June 12, 2017

Acceleron Announces Top-Line Results from DART Phase 2 Study of Dalantercept in Advanced Renal Cell Carcinoma

- DART Phase 2 study of dalantercept plus axitinib did not achieve its primary endpoint -

- Acceleron to discontinue development of dalantercept -

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Acceleron Pharma Inc. (NASDAQ:XLRN), a clinical stage biopharmaceutical company focused on the discovery, development and commercialization of innovative therapeutics to treat serious and rare diseases, today announced that the DART Phase 2 study of dalantercept plus axitinib did not achieve its primary endpoint in advanced renal cell carcinoma (RCC). The primary efficacy endpoint of the study was to demonstrate a statistically significant increase in progression-free survival (PFS) for treatment of dalantercept plus axitinib versus placebo plus axitinib in advanced RCC patients.

"We designed a robust Phase 2 study to evaluate the efficacy of dalantercept in combination with anti-VEGF therapy in advanced renal cell carcinoma patients whose disease has progressed on prior anti-VEGF therapy. We are disappointed by the results given the need for new agents that improve outcomes for patients with advanced RCC. We would like to thank the patients, caregivers, investigators, and our team who made the DART study possible," said Habib Dable, President and Chief Executive Officer of Acceleron. "Based on the lack of efficacy, we are discontinuing the development of dalantercept. We remain focused on the development of luspatercept across multiple Phase 3 and Phase 2 studies and ACE-083 across two neuromuscular diseases, and will continue to pursue additional candidates in areas of high unmet medical need."

The DART study enrolled 131 patients with advanced RCC. The efficacy data are based on the All-Treated Set (ATS) which is defined as all randomized patients who received any study drug (n=119) as of the database cutoff. In the ATS, 58 patients were randomized to dalantercept plus axitinib and 61 patients were randomized to placebo plus axitinib.

The median PFS for dalantercept plus axitinib was 6.8 months versus 5.6 months for placebo plus axitinib. Dalantercept plus axitinib did not decrease the rate of disease progression or death (HR 1.11, two-sided 95% CI [0.71, 1.73], one-sided p-value 0.67). The key secondary endpoint for the study was PFS for patients who received two or more prior systemic anti-cancer therapies. In this analysis, the median PFS for dalantercept plus axitinib was 8.1 months versus 7.0 months for placebo plus axitinib (HR 0.78, two-sided 95% CI [0.33, 1.87], one-sided p-value 0.29). The confirmed objective response rate (ORR) for dalantercept plus axitinib was 19% versus 25% for placebo plus axitinib (p-value 0.43, Cochran-Mantel-Haenszel test).

The safety data are based on the 119 ATS patients. The frequency of Grade 3 or higher adverse events (AEs) regardless of causality were similar overall in the dalantercept plus axitinib (59%) and the placebo plus axitinib (64%) study arms. The frequency of serious AEs of any grade regardless of causality were also similar in the dalantercept plus axitinib (29%) and the placebo plus axitinib (26%) study arms. The AEs associated with dalantercept were consistent with those previously observed.

About the DART Phase 2 Study

The Phase 2 DART clinical trial is a two-part study in patients with advanced renal cell carcinoma. Part 1 is a dose-escalation study of dalantercept plus axitinib to evaluate the safety and tolerability of the combination in patients whose disease has progressed following one to three lines of prior therapy. Part 2 is a randomized, double-blind study of 130 patients with advanced renal cell carcinoma who have progressed following treatment with a VEGF receptor tyrosine kinase inhibitor. Patients may have also received prior mTOR therapy and/or immunotherapy. For additional information on this clinical trial, please visit www.clinicaltrials.gov, identifier NCT01727336.

About Dalantercept

Dalantercept is an investigational protein therapeutic that inhibits angiogenesis by preventing BMP9, a protein in the transforming growth factor-beta (TGF-beta) superfamily, from interacting with activin receptor-like kinase 1 (ALK1), a cell-surface receptor found on proliferating vascular endothelial cells. Dalantercept inhibits ALK1 signaling, which is required for the development of mature, functional vasculature.

About Acceleron

Acceleron is a clinical stage biopharmaceutical company focused on the discovery, development and commercialization of innovative therapeutics to treat serious and rare diseases. Its pioneering research platform leverages the powerful biology behind the body's ability to rebuild and repair its own cells and tissues. This approach to drug discovery has generated four therapeutic candidates that are currently in clinical trials. The Company's lead therapeutic candidate, luspatercept, is being evaluated in Phase 3 studies for the treatment of the hematologic diseases myelodysplastic syndromes (MDS) and beta-thalassemia under a global partnership with Celgene. Acceleron is also advancing its ACE-083 clinical program in the field of neuromuscular disease, and has a comprehensive preclinical research effort targeting fibrotic and other serious diseases.

For more information, please visit www.acceleronpharma.com/. Follow Acceleron on Social Media: @AcceleronPharma and LinkedIn.

Cautionary Note on Forward-Looking Statements

This press release contains forward-looking statements about the Company's strategy, future plans and prospects, including statements regarding the development of the Company's compounds, including dalantercept, the timeline for clinical development and regulatory approval of the Company's compounds, the expected timing for the reporting of data from ongoing trials, and the structure of the Company's planned or pending clinical trials. The words "anticipate," "appear," "believe," "continue," "could," "estimate," "expect," "forecast," "goal," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "will," "would," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

Each forward-looking statement is subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statement. Applicable risks and uncertainties include the risks that preclinical testing of the Company's compounds and data from clinical trials may not be predictive of the results or success of ongoing or later clinical trials, that data may not be available when the Company expects it to be, that the Company or its collaboration partner, Celgene, will be unable to successfully complete the clinical development of the Company's compounds, that the development of the Company's compounds will take longer or cost more than planned, that the Company or Celgene may be delayed in initiating, enrolling or completing any clinical trials, that the Company's drug discovery activities may not yield drug candidates for which the Company can commence clinical trials at the rate at which the Company currently anticipates or at all, and that the Company's compounds will not receive regulatory approval or become commercially successful products.

Other risks and uncertainties include those identified under the heading "Risk Factors" included in the Company's Annual Report on Form 10-K which was filed with the Securities and Exchange Commission (SEC) on March 1, 2017, and other filings that the Company has made and may make with the SEC in the future. The forward-looking statements contained in this press release reflect the Company's current views with respect to future events, and the Company does not undertake and specifically disclaims any obligation to update any forward-looking statements.

View source version on businesswire.com: http://www.businesswire.com/news/home/20170612006226/en/

Acceleron Pharma Inc.
Todd James, IRC, 617-649-9393
Vice President, Investor Relations and Corporate Communications
or
Candice Ellis, 617-649-9226
Manager, Investor Relations and Corporate Communications
or
Media:
BMC Communications
Brad Miles, 646-513-3125

Source: Acceleron Pharma