Substantial Disease Burden of PNH Demonstrated in Data from International and Asian Patient Registries Presented at European Hematology Association

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CHESHIRE, Conn., Jun 14, 2010 (BUSINESS WIRE) -- Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) today announced new research evaluating the substantial disease burden of paroxysmal nocturnal hemoglobinuria (PNH), an ultra-rare blood disorder, in patients worldwide. The International PNH Registry, involving 580 patients from 99 sites in 14 countries as of May 2010, found that the debilitating symptoms and life-threatening complications of PNH are similar across patient populations around the world. Data from a separate Asian patient registry, which included 286 patients with PNH in South Korea, examined the clinical manifestations of PNH and concluded that thrombosis is a strong predictor of mortality in these patients. The findings of these patient registries support the need for treatment with Soliris(R) (eculizumab), a first-in-class terminal complement inhibitor, to improve the prognosis and quality of life for patients living with PNH. The data were presented at the 15th Congress of the European Hematology Association held in Barcelona, Spain on June 10-13, 2010.

"These data quantify the severe and consistent impact of PNH on patients around the world and make clear the specific risks faced by patients in Asia," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "The upcoming launch of Soliris in Japan represents our first major expansion into the Asia-Pacific region, where we will continue our disease education and access initiatives to provide the life-transforming benefits of Soliris to patients in a growing number of countries."

As previously announced, Alexion has accelerated plans for the launch of Soliris in Japan based on recent approval of pricing and reimbursement for Soliris by an advisory committee of Japan's Ministry of Health, Labour and Welfare (MHLW). The Company now expects to serve initial and increasing numbers of patients with PNH in Japan in the third and fourth quarters of 2010. In April 2010, Japan's MHLW approved Soliris as a treatment for patients with PNH, making it the first therapy approved in Japan for this patient population. Soliris is also approved in the United States, European Union, Australia, Canada and South Korea as a treatment for patients with PNH.

Global Disease Burden of PNH

Results from the International PNH Registry, which has enrolled 580 patients from 99 clinical sites in 14 countries as of May 2010, showed that a history of thrombotic events (TE) was present in patients across all clone sizes but was significantly higher in patients with larger clones. Patients with PNH clone greater-than or equal to 50% were more likely to have a history of TE (20%) compared to patients with smaller PNH clones (p=0.01), however the risk of a TE is still substantially higher in patients with PNH clones <10% (4%) than expected in a normal population. (1) The registry, which continues to evaluate disease burden and determine the long-term natural history of PNH and treatment outcomes, also showed:

- Abdominal pain, chest pain, shortness of breath, and fatigue are prevalent symptoms in PNH patients.
- These patient-reported symptoms were equally prevalent across all PNH clone sizes and independent of history of bone marrow disorder.
- Transfusions are the most common treatment (55%), followed by anti-coagulants (36%), Soliris (34%) and immunosuppressive therapies (25%). As Alexion introduces Soliris in additional markets worldwide, sites participating in the registry are updating the information collected about specific treatments.

These data were presented by Dr. Alvaro Urbano-Ispizua and colleagues on Saturday, June 12 from 17:30-18:45 in Hall 6 in a poster presentation entitled, "Evaluation of Paroxysmal Nocturnal Hemoglobinuria Disease Burden in Patients Enrolled in the International PNH Registry" (Abstract #1022).

Fatigue and Impaired Quality of Life in Japanese Patients with PNH

Burden of disease data from the AEGIS study, a 12-week, open-label Phase II study of Soliris in 29 Japanese patients with PNH, was also presented at the EHA meeting. The data demonstrate that hemolysis, drives the risks and burden of disease in PNH independent of anemia. In this study, the primary endpoint of hemolysis reduction was achieved with high statistical significance with Soliris treatment. In addition, inhibition of terminal complement activation with Soliris also improved fatigue, dyspnea (difficulty breathing) and other significant morbidities of disease - also independent of anemia - in Japanese patients with PNH.

The data were presented by Dr. Yuzuru Kanakura and colleagues on Saturday, June 12 from 17:30-18:45 in Hall 6 in a poster presentation entitled, "Fatigue and Impaired Quality of Life in Patients with Paroxysmal Nocturnal Hemoglobinuria (PNH) is Associated with Hemolysis, But Not With Anemia" (Abstract #1042).

