Acceleron Reports Third Quarter 2019 Operating and Financial Results

November 6, 2019

- FDA's review of luspatercept BLA for the beta-thalassemia and lower-risk MDS indications remains ongoing -

- Six clinical abstracts accepted for presentation at the 61st American Society of Hematology (ASH) Annual Meeting -

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 6, 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 third quarter ended September 30, 2019.

"With the FDA's decision on the luspatercept regulatory submission for the treatment of beta-thalassemia expected in less than one month, we are close to achieving a major company milestone-the potential approval for the first Acceleron-discovered medicine," said Habib Dable, President and Chief Executive Officer of Acceleron. "Alongside our global collaboration partner, Celgene, we are preparing for luspatercept's potential commercial launch in the U.S. We also await the FDA's decision on our BLA for the MDS indication in April 2020 and the European Medicines Agency's decision on the MAA for both indications, which is expected in the second half of 2020. We continue to advance our ongoing clinical trials in first-line lower-risk MDS-, non-transfusion-dependent beta-thalassemia- and myelofibrosis-associated anemia. In addition, we are looking forward to presenting luspatercept updates on our Phase 3 MEDALIST and BELIEVE trials, as well as interim results from the ongoing Phase 2 myelofibrosis trial at the upcoming ASH meeting in December."

Added Mr. Dable: "While our hematology program continues to grow, we are also advancing our two Acceleron-led clinical programs in pulmonary and neuromuscular disease. We anticipate reporting results in the first quarter of 2020 for both our PULSAR Phase 2 sotatercept trial in patients with PAH, and our ACE-083 trial in patients with CMT."

Development Program Highlights

Hematology

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

Luspatercept is an investigational first-in-class erythroid maturation agent 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 U.S. Food and Drug Administration's (FDA) review of the Biologics License Application (BLA) for luspatercept is ongoing for the treatment of anemia in adult patients with very low- to intermediate-risk MDS, who have ring sideroblasts and require red blood cell (RBC) transfusions, and for the treatment of anemia in adult patients with beta-thalassemia, who require regular RBC transfusions.
    - The FDA's target action date for the beta-thalassemia indication is December 4, 2019, and April 4, 2020 for the MDS indication.
- The European Medicines Agency's (EMA) review of the Marketing Authorization Application (MAA) for luspatercept for the treatment of anemia in adult patients with MDS or beta-thalassemia is ongoing. The EMA decision on the MAA is expected in the second half of 2020.
- Six clinical abstracts of luspatercept have been accepted for presentation at the 61st American Society of Hematology (ASH) Annual Meeting and Exposition in Orlando on December 7-10, 2019, including
    - Five clinical abstracts highlighting new and updated analyses from the MEDALIST and BELIEVE Phase 3 trials;
    - One clinical abstract including interim results from the ongoing Phase 2 trial in MF.
- Enrollment is ongoing in the COMMANDS Phase 3 trial in patients with treatment-naïve, lower-risk MDS.
- The BEYOND Phase 2 trial in adult patients with non-transfusion-dependent beta-thalassemia is ongoing, with topline results expected by year-end 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 (RAP-011) reversed pulmonary vessel muscularization and improved indicators of right heart failure.

- The FDA granted Orphan Drug Designation to sotatercept for the treatment of patients with PAH.

- Topline results from the PULSAR Phase 2 trial in patients with PAH are expected in the first quarter of 2020.
- Enrollment is ongoing in the exploratory SPECTRA trial in patients with PAH, with preliminary results expected in 2020.

Neuromuscular Disease

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

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.

- Topline results from Part 2 of the Phase 2 trial in patients with CMT are expected in the first quarter of 2020.
- Clinical development of ACE-083 in patients with FSHD has been discontinued following the Phase 2 topline results.

Financial Results

- **Cash Position -** Cash, cash equivalents and investments as of September 30, 2019 were $468.3 million, as compared to $291.3 million as of December 31, 2018.

  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** - Revenue for the third quarter of 2019 was $4.2 million. This revenue was derived from the Company's collaboration partnership with Celgene and is largely related to expenses incurred by the Company in support of luspatercept.
- **Costs and Expenses** - Total costs and expenses for the third quarter of 2019 were $53.1 million. This includes R&D expenses of $37.6 million and G&A expenses of $15.5 million.
- **Net Loss** - The Company's net loss for the third quarter of 2019 was $45.4 million.

Conference Call and Webcast

The Company will host a webcast and conference call to discuss its third quarter 2019 financial results and provide an update on recent corporate activities on November 6, 2019, at 10:00 a.m. EST.

The webcast will be accessible under "Events & Presentations" in the Investors/Media page of the Company's website at 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 Third 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 superfamily 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 Charcot-Marie-Tooth disease and is conducting a Phase 2 pulmonary program with sotatercept in pulmonary arterial hypertension.

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

ACCELERON PHARMA INC.

CONDENSED CONSOLIDATED BALANCE SHEET

(Amounts in thousands)

(unaudited)

	September 30, 2019	December 31, 2018
Cash and cash equivalents	$ 169,542	$ 144,052
Short and long-term investments	298,747	147,260
Operating lease assets	25,247	—
Other assets	26,131	23,509
Total assets	$ 519,667	$ 314,821
Short-term and long-term operating lease liabilities	$ 27,813	$ —
Warrants to purchase common stock	1,326	1,491
Other liabilities	25,727	21,293
Total liabilities	54,866	22,784
Total stockholders' equity	464,801	292,037
Total liabilities and stockholders' equity	$ 519,667	$ 314,821

ACCELERON PHARMA INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

(Amounts in thousands except per share data)

(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Revenue:				
Collaboration revenue	$ 4,208	$ 3,258	$ 34,655	$ 10,175
Costs and expenses:				
Research and development	37,630	24,667	105,125	74,027
General and administrative	15,501	8,653	40,394	23,756
Total costs and expenses	53,131	33,320	145,519	97,783
Loss from operations	(48,923)	(30,062)	(110,864)	(87,608)

Other income (expense):				
Total other income, net	3,520	1,071	9,523	3,481
Loss before income taxes	(45,403)	(28,991)	(101,341)	(84,127)
Income tax benefit (provision)	34	12	58	(9)
Net loss applicable to common stockholders- basic and diluted	$ (45,369)	$ (28,979)	$ (101,283)	$ (84,136)
Net loss per share applicable to common stockholders- basic and diluted	$ (0.86)	$ (0.63)	$ (1.94)	$ (1.84)
Weighted-average number of common shares used in computing net loss per share applicable to common stockholders	52,882	46,051	52,239	45,787

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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Source: Acceleron Pharma Inc.

Acceleron Pharma Inc.
Investors:
Todd James, IRC, 617-649-9393
Vice President, Investor Relations and Corporate Communications

Ed Joyce, 617-649-9242
Director, Investor Relations

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