Acceleron Reports Second Quarter 2019 Operating and Financial Results

August 5, 2019

- BLA and MAA filings of luspatercept for beta-thalassemia- and myelodysplastic syndromes-associated anemia accepted by U.S. Food and Drug Administration and European Medicines Agency, respectively -

- Acceleron receives $25 million milestone payment from Celgene for luspatercept's BLA acceptance and MAA validation -

- The ACE-083 Phase 2 trial in Charcot-Marie-Tooth disease (CMT) has completed enrollment with topline results anticipated in Q1 2020, and results from the facioscapulohumeral muscular dystrophy (FSHD) trial are expected in 2H19 -

- PULSAR Phase 2 trial of sotatercept in pulmonary arterial hypertension (PAH) is fully enrolled, with topline results expected in Q1 2020 -

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 5, 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 second quarter ended June 30, 2019.

"The significant progress across our entire pipeline over the last few years has us well positioned to execute during one of the most exciting times in Acceleron's 16-year history. With the luspatercept U.S. and European regulatory approval filings under review, we are now one step closer to the first-ever potential approval of an Acceleron-discovered medicine," said Habib Dable, President and Chief Executive Officer of Acceleron. "Alongside our global collaboration partner, Celgene, we are focused on preparing for luspatercept's potential commercial launch, and we continue to execute on our ongoing clinical trials in first-line lower-risk MDS-, non-transfusion-dependent beta-thalassemia- and myelofibrosis-associated anemia."

Added Mr. Dable: "In parallel, we have advanced our two Acceleron-led clinical programs in neuromuscular and pulmonary disease as we work to establish key proof-of-concept results in three placebo-controlled Phase 2 trials over the next nine months. Following robust enrollment in our PULSAR Phase 2 trial in patients with PAH, we now expect topline results in the first quarter of 2020. For ACE-083, we anticipate topline results in patients with FSHD and CMT in the second half of this year and early 2020, respectively."

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 (FDA) accepted the Biologics License Application (BLA) for luspatercept for the treatment of adult patients with very low- to intermediate-risk MDS-associated anemia who have ring sideroblasts and require red blood cell (RBC) transfusions, and for the treatment of adult patients with beta-thalassemia-associated anemia who require RBC transfusions.
- The FDA granted priority review for the beta-thalassemia indication and set a target action date of December 4, 2019, and set a target action date of April 4, 2020, for the MDS indication.
- The Marketing Authorization Application (MAA) for luspatercept in adult patients with MDS- or beta-thalassemia-associated anemia has been validated by the European Medicines Agency (EMA). The EMA decision on the MAA is expected in the second half of 2020.
- Results from the Phase 2 trial of luspatercept in patients with MF are expected later this year.
- Enrollment is ongoing in the COMMANDS Phase 3 trial in patients with treatment-naïve lower-risk MDS and the BEYOND Phase 2 trial in patients with non-transfusion-dependent beta-thalassemia. Topline results from the BEYOND trial are expected by year-end 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.

- Topline results from Part 2 of the Phase 2 trial in patients with FSHD are expected in the second half of 2019.

- Enrollment was recently completed in Part 2 of the Phase 2 trial in patients with CMT, with topline results expected in the first quarter of 2020.
- The Phase 2 extension trial is open for patients who participated in the FSHD and CMT Phase 2 trials of ACE-083.

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.

- The PULSAR Phase 2 trial in patients with PAH has completed enrollment, with topline results expected in Q1 2020.
- Enrollment is ongoing in the exploratory SPECTRA trial in patients with PAH, with preliminary results expected in 2020.

Financial Results

- **Cash Position** – Cash, cash equivalents and investments as of June 30, 2019 were $500.9 million. This cash balance includes the receipt of a $25.0 million gross milestone payment for the acceptance of the luspatercept BLA and MAA filings. 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** – Second quarter revenue was $27.7 million. The revenue is all from the Company's collaboration partnership with Celgene, and is largely related to the milestone payment received, as well as expenses incurred by the Company in support of luspatercept.
- **Costs and Expenses** – Total costs and expenses for the second quarter were $48.8 million. This includes R&D expenses of $34.8 million and G&A expenses of $14.0 million.
- **Net Loss** – The Company's net loss for the second quarter ended June 30, 2019 was $17.9 million.

Conference Call and Webcast

The Company will host a webcast and conference call to discuss its second quarter 2019 financial results and provide an update on recent corporate activities on August 5, 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 Second 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 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)

	June 30, 2019	December 31, 2018
Cash and cash equivalents	$ 163,609	$ 144,052
Short and long-term investments	337,307	147,260
Operating lease assets	26,549	—

Other assets	23,403	23,509
Total assets	$ 550,868	$ 314,821
Short-term and long-term operating lease liabilities	$ 29,153	$ —
Warrants to purchase common stock	1,389	1,491
Other liabilities	20,429	21,292
Total liabilities	50,971	22,784
Total stockholders' equity	499,897	292,037
Total liabilities and stockholders' equity	$ 550,868	$ 314,821

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	**2018**	**2019**	**2018**
Revenue:				
Collaboration revenue	$ 27,666	$ 3,685	$ 30,447	$ 6,917
Costs and expenses:				
Research and development	34,765	25,933	67,536	49,363
General and administrative	14,037	7,658	24,851	15,099
Total costs and expenses	48,802	33,591	92,387	64,462
Loss from operations	(21,136)	(29,906)	(61,940)	(57,545)
Total other income, net	3,230	979	6,003	2,410
Loss before income taxes	(17,906)	(28,927)	(55,937)	(55,135)
Income tax benefit (provision)	44	(11)	24	(21)
Net loss applicable to common stockholders- basic and diluted	$ (17,862)	$ (28,938)	$ (55,913)	$ (55,156)
Net loss per share applicable to common stockholders- basic and diluted	$ (0.34)	$ (0.63)	$ (1.08)	$ (1.21)

Weighted-average number of common shares used in computing net loss per share applicable to common stockholders	52,689	45,789	51,912	45,654

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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