# Acceleron Reports Fourth Quarter and Full Year 2018 Operating and Financial Results

February 27, 2019

*- MEDALIST and BELIEVE Phase 3 trial presentations at the 60th American Society of Hematology (ASH) Annual Meeting both selected for "Best of ASH" -*

*- Luspatercept Biologics License Application (BLA) submission expected in April 2019 -*

*- Part 2 results from the ACE-083 Phase 2 trials in facioscapulohumeral muscular dystrophy (FSHD) and Charcot-Marie-Tooth disease (CMT) anticipated during 2019 -*

*- Pulmonary program on track with two Phase 2 trials of sotatercept in pulmonary arterial hypertension (PAH) currently active -*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 27, 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 fourth quarter and full year ended December 31, 2018.

"A series of significant achievements in 2018 have positioned Acceleron to execute on a number of key milestones across the entire pipeline during 2019 and 2020," said Habib Dable, President and Chief Executive Officer of Acceleron. "First and foremost, we and our global collaboration partner, Celgene, plan to submit marketing applications in the U.S. and E.U. for our lead product candidate, luspatercept, in lower-risk myelodysplastic syndromes and transfusion-dependent beta-thalassemia in the first half of the year. At the same time, we're continuing to evaluate luspatercept's potential to treat a range of anemias, from first-line therapy in MDS via the ongoing COMMANDS Phase 3 trial, to non-transfusion-dependent beta-thalassemia, myelofibrosis and beyond."

"In addition, our wholly-owned programs in neuromuscular and pulmonary disease are all advancing. We're anticipating topline results from the placebo controlled part of our Phase 2 trials evaluating our locally-acting muscle agent, ACE-083, in FSHD and CMT by the end of the year. Lastly, 2020 will bring important Phase 2 trial results in PAH with sotatercept, which we believe has the potential to alter the treatment landscape for this devastating disease."

**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.*

- The MEDALIST and BELIEVE Phase 3 trial results in patients with lower-risk MDS and transfusion-dependent beta-thalassemia, respectively, were presented at the 60th ASH Annual Meeting and Exposition in December 2018.
  - The MEDALIST and BELIEVE presentations were both selected for presentation during the "Best of ASH" session at the meeting.
  - Acceleron and Celgene plan to submit a Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) for luspatercept in patients with anemia related lower-risk MDS and beta-thalassemia in April 2019.
  - Acceleron and Celgene remain on track to submit a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) for luspatercept in patients with anemia related to lower-risk MDS and beta-thalassemia in the first half of 2019.
- The ongoing Phase 2 trial of luspatercept in patients with MF has completed target enrollment, with preliminary results expected 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.*

- Preliminary results from Part 1 of the Phase 2 trials evaluating ACE-083 in patients with FSHD and CMT, were presented

at the 2018 World Muscle Society (WMS) Annual Meeting in October 2018.
- Part 2 of the Phase 2 FSHD trial has completed patient enrollment, with preliminary topline results expected in the second half of 2019.
- Enrollment is ongoing in Part 2 of the Phase 2 CMT trial, with preliminary results expected by the end of 2019.

**ACE-2494: Neuromuscular Disease**

*ACE-2494 is designed to have a systemic effect on muscle mass and strength for diseases that cause muscle weakness throughout the body. ACE-2494 utilizes the "Myostatin+" approach to inhibit multiple TGF-beta superfamily ligands involved in muscle formation.*

- Enrollment is ongoing in the Phase 1 healthy volunteer trial, with preliminary results expected in the first half of 2019.

***Pulmonary Disease***

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

*Sotatercept acts as a multi-ligand trap for certain members of the TGF-beta superfamily to rebalance BMPRII 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.*

- Preclinical results from multiple studies of sotatercept in PAH were presented at the American Heart Association Scientific Sessions in November 2018.
- Enrollment is ongoing in the PULSAR Phase 2 trial in patients with PAH, with preliminary results expected in the first half of 2020.
- The exploratory SPECTRA trial in patients with PAH has been initiated, with preliminary results expected in 2020.

**Corporate Highlights**

- The Company recently raised approximately $264.5 million of gross proceeds in a follow-on offering of common stock.

**Financial Results**

- **Cash Position** – Cash, cash equivalents and investments as of December 31, 2018 were $291.3 million. As of December 31, 2017, the Company had cash, cash equivalents and investments of $372.9 million. Based on the Company's current operating plan and projections, it believes that current cash, cash equivalents and investments, together with the net proceeds of $248.2 million from its recent common stock offering, 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 year was $14.0 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 year were $138.4 million. This includes R&D expenses of $103.9 million and G&A expenses of $34.5 million.
- **Net Loss** – The Company’s net loss for the year ended December 31, 2018 was $118.9 million.

**Conference Call and Webcast**

The Company will host a webcast and conference call to discuss its fourth quarter and full year financial results for 2018 and provide an update on recent corporate activities on February 27, 2019, at 5:00 p.m. EST.

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 Fourth Quarter and Full Year 2018 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 franchise with two distinct Myostatin+ agents, ACE-083 and ACE-2494, and 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)**

| | December 31, 2018 | December 31, 2017 |
|---|---|---|
| Cash and cash equivalents | $ 144,052 | $ 100,150 |
| Short and long-term investments | 147,260 | 272,800 |
| Other assets | 23,509 | 16,227 |
| Total assets | $ 314,821 | $ 389,177 |
| | | |
| Deferred revenue | $ — | $ 3,703 |
| Warrants to purchase common stock | 1,491 | 2,236 |
| Other liabilities | 21,293 | 18,021 |
| Total liabilities | 22,784 | 23,960 |
| Total stockholders' equity | 292,037 | 365,217 |
| Total liabilities and stockholders' equity | $ 314,821 | $ 389,177 |

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

| | Three Months Ended December 31, | | Year Ended December 31, | |
|---|---|---|---|---|
| | 2018 | 2017 | 2018 | 2017 |
| Revenue: | | | | |
| Collaboration revenue | $ 3,816 | $ 3,705 | $ 13,991 | $ 13,481 |
| Costs and expenses: | | | | |
| Research and development | 29,867 | 25,339 | 103,902 | 89,726 |
| General and administrative | 10,755 | 7,002 | 34,503 | 33,738 |
| Total costs and expenses | 40,622 | 32,341 | 138,405 | 123,464 |
| Loss from operations | (36,806) | (28,636) | (124,414) | (109,983) |
| Total other income, net | 2,036 | 770 | 5,516 | 1,561 |
| Loss before income taxes | (34,770) | (27,866) | (118,898) | (108,422) |
| Income tax benefit (provision) | 36 | (60) | 27 | (32) |
| Net loss | $ (34,734) | $ (27,926) | $ (118,871) | $ (108,454) |
| | | | | |
| Net loss per share - basic and diluted | $ (0.75) | $ (0.62) | $ (2.59) | $ (2.68) |
| | | | | |
| Weighted-average number of common shares used in computing net loss per share | 46,227 | 45,217 | 45,898 | 40,420 |

**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 trials may not be predictive of the results or success of other 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.
Investors:
Todd James, IRC, 617-649-9393
Vice President, Investor Relations and Corporate Communications

Media:
Matt Fearer, 617-301-9557
Director, Corporate Communications