# Acceleron Reports First Quarter 2019 Operating and Financial Results

May 9, 2019

*– Luspatercept Biologics License Application (BLA) and Marketing Authorization Application (MAA) submitted in April 2019–*

*– Part 2 results from the ACE-083 Phase 2 trials in facioscapulohumeral muscular dystrophy (FSHD) expected in the second half of 2019 and Charcot-Marie-Tooth disease (CMT) anticipated in Q1 2020 –*

*– PULSAR and SPECTRA Phase 2 trials of sotatercept in pulmonary arterial hypertension (PAH) on track with topline results expected from PULSAR in 1H 2020 –*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 9, 2019-- Acceleron Pharma Inc. (Nasdaq:XLRN), a leading biopharmaceutical company in the discovery and development of TGF-beta superfamily therapeutics to treat serious and rare diseases, today provided a corporate update and reported financial results for the first quarter ended March 31, 2019.

"With the submission of marketing applications for luspatercept in the U.S. and E.U. last month, we and our global collaboration partner, Celgene, are excited about the potential to bring a new therapy to patients with myelodysplastic syndromes and beta-thalassemia within the next year," said Habib Dable, President and Chief Executive Officer of Acceleron. "At the same time, our pulmonary program remains on track, with enrollment ongoing in two Phase 2 trials of sotatercept in PAH, and we are anticipating topline results from the placebo-controlled part of the Phase 2 trial of our locally-acting muscle agent, ACE-083, in FSHD during the second half of this year."

**Development Program Highlights**

***Hematology***

**Luspatercept: Myelodysplastic Syndromes (MDS), Beta-Thalassemia, and Myelofibrosis (MF)**

*Luspatercept is an investigational first-in-class erythroid maturation agent (EMA) designed to address a late-stage erythroid maturation defect that results in chronic anemia and the need for regular red blood cell transfusions in adults with serious hematologic diseases. Luspatercept is part of the global collaboration between Acceleron and Celgene.*

- Celgene recently submitted a Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) and a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) for luspatercept in patients with MDS- and beta-thalassemia-associated anemia based on the safety and efficacy results of the pivotal Phase 3 studies MEDALIST and BELIEVE.
- The Companies expect to announce preliminary topline results from the Phase 2 trial of luspatercept in patients with MF in the second half of 2019.
- Enrollment is ongoing in the COMMANDS Phase 3 trial in patients with first-line lower-risk MDS and the BEYOND Phase 2 trial in patients with non-transfusion-dependent beta-thalassemia, with preliminary results expected from the BEYOND trial in 2020.

***Neuromuscular Disease***

**ACE-083: Facioscapulohumeral Muscular Dystrophy (FSHD) and Charcot-Marie-Tooth Disease (CMT)**

*ACE-083 is an investigational locally-acting therapeutic designed to have a concentrated effect on muscle mass and strength in target muscles for diseases that cause focal muscle weakness. ACE-083 utilizes the "Myostatin+" approach to inhibit multiple TGF-beta superfamily ligands involved in muscle formation.*

- Previously presented results from Part 1 of the Phase 2 trial evaluating ACE-083 in patients with FSHD were highlighted in an encore presentation at the Muscular Dystrophy Association (MDA) Clinical & Scientific Conference in April 2019.
- Preliminary results from Part 2 of the Phase 2 trial in patients with FSHD are expected in the second half of 2019.
- Previously presented results from Part 1 of the Phase 2 trial evaluating ACE-083 in patients with CMT will be highlighted in a platform presentation at the American Academy of Neurology (AAN) 71$^{st}$ Annual Meeting on May 10, 2019.
- Enrollment is ongoing in Part 2 of the Phase 2 trial in patients with CMT, with preliminary results expected in the first quarter of 2020.

***Pulmonary Disease***

**Sotatercept: Pulmonary Arterial Hypertension (PAH)**

*Sotatercept is an investigational agent designed to be a selective ligand trap for members of the TGF-beta superfamily to rebalance BMPR2 signaling, which is a key molecular driver of PAH. In preclinical studies of PAH, sotatercept reversed pulmonary vessel muscularization and improved indicators*

*of right heart failure.*

- Enrollment is ongoing in the PULSAR Phase 2 trial in patients with PAH, with topline results expected in the first half of 2020.
- Enrollment is ongoing in the exploratory SPECTRA trial in patients with PAH, with preliminary results expected in 2020.
- A preclinical abstract of sotatercept in PAH has been accepted for presentation at the American Thoracic Society (ATS) 2019 International Conference on May 21, 2019.

