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New Data Highlighting Long-Term Efficacy and Safety Outcomes of PNH Patients Treated with Soliris® Reported at ASH Annual Meeting

— Large UK PNH Study Shows Significant Clinical Benefits Sustained Over 10 Years —

— Two-year Data Demonstrating Long-Term Benefits of Chronic Soliris Therapy in Patients with aHUS also Presented —

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) today announced the presentation of new data demonstrating the long-term benefits of Soliris® (eculizumab) in 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). Data including the clinical benefits of Soliris in the treatment of patients with PNH and atypical hemolytic uremic syndrome (aHUS) were presented at the 54th Annual Meeting of the American Society of Hematology (ASH) in Atlanta:

- Ten-year data from a large cohort of patients with PNH from the United Kingdom (UK) confirm the long-term safety and efficacy of chronic Soliris therapy and demonstrate the impact of Soliris on quality of life.[1]
- The long-term safety of Soliris was also demonstrated in PNH clinical development trials in which patients were treated continuously with Soliris and followed for up to 5.5 years.[2]
- Data from an international PNH registry showed that Soliris significantly reduced the risk of thromboembolism (TE) in patients with PNH.[3]
- Researchers from South Korea presented data confirming that hemolysis is an independent risk factor for TE in patients with PNH.[4]
- Researchers in Japan observed a rare genetic polymorphism in the terminal complement protein C5 in Japanese patients with PNH who had no or minimal reduction in lactate dehydrogenase (LDH) while on Soliris therapy (9 out of 250 treated, or 3.6%).[5]
- The ASH meeting also featured presentations of two-year data highlighting the long-term benefits of chronic Soliris therapy in patients with aHUS,[6,7] an ultra-rare, genetic disease characterized by complement-mediated thrombotic microangiopathy (TMA), the formation of blood clots in small vessels throughout the body.

Soliris, a first-in-class terminal complement inhibitor, specifically targets uncontrolled complement activation, and is approved in the United States, European Union, Japan and other countries as the first and only treatment for patients with PNH. Soliris is also approved in the US and EU as the first and only treatment for patients with aHUS.

"Extensive clinical data and real-world clinical experience spanning more than 10 years further establish the benefits that Soliris is providing to patients with PNH," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "The presentation of the two-year aHUS data also demonstrates the continued benefits of long-term Soliris therapy in patients with aHUS, and underscores the important role of hematologists in diagnosing and treating patients with this severe and ultra-rare complement-mediated disease."

Ten-Year Experience with Soliris in All Patients with PNH Treated in the UK

In a poster presentation today, researchers presented data from all 153 patients who participated in a nationally commissioned PNH service led by two UK medical centers. The objective of the study was to describe the long-term safety, efficacy, and outcomes in patients with PNH from the UK who received Soliris treatment from May 2002 to April 2012.[1]

The results demonstrated that the significant clinical benefits and long-term safety of Soliris were sustained over 10 years of chronic treatment. The investigators reported that long-term Soliris treatment led to an improvement in survival when compared with historical controls, and a significant reduction in the incidence of TE.[8,9] Researchers also reported data on the effect of discontinuation of anti-coagulant therapy in selected PNH patients. Transfusion independence was observed in the majority of patients, and the number of units transfused was significantly reduced in those patients still receiving transfusions. Results also confirmed the long-term safety and efficacy of continuous Soliris treatment.[1]

Researchers presented the following data in support of these conclusions:[1]

- Long-term Soliris therapy significantly reduced intravascular hemolysis by 83.4%, as assessed by levels of LDH ($p < 0.001$).
- The researchers noted that UK patients on long-term Soliris therapy had improved survival compared with previously published historical controls.[8,9] Researchers also compared the survival of PNH patients treated with Soliris to a normal population of the same age and gender. Although the survival of PNH patients after 10 years of Soliris therapy was slightly inferior to this normal healthy population group, the causes of death in PNH patients were related to bone marrow failure and not due to hemolysis or TE associated with the underlying PNH.
- In the 12 months prior to starting Soliris, 36 thrombotic episodes were reported in 22 patients. None of those patients had a further thrombotic episode while on Soliris therapy. In the most recent 12 months on therapy, TEs were significantly reduced with only 3 events reported in a total of 3 patients ($p < 0.05$).
- Of 117 patients transfused in the 12 months before receiving Soliris, 77 (65.8%) were transfusion-independent in the most recent 12 months on treatment. Among those patients still receiving transfusions (n=40), there was a significant 69% reduction in the number of units transfused, from a median of 26 units, 12 months before therapy, to 8 units in the most recent 12 months on therapy ($P < 0.05$).

"More than 10 years of experience in the UK show the continuing profound positive impact of Soliris on the lives of patients living with PNH. With Soliris therapy, PNH-related morbidities are significantly reduced, and in addition to this positive efficacy, there is continuing safety as well as highly significant improvement in symptoms and quality of life," said Anita Hill[10], M.D. Ph.D., lead author of the poster and Consultant Haematologist at the Leeds Teaching Hospitals, Leeds, UK.

