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Acceleron Showcases Vision and Strategy at 2017 R&D Day

- Clinical development and research efforts focused on hematological, neuromuscular and pulmonary diseases -

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Acceleron Pharma Inc. (NASDAQ:XLRN), a leading biopharmaceutical company in the discovery and development of TGF-beta therapeutics to treat serious and rare diseases, hosted its Research and Development (R&D) Day in New York City today. The Company discussed clinical development plans to build therapeutic area leadership, and its long-term strategy to advance key programs in hematologic, neuromuscular, and pulmonary diseases.

"We have several, near-term milestones in our mid- to late-stage pipeline that could transform our business from a development-stage organization to a fully-integrated, commercial biopharmaceutical company," said Habib Dable, Chief Executive Officer of Acceleron. "With the opportunity to launch and co-promote luspatercept in North America, and the continued advancement of our neuromuscular candidate as well as our recent entry into pulmonary disease, we are positioned to bring potentially life-changing new treatments to patients and drive value for our shareholders. We expect to report top-line Phase 3 results of luspatercept in myelodysplastic syndromes and beta-thalassemia alongside our collaboration partner Celgene, in mid-2018. ACE-083 continues to advance through multiple Phase 2 clinical trials, and ACE-2494, the second asset in our neuromuscular franchise, is expected to initiate a clinical trial later this year. The recent announcement of our plan to develop sotatercept as a Phase 2 therapeutic candidate for the potential treatment of pulmonary arterial hypertension is a tremendous opportunity for us, given that it launches our therapeutic focus in pulmonary disease and enhances our commitment to develop new therapies that could transform patients' lives."

Key R&D Day Highlights:

Hematology

- Luspatercept
  - Significant market expansion efforts across multiple new indications are being pursued by Acceleron and Celgene in addition to the potential blockbuster market opportunity in initial MEDALIST and BELIEVE Phase 3 indications
  - A third Phase 3 trial, also known as the COMMANDS trial, is expected to begin in early 2018
    - The COMMANDS trial will evaluate luspatercept versus erythropoiesis-stimulating agent (ESA) treatment in first-line, lower-risk MDS, and include patients regardless of ring sideroblast (RS) status
  - A Phase 2 trial in non-transfusion-dependent (NTD) beta-thalassemia patients, also known as the BEYOND trial, is expected to begin later this year, and the Phase 2 trial in myelofibrosis is underway

Neuromuscular

- ACE-083 and ACE-2494
  - ACE-083 and ACE-2494 utilize the Company's "Myostatin+" approach to increase muscle strength and function by inhibiting multiple ligands of the TGF-beta superfamily
  - ACE-083, a targeted muscle agent being developed for the treatment of facioscapulohumeral dystrophy (FSHD) and Charcot-Marie-Tooth (CMT) disease, has shown increases in muscle mass of 9% to 15% in healthy volunteers
    - Company expects Phase 2 FSHD part 1 preliminary results from cohort 1 to be available in late 2017
  - ACE-2494, a systemic muscle agent, has demonstrated preclinical activity across multiple neuromuscular diseases
    - Company expects to initiate Phase 1 clinical trial in late 2017

Pulmonary

- Sotatercept
  - Acceleron gains rights for pulmonary hypertension indications from Celgene

- Preclinical results show potential for sotatercept to be a first-in-class disease-modifying therapy that addresses fundamental molecular causes of disease in pulmonary arterial hypertension (PAH)
- Company expects to initiate a Phase 2 trial in PAH during the first half of 2018

- ACE-1334
  - Multiple, ongoing preclinical studies in pulmonary disease

About Acceleron

Acceleron is a biopharmaceutical leader in TGF-beta science, focused on the discovery and development of innovative therapeutics to treat patients with serious and rare diseases. Its pioneering research and protein engineering platform engages the target-rich TGF-beta superfamily and its ability to regulate cellular growth and repair.

Under a global partnership with Celgene, Acceleron is in Phase 3 development with luspatercept, a potential first-in-class chronic anemia therapy for the treatment of rare blood diseases. The Company is also advancing a neuromuscular franchise with two distinct Myostatin+ agents, ACE-083 and ACE-2494, and a pulmonary program with a Phase 2 trial of sotatercept planned in pulmonary arterial hypertension. Acceleron has ongoing preclinical research efforts targeting additional indications in these three disease areas where there is significant unmet medical need.

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, the timeline for clinical development and regulatory approval of the Company's compounds and the expected timing for reporting of data from ongoing clinical trials. The words "anticipate," "believe," "could," "estimate," "expect," "intend," "may," "plan," "potential," "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.

Actual results could differ materially from those included in the forward-looking statements due to various risks and uncertainties, including, but not limited to, 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 the development of the Company's compounds will take longer and/or cost more than planned, that the Company or its collaboration partner, Celgene, will be unable to successfully complete the clinical development of the Company's compounds, that the Company or Celgene may be delayed in initiating, enrolling or completing any clinical trials, and that the Company's compounds will not receive regulatory approval or become commercially successful products. These and other risks and uncertainties are identified under the heading "Risk Factors" included in the Company's most recent Annual Report on Form 10-K, 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 are based on management's current views, plans, estimates, assumptions and projections with respect to future events, and the Company does not undertake and specifically disclaims any obligation to update any forward-looking statements.

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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