Acceleron Reports First Quarter 2018 Operating and Financial Results

May 8, 2018

– Top-line results from the MEDALIST and BELIEVE Phase 3 trials of luspatercept expected in mid-2018 –

– ACE-083 advances into Part 2 of the ongoing Phase 2 trial in facioscapulohumeral muscular dystrophy –

– PULSAR Phase 2 trial with sotatercept in pulmonary arterial hypertension on track to initiate in Q2 2018 –

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 8, 2018-- Acceleron Pharma Inc. (NASDAQ:XLRN), a leading biopharmaceutical company in the discovery and development of TGF-beta therapeutics to treat serious and rare diseases, today provided a corporate update and reported financial results for the first quarter ended March 31, 2018.

“2018 is an important year for our Company, as we prepare for the upcoming top-line results from our MEDALIST and BELIEVE Phase 3 trials with luspatercept expected mid-year. We and Celgene continue to invest heavily in the overall luspatercept opportunity by targeting myelodysplastic syndromes, beta-thalassemia, and myelofibrosis,” said Habib Dable, Chief Executive Officer of Acceleron. “The joint collaboration teams recently kicked off strategic preparations for the anticipated regulatory and commercial activities planned to follow the luspatercept Phase 3 results.”

Added Mr. Dable: “Our team continues to make meaningful progress in the advancement of our wholly-owned clinical programs. In neuromuscular disease, we presented preliminary results from our Phase 2 trial of ACE-083 in FSHD for the first time and recently initiated Part 2 of the trial. In pulmonary, we expect to initiate our first Phase 2 trial with sotatercept in pulmonary arterial hypertension in the second quarter.”

Development Program Highlights

Hematology

Luspatercept:

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

Luspatercept is a first-in-class erythroid maturation agent (EMA) designed to treat the late-stage red blood cell (RBC) maturation defect that results in chronic anemia and the need for regular RBC transfusions in adults with rare blood disorders. Luspatercept is being developed as part of the global collaboration between Acceleron and Celgene.

- Top-line results from the MEDALIST and BELIEVE Phase 3 trials in MDS and beta-thalassemia, respectively, are expected in mid-2018.
- The initiation of the COMMANDS Phase 3 trial in first-line, lower-risk MDS patients is planned for the first half of 2018.
- Enrollment and treatment are ongoing in the BEYOND Phase 2 trial in non-transfusion-dependent beta-thalassemia and the Phase 2 trial in MF.
- The Company expects to provide updates from the ongoing long-term Phase 2 extension trials in MDS and beta-thalassemia at the 2018 American Society of Clinical Oncology (ASCO) Annual Meeting and 23rdCongress of the European Hematology Association (EHA) in June.

Neuromuscular Disease

ACE-083:

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

ACE-083 is a 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 ligands.

- Preliminary results from dose cohorts 1 and 2 in Part 1 of the ACE-083 Phase 2 trial in patients with FSHD were highlighted in a “Best of Neuromuscular Disease” clinical session at the American Academy of Neurology (AAN) 70th Annual Meeting on April 26, 2018.
    - The Company plans to present additional preliminary results from all Part 1 dose cohorts during the second half of 2018.
    - Part 2 of the Phase 2 trial was recently initiated and preliminary results are expected in the second half of 2019.
    - ACE-083 received FDA Fast Track designation in FSHD.
- Enrollment and treatment are ongoing in Part 1 of the Phase 2 trial in patients with CMT disease.
    - The Company plans to present preliminary Part 1 results in the second half of 2018 and to initiate Part 2 of the Phase 2 trial by the end of 2018.

ACE-2494:

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 ligands.

- Enrollment and treatment are ongoing in the Phase 1 healthy volunteer trial.
- Preliminary results from the Phase 1 trial are expected in the first half of 2019.

Pulmonary Disease

Sotatercept:

Pulmonary Arterial Hypertension (PAH)

Sotatercept is a ligand trap for members in the TGF-beta superfamily involved in remodeling a variety of different tissues, including the vasculature and fibrosis. In multiple preclinical studies in PAH, sotatercept decreased vessel muscularization, improved pulmonary arterial pressures, and decreased indicators of right heart failure.

- The Company expects to initiate the PULSAR Phase 2 trial during the second quarter of 2018.
- Preliminary results from the PULSAR Phase 2 trial are expected in the first half of 2020.

Financial Results

- **Cash position** – Cash, cash equivalents and investments as of March 31, 2018 were $353.3 million. As of December 31, 2017, the Company had cash, cash equivalents and investments of $372.9 million. The Company believes that existing cash, cash equivalents and investments will be sufficient to fund projected operating requirements into 2021.
- **Revenue** – Collaboration revenue for the first quarter was $3.2 million. The revenue is all from Acceleron'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 $30.8 million. This includes R&D expenses of $23.4 million and G&A expenses of $7.4 million.
- **Net loss** – The Company's net loss for the first quarter ended March 31, 2018 was $26.2 million.

Conference Call and Webcast

The Company will host a webcast and conference call to discuss its first quarter financial results for 2018 and provide an update on recent corporate activities on May 8, 2018, 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 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 Cambridge-based, 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 pulmonary program with an ongoing Phase 2 trial of 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, 2018	December 31, 2017
Cash and cash equivalents	$ 69,736	$ 100,150
Short and long-term investments	283,607	272,800
Other assets	16,691	16,227
Total assets	$ 370,034	$ 389,177
Deferred revenue	$ —	$ 3,702
Warrants to purchase common stock	1,320	2,236

Other liabilities	14,934	18,021
Total liabilities	16,254	23,960
Total stockholders' equity	353,780	365,217
Total liabilities and stockholders' equity	$ 370,034	$ 389,177

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

	Three Months Ended March 31,	
	2018	**2017**
Revenue:		
Collaboration revenue	$ 3,232	$ 3,705
Costs and expenses:		
Research and development	23,431	21,727
General and administrative	7,441	7,836
Total costs and expenses	30,872	29,563
Loss from operations	(27,640)	(25,858)
Total other income, net	1,431	457
Loss before income taxes	(26,209)	(25,401)
Income tax provision	(10)	(6)
Net loss applicable to common stockholders- basic and diluted	$ (26,219)	$ (25,407)
Net loss per share applicable to common stockholders- basic and diluted	$ (0.58)	$ (0.66)
Weighted-average number of common shares used in computing net loss per share applicable to common stockholders	45,516	38,404

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 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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