Long-Term Safety of Sustained Soliris Treatment in Patients with PNH

In a poster presentation on December 8, researchers presented results of a long-term safety analysis in 195 patients receiving continuous Soliris treatment (mean duration: 30.3 months) in the Soliris PNH clinical development trials and associated extension studies. They compared the reported incidence of adverse events (AEs), irrespective of relation to treatment, during the first 26 weeks of Soliris treatment with the reported incidence of AEs in the last 26 weeks.[2]

The study demonstrated the long-term safety of Soliris in PNH patients treated for up to 5.5 years, with no evidence of cumulative toxicity. Further, the incidence of patients reporting an AE in the last 26 weeks of treatment was significantly reduced compared with the incidence in the first 26 weeks ($p < 0.001$). Additionally, the probability of a patient experiencing an AE decreased significantly with time ($p < 0.001$). There was also a low rate of meningococcal infection with long-term Soliris treatment (0.422 per 100 patient-years). Two patients experienced meningococcal infections from strains not covered by their vaccinations. Both infections were successfully treated and both resolved without sequelae. Four patient deaths were reported over the course of the study; none were considered by the investigators to be related to Soliris treatment.[2]

"Our data give us even greater confidence in the long-term safety of Soliris in patients with PNH," stated lead investigator Jeffrey Szer[11], M.D., Professor/Director of the Department of Clinical Haematology at Royal Melbourne Hospital in Melbourne, Australia. "The adverse event rate among patients on continuous Soliris therapy suggests a favorable risk-to-benefit ratio over the long term."

Reduced Risk of Thromboembolism (TE): An International PNH Registry Study

In a separate poster presentation today, researchers reported that Soliris is associated with a reduced risk of TE and mortality, based on findings from 1047 patients enrolled in an international PNH registry. Over a mean follow-up period of 22.5 months, patients receiving Soliris treatment had a cumulative incidence of TE of 0.41% at one year and 1.35% at two years. Among patients not taking Soliris, the corresponding one- and two-year incidences of TE were 1.70% and 2.61%, respectively. The cumulative incidence of mortality in Soliris-treated patients was 2.31% and 4.21% at one and two years, respectively, while in untreated patients it was 4.40% and 7.01%, respectively. The study authors noted that results of these analyses and the conclusions that can be drawn from them are limited due to small number of TE and mortality outcomes.[3]

"The International PNH Registry provides real-world data that are consistent with prior research which indicated a reduced risk of thromboembolism and mortality in patients treated with Soliris," said Gerard Socié[12], M.D., Ph.D., lead author of the poster and Professor of Haematology at Hospital Saint Louis, APHP, University Paris VII Denis Diderot, Paris, France.

Other PNH Data Presentations

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

- In a poster presentation on December 8, researchers in South Korea evaluated the risk of TE in patients with PNH and elevated hemolysis (as identified by LDH ≥1.5 x ULN) in addition to any of the four clinical symptoms of abdominal pain, chest pain, dyspnea, or hemoglobinuria, compared with patients who had elevated LDH alone. The analyses confirm that elevated hemolysis was associated with a seven-fold increased risk of TE in patients with PNH. The risk of TE was significantly further increased in patients with elevated hemolysis and additional symptoms of abdominal pain, chest pain, dyspnea or hemoglobinuria compared with patients without the symptom and LDH < 1.5 x ULN. These results underscore the importance of early therapeutic intervention and monitoring of PNH patients with elevated LDH.[4]
- In a separate poster presentation on December 8, data supported the need to test high-risk PNH patients for diagnostic markers as an aid to treatment selection. Researchers presented an updated analysis of 7,699 high-risk patients whose blood cells were screened for a PNH clone using high-sensitivity flow cytometry. Among the 481 PNH-positive patients, those with large PNH clone sizes (> 20%) were found to be more likely to have clinical symptoms, particularly those associated with hemolysis, and were thus deemed more likely to benefit from therapy.[13]
- A poster presentation today identified a polymorphism in the terminal complement protein C5 that appears to be associated with no or minimal reduction in LDH in nine Japanese patients with PNH who received Soliris therapy, although there is no evidence of the mutation outside of Japan or in any other Asian populations. The study authors concluded that further research is needed to verify that the polymorphism in the C5 gene is responsible for the suboptimal reduction in LDH and to determine a more accurate prevalence of this C5 polymorphism in Japanese populations.[5]

Soliris in Patients with aHUS

During the ASH annual meeting, researchers presented two-year data that highlight the long-term benefits of chronic Soliris therapy in patients with aHUS. As presented last month at the annual meeting of the American Society of Nephrology (ASN)[14], data from two pivotal phase 2 studies demonstrated that ongoing Soliris treatment for two years was associated with sustained ongoing inhibition of complement-mediated TMA, as indicated by a maintained increase in platelet count, and sustained improvement in renal function and TMA event-free status.[6,7]

