January 7, 2015

Alexion Provides Update on Phase 2 Clinical Trial with Eculizumab in Antibody Mediated Rejection (AMR) in Living-Donor Kidney Transplant Recipients

CHESHIRE, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today reported results from its randomized, open-label, multicenter Phase 2 clinical trial to determine the safety and efficacy of eculizumab in the prevention of antibody mediated rejection (AMR) in living-donor kidney transplant recipients requiring desensitization. The primary composite endpoint, defined as the occurrence of biopsy-proven AMR, graft loss, patient death, or loss to follow-up at Week 9 post-transplant, did not reach statistical significance. While the primary composite endpoint rate in the eculizumab arm was as expected from earlier studies with eculizumab, the rate in the control arm was lower than was expected, based on natural history studies reported in the literature.

"Although eculizumab performed in line with the results reported from prior eculizumab studies, which showed similarly low AMR rates in high-risk patient populations, we are disappointed that this trial did not meet its primary composite endpoint," said Martin Mackay, Ph.D., Executive Vice President and Global Head of R&D at Alexion. "We expect to complete the data analyses and discuss these results with regulators, and are currently developing plans to commence a clinical trial with eculizumab as a treatment for patients diagnosed with AMR."

About the Clinical Trial

This Phase 2 trial enrolled 102 patients receiving kidney transplants from living donors, all of whom were at risk of AMR, based on elevated levels of donor-specific antibodies. After screening, patients were randomized into two groups of 51 patients each, with one group receiving eculizumab, and a control group receiving the anti-rejection standard of care specified by the institution in which each patient's transplant took place. Patients in the standard of care control arm were eligible to receive eculizumab treatment for presumed AMR. Data were collected during the nine-week treatment period for the primary composite endpoint, with additional safety and efficacy endpoints measured through 12 months post-transplant. In the analysis of the 9-week data, for the primary composite endpoint, the rate was 9.8% in the eculizumab arm and 15.7% in the control arm (P=0.554). No safety signal has been reported to the Company by the independent data monitoring committee. Alexion expects that the data from the study will be presented at a future medical meeting.

About AMR

Acute antibody mediated rejection (AMR) is a severe and potentially life-threatening condition that can lead to severe allograft damage resulting in rapid loss of function and possible loss of the transplanted organ.[1] Patients who are sensitized (have high levels of donor-specific-antibodies [DSAs]) are at high risk for developing acute AMR.[1,2] The historical rate of AMR in high-risk living-donor kidney transplant recipients has been reported as high as 41%.[3] Acute AMR is believed to be primarily a result of uncontrolled complement activation caused by DSAs.[1,2] Currently, there are no approved therapies for the prevention or treatment of acute AMR.

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 paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (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.

[ALXN-G]

Safe Harbor Statement

This news release contains forward-looking statements, including statements related to potential medical benefits of eculizumab in AMR, our future plans for eculizumab in AMR, and the results of discussions with regulators regarding eculizumab in AMR.

Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ from those expected, including, for example, that results from the Phase 2 study may not support a regulatory submission, the possibility that studies of eculizumab in AMR may be delayed, prevented or suspended, the possibility that results of clinical trials are not predictive of safety and efficacy results of eculizumab in AMR, 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. 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.

References

1. Takemoto SK, Zeevi A, Feng S, et al. National conference to assess antibody-mediated rejection in solid organ transplantation. Am J Transplant. 2004; 4(7):1033-41.

2. Collins AB, Schneeberger EE, Pascual MA, et al. Complement activation in acute humoral renal allograft rejection: diagnostic significance of C4d deposits in peritubular capillaries. J Am Soc Nephrol. 1999;10(10):2208-14.

3. Stegall MD1, Diwan T, Raghavaiah S, et al. Terminal complement inhibition decreases antibody-mediated rejection in sensitized renal transplant recipients. Am J Transplant. 2011 Nov;11(11):2405-13.

Alexion:
Media
Irving Adler, 203-271-8210
Executive Director, Corporate Communications
or
Kim Diamond, 203-439-9600
Senior Director, Corporate Communications
or
Investors
Elena Ridloff, CFA, 203-699-7722
Executive Director, Investor Relations

Source: Alexion Pharmaceuticals, Inc.

News Provided by Acquire Media