Clinical Manifestations and Disease Burden in Asian Patients with PNH

Results from a national data registry in South Korea retrospectively examined 286 PNH patients and demonstrated that Asian patients with PNH suffer similar disabling symptoms to those seen in other populations, including thrombosis, late stage kidney disease, liver dysfunction and symptoms of

pulmonary hypertension. TE are a strong predictor of mortality in Asian patients (p<0.0001). Other findings include:

- Abdominal pain is a significant predictor of both early mortality and TE in Asian patients (p=0.046 and 0.0004, respectively).
- Prominent symptoms of PNH such as hemoglobinuria (presence of hemoglobin in the urine), dyspnea and chest pain are also significant predictors of TE (p=0.015, 0.046 and 0.024, respectively).
- The risk of TE is high across all granulocyte clone sizes, and presence of concomitant bone marrow dysfunction does not diminish the risk of TE.
- Despite medical intervention with supportive care (78% of patients used corticosteroids), patients continued to show disabling symptoms, progressive complications and early mortality.

The data were presented by Dr. Lee Jong Wook and colleagues on Friday, June 11 from 17:45-19:00 in Hall 6 in two poster presentations entitled, "Clinical Symptoms of Hemolysis Are Predictive of Disease Burden and Mortality in Asian Patients with Paroxysmal Nocturnal Hemoglobinuria" (Abstract # 0506) and "High Prevalence and Mortality Associated with Thromboembolism in Asian Patients with Paroxysmal Nocturnal Hemoglobinuria (PNH)" (Abstract #0505).

About PNH

PNH is a rare blood disorder that strikes people of all ages, with an average age of onset in the early 30s. (2) Approximately 10 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 ranging from one to more than 10 years. (4) It is estimated that approximately one-third of patients with PNH do not survive more than five years from the time of diagnosis. (4) PNH has been identified more commonly among patients with disorders of the bone marrow, including aplastic anemia (AA) and myelodysplastic syndromes (MDS). (5,6,7) In patients with thrombosis of unknown origin, PNH may be an underlying cause. (2) More information on PNH is available at www.pnhsource.com.

About Soliris

Soliris (eculizumab) is a first-in-class terminal complement inhibitor developed from the laboratory through regulatory approval by Alexion. Soliris has been approved by the healthcare authorities in the U.S., European Union, Japan and other countries as the first treatment for patients with PNH, a rare, debilitating and life-threatening blood disorder defined by hemolysis, or the destruction of red blood cells. Prior to these approvals, there was no therapy specifically available for the treatment of PNH.

Patients with PNH in more than 20 countries now have access to Soliris therapy through national or private healthcare providers. As the first terminal complement inhibitor to be approved in countries around the world, Soliris represents a long-sought breakthrough in medical innovation. Alexion's innovative approach to complement inhibition has received some of 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 on Soliris is available at www.soliris.net.

Important Safety Information

Soliris is generally well tolerated in patients with PNH. The most frequent adverse events observed in clinical studies of patients with PNH were headache, nasopharyngitis (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. Meningococcal infection may become rapidly life-threatening or fatal if not recognized and treated early. 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 PNH 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 encouraged to enroll in the PNH 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.

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 and kidney diseases, transplant, other inflammatory disorders, and cancer. Soliris is Alexion's first marketed product. 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.alexionpharma.com.

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Safe Harbor Statement

This news release contains forward-looking statements, including statements related to potential health and medical benefits from Soliris, and the timing of regulatory and commercial milestones for Soliris in Japan. 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, delays in arranging satisfactory manufacturing capability and establishing commercial infrastructure, delays in developing or adverse changes in commercial relationships, the possibility that results of published reports or clinical trials are not predictive of safety and efficacy results of Soliris in broader patient populations, the risk that clinical trials may not be completed successfully, the possibility that initial results of commercialization are not predictive of future rates of adoption of Soliris, the risk that third parties won't agree to license any necessary intellectual property to Alexion on reasonable terms or at all, the risk that third party payors will not reimburse for the use of Soliris at acceptable rates or at all, 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 Annual Report on Form 10-Q for the period ended March 31, 2010, 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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