In a poster presented on December 8, researchers presented two-year findings from a prospective, open-label, single-arm phase 2 trial of Soliris in adult and adolescent patients with a long duration of aHUS and chronic kidney damage who were undergoing prolonged plasma exchange or plasma infusion (PE/PI) before starting treatment with Soliris. Patients had been diagnosed with aHUS a median of 48 months prior to starting the study. Twenty patients were enrolled in the initial study and received Soliris for 26 weeks. Nineteen of the 20 patients continued into a long-term extension phase. Patients were evaluated for a median duration of 114 weeks.[6]

The study achieved its primary endpoint, TMA event-free status (at least 12 consecutive weeks of stable platelet count, no PE/PI, and no new dialysis), in 16 of 20 (80%) patients through 26 weeks. TMA event-free status was achieved and maintained by 95% of patients through two years, indicating that chronic treatment with Soliris continued to significantly inhibit complement-mediated TMA. Patients achieved and maintained TMA event-free status regardless of the identification of a genetic complement mutation. Importantly, no patient required new dialysis and only one patient required any PE/PI through data cutoff.[6]

In patients treated with Soliris over two years, researchers observed continued improvement in renal function, including a sustained improvement in estimated glomerular filtration rate (eGFR), with a mean change from baseline of 6.1 mL/min/1.73m^2 through 26 weeks (p=0.0001) and 7.2 mL/min/1.73m^2 through two years (p < 0.05). Earlier intervention with Soliris therapy was associated with greater increases in eGFR (p=0.001). Seven of 20 patients (35%) experienced chronic kidney disease (CKD) improvement of at least one stage by 26 weeks, compared with 12 of 20 patients (60%) by two years.[6]

In a poster presented on December 9, researchers presented two-year follow-up data from a prospective, open-label, single-arm phase 2 study in 17 adult and adolescent patients with aHUS who had presented with progressive clinical TMA complications despite intensive PE/PI. Patients had been diagnosed with aHUS for a median of 10 months before the start of the study. Seventeen patients were enrolled in the initial study and received Soliris for 26 weeks. Seventeen patients continued into a long-term extension phase. Patients were evaluated for a median duration of 100 weeks.[7]

The study achieved its primary endpoint, as mean platelet count improved from baseline at 26 weeks (p=0.0001). Additionally, the improved platelet count continued over two years (p < 0.001), indicating sustained inhibition of complement-mediated TMA with ongoing eculizumab treatment. Platelet normalization (≥150x10^9/L) was achieved in 13 of 15 patients (87%) who had low platelets at baseline by 26 weeks and was maintained through two years for 12 of the 13 patients. TMA event-free status was also achieved rapidly and maintained through two years, with 15 of 17 (88%) Soliris-treated patients achieving TMA event-free status through each data cut-off point (26 weeks, one year, and two years), and TMA event-free status was achieved regardless of the identification of a genetic complement mutation.[7]

"These longer-term data demonstrate the sustained benefits of Soliris therapy in patients with aHUS and reveal a significant and continued improvement in renal function over time," commented Larry Greenbaum[15], M.D., Ph.D., Director of Pediatric Nephrology at Emory University and Children's Healthcare of Atlanta, who presented the aHUS poster on December 9. "Further, the data support that earlier treatment leads to even better outcomes for patients with aHUS."

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.[16] Approximately 10% of all patients first develop symptoms at 21 years of age or younger.[17] 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.[8] 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.[16] PNH has been identified more commonly among patients with disorders of the bone marrow, including aplastic anemia (AA) and myelodysplastic syndromes (MDS).[18-20] In patients with thrombosis of unknown origin, PNH may be an underlying cause.[16]

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.[21,22] 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.[21,23] Sixty-five percent of all patients with aHUS require kidney dialysis, have permanent kidney damage or die within the first year after diagnosis despite plasma exchange or plasma infusion (PE/PI).[24,25] The majority of patients with aHUS who receive a kidney transplant commonly experience subsequent systemic TMA, resulting in a 90% transplant failure rate in these TMA patients.[26]

aHUS affects both children and adults.[27] 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% of patients with a confirmed diagnosis of aHUS.[27]

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, European Union, Japan 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 and the European Union 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 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).

Alexion's breakthrough approach in complement inhibition has 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, is available at www.soliris.net.

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 (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 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 more than 40 countries for the treatment of PNH, and in the United States and the European Union for the treatment of aHUS. Alexion is evaluating other potential indications for Soliris and is developing four other highly innovative biotechnology product candidates, which are being investigated across eight severe and ultra-rare disorders beyond PNH and aHUS. 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 potential health and medical benefits of Soliris® (eculizumab) for the 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, 2012, 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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