet count, and by 2 weeks for eGFR. Significant improvements from baseline were observed by 8 weeks for hemoglobin and haptoglobin. All improvements were sustained through the end of the study period. Achievement of criteria for hematologic normalization (platelet count and hematologic normalization) and renal (eGFR increase ≥15 mL/min/1.73 m^2, and decrease of ≥25% in serum creatinine) parameters began at Weeks 4 and 6, respectively. Soliris was well-tolerated over the study period. The authors concluded that these data highlight that importance of early treatment in aHUS patients with progressing TMA.

Data in Patients with PNH

The ASH meeting also featured the following data presentations in patients with PNH:

- In a poster presentation (Abstract 3720)[4], researchers presented data from a prospective Korean PNH registry. Analyses of the 106 Korean patients with PNH demonstrated that, similar to transfused patients, non-transfused patients also suffered from elevated hemolysis, debilitating clinical symptoms of PNH, CKD, and life-threatening complications, such as thromboembolism (TE).

  "These data indicate that patients with or without transfusion history during the 12 months prior to study enrollment are at similar risk for severe clinical outcomes including thrombosis and chronic kidney disease, the two leading causes of mortality in PNH," said Jong Wook Lee, M.D., Ph.D., Professor of Medicine, St. Mary's Hospital, The Catholic University,

Seoul, South Korea and lead author of the poster.[13] "To ensure the best outcomes for patients with PNH, treatment management should be based on presenting risk factors, regardless of transfusion history."

- In another poster (Abstract 1241)[5], interim data were reported from a multi-center observational study (OPTIMA). This study was conducted to determine the prevalence of PNH cells in Japanese patients with various bone marrow failure syndromes and those with suspected PNH. By implementing a uniform flow-cytometry protocol in six laboratories across the country, investigators established high-sensitivity flow cytometry in each laboratory to enable reliable detection of PNH cells. Out of 1451 samples examined, 38% were positive for PNH cells with 12% ≥1% PNH cells and LDH levels exceeding the > 1.5 x upper limits of normal in 68% of patients with ≥1% PNH cells.
- In a separate poster presentation (Abstract 3715)[6], data from the Spanish cohort of the International PNH Registry suggest that periodic evaluation of clone size is recommended in PNH. One hundred seventeen (117) Spanish patients were categorized into three groups according to physician-reported PNH type: (1) hemolytic PNH, (2) PNH with another bone marrow disorder, and (3) subclinical PNH. Median clone sizes for groups 1 and 2 increased between diagnosis and enrollment. Further, median LDH activities, considered to be a possible indicator for thrombosis risk, were highest in group 1 at diagnosis. A high prevalence of TEs was also shown in this patient cohort (almost 1 in 4 patients had a TE).

About aHUS

aHUS is a chronic, ultra-rare, and life-threatening disease in which a 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.[14,15] 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.[14,16] Complement-mediated TMA also causes reduction in platelet count (thrombocytopenia) and red blood cell destruction (hemolysis).

aHUS affects both children and adults. Sixty-five percent of all patients with aHUS die, require kidney dialysis, or have permanent kidney damage within the first year after diagnosis despite supportive care with plasma exchange or plasma infusion (PE/PI).[17,18] The majority of patients with aHUS who receive a kidney transplant experience subsequent systemic TMA, resulting in a 90% transplant failure rate in these TMA patients.[19] While mutations have been identified in at least ten different complement regulatory genes, mutations are not identified in 30-50% of patients with a confirmed diagnosis of aHUS.[20]

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.[21] Approximately 10% of all patients first develop symptoms at 21 years of age or younger.[22] 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.[23] 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.[21] PNH has been identified more commonly among patients with disorders of the bone marrow, including aplastic anemia (AA) and myelodysplastic syndromes (MDS).[24,25,26] In patients with thrombosis of unknown origin, PNH may be an underlying cause.[21]

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 US (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 US (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 the effects on TMA and renal function. Prospective clinical trials in additional patients, the preliminary results of which are reported here at ASH 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 U.S. prescribing information on Soliris is available at www.soliris.net. The full prescribing information for Soliris in Europe, is available at: http://www.ema.europa.eu/ema/index.jsp?curl=pages/medicines/human/medicines/000791/human_med_001055.jsp&mid=WC0b01ac058001d124.

Important Safety Information

The US 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 2 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 Pharmaceuticals, Inc. is a biopharmaceutical company focused on serving patients with severe and ultra-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 Pharmaceuticals, Inc. 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, 2013, 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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