**Financial Results**

- **Cash Position** – Cash, cash equivalents and investments as of March 31, 2019 were $513.1 million. As of December 31, 2018, the Company had cash, cash equivalents and investments of $291.3 million. Based on the Company's current operating plan and projections, it believes that current cash, cash equivalents and investments will be sufficient to fund projected operating requirements until such time as it expects to receive significant royalty revenue from luspatercept sales.
- **Revenue** – Collaboration revenue for the first quarter was $2.8 million. The revenue is all from the Company's partnership with Celgene and is primarily related to expenses incurred by the Company in support of luspatercept.
- **Costs and Expenses** – Total costs and expenses for the first quarter were $43.6 million. This includes R&D expenses of $32.8 million and G&A expenses of $10.8 million.
- **Net loss** – The Company's net loss for the first quarter ended March 31, 2019 was $38.1 million.

**Conference Call and Webcast**

The Company will host a webcast and conference call to discuss its first quarter 2019 financial results and provide an update on recent corporate activities on May 9, 2019, at 5:00 p.m. EDT.

The webcast will be accessible under "Events & Presentations" in the Investors/Media page of the Company's website at www.acceleronpharma.com. Individuals can participate in the conference call by dialing 877-312-5848 (domestic) or 253-237-1155 (international) and referring to the "Acceleron First Quarter 2019 Earnings Call."

The archived webcast will be available for replay on the Acceleron website approximately two hours after the event.

**About Acceleron**

Acceleron is a clinical-stage biopharmaceutical company dedicated to the discovery, development, and commercialization of therapeutics to treat serious and rare diseases. The Company's leadership in the understanding of TGF-beta biology and protein engineering generates innovative compounds that engage the body's ability to regulate cellular growth and repair.

Acceleron focuses its research and development efforts in hematologic, neuromuscular, and pulmonary diseases. In hematology, the Company and its global collaboration partner, Celgene, are developing luspatercept for the treatment of chronic anemia in myelodysplastic syndromes, beta-thalassemia, and myelofibrosis. Acceleron is also advancing its neuromuscular program with ACE-083, a locally-acting Myostatin+ agent in Phase 2 development in facioscapulohumeral muscular dystrophy and Charcot-Marie-Tooth disease and is conducting a Phase 2 pulmonary program with sotatercept in pulmonary arterial hypertension.

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

**ACCELERON PHARMA INC.**
**CONDENSED CONSOLIDATED BALANCE SHEET**
**(Amounts in thousands)**
**(unaudited)**

| | March 31, 2019 | December 31, 2018 |
|---|---|---|
| Cash and cash equivalents | $ 214,490 | $ 144,052 |
| Short and long-term investments | 298,615 | 147,260 |
| Operating lease assets | 27,817 | — |
| Other assets | 19,408 | 23,509 |
| Total assets | $ 560,330 | $ 314,821 |
| | | |
| Short-term and long-term operating lease liabilities | $ 30,455 | $ — |
| Warrants to purchase common stock | 1,607 | 1,491 |
| Other liabilities | 17,733 | 21,292 |
| Total liabilities | 49,795 | 22,784 |
| Total stockholders' equity | 510,535 | 292,037 |
| Total liabilities and stockholders' equity | $ 560,330 | $ 314,821 |

**ACCELERON PHARMA INC.**
**CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS**
**(Amounts in thousands except per share data)**
**(unaudited)**

| | Three Months Ended March 31, | |
|---|---|---|
| | **2019** | **2018** |
| Revenue: | | |
| Collaboration revenue | $ 2,780 | $ 3,232 |
| Costs and expenses: | | |
| Research and development | 32,771 | 23,431 |
| General and administrative | 10,814 | 7,441 |
| Total costs and expenses | 43,585 | 30,872 |
| Loss from operations | (40,805) | (27,640) |
| Total other income, net | 2,772 | 1,431 |
| Loss before income taxes | (38,033) | (26,209) |
| Income tax provision | (20) | (10) |
| Net loss applicable to common stockholders - basic and diluted | $ (38,053) | $ (26,219) |
| Net loss per share applicable to common stockholders - basic and diluted | $ (0.74) | $ (0.58) |
| Weighted-average number of common shares used in computing net loss per share applicable to common stockholders | 51,126 | 45,516 |

**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," "goal," "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 factors, 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 results of any clinical trial may not be predictive of the results or success of other clinical trials of the same product candidate, 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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