Acceleron Reports Second Quarter 2018 Operating and Financial Results

August 2, 2018

– MEDALIST and BELIEVE Phase 3 trials met all primary and key secondary endpoints –

– ACE-083 Part 2 of the Phase 2 trials in facioscapulohumeral muscular dystrophy and Charcot-Marie-Tooth disease are underway –

– PULSAR Phase 2 trial initiated with sotatercept in pulmonary arterial hypertension –

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 2, 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 second quarter ended June 30, 2018.

“We have had a very successful start to 2018, and we look forward to carrying this momentum forward throughout the rest of the year and beyond. We are extremely pleased that luspatercept, our lead product candidate and first-in-class erythroid maturation agent, achieved positive Phase 3 results in both the MEDALIST and BELIEVE trials, confirming our confidence in its clinical profile in myelodysplastic syndromes and beta-thalassemia,” said Habib Dable, President and Chief Executive Officer of Acceleron. “We and our collaboration partner, Celgene, look forward to presenting the Phase 3 data at an upcoming medical congress and are focused on the execution of key regulatory activities, including US and EU application submissions in the first half of 2019. We believe luspatercept is a potential platform treatment to transform the lives of patients suffering from a range of hematologic diseases associated with anemia.”

Added Mr. Dable: “Our neuromuscular and pulmonary programs achieved critical milestones, putting us in a position for important Phase 2 readouts for ACE-083 and sotatercept in 2019 and 2020, respectively.”

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 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 trials in patients with lower-risk MDS and transfusion-dependent beta-thalassemia, respectively, met all primary and key secondary endpoints.
  - Data will be submitted to a future medical meeting for presentation in late 2018.
  - Acceleron and Celgene plan to submit regulatory applications in the United States and Europe in the first half of 2019.
- Updated results from the ongoing Phase 2 trials in MDS and beta-thalassemia were presented at the 2018 American Society of Clinical Oncology (ASCO) Annual Meeting and the 23rdCongress of the European Hematology Association (EHA) in June 2018.
- The initiation of the COMMANDS Phase 3 trial in patients with lower-risk MDS who are treatment naïve is planned for the third quarter 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.

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 Part 1 of the ACE-083 Phase 2 trial in patients with CMT disease were highlighted in oral and poster presentations at the 2018 Peripheral Nerve Society (PNS) Annual Meeting in July.
- Part 2 of the Phase 2 trial in patients with CMT was recently initiated with preliminary results expected by the end of 2019.
- The Company plans to present results from all Part 1 dose cohorts in the FSHD Phase 2 trial in October 2018.
- Enrollment and treatment are ongoing in Part 2 of the Phase 2 FSHD trial with preliminary results expected in the second half of 2019.

- ACE-083 received FDA Fast Track status and Orphan Drug designation in FSHD.

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 with preliminary results expected in the first half of 2019.

Pulmonary Disease

Sotatercept:

Pulmonary Arterial Hypertension (PAH)

Sotatercept acts as a ligand trap for members of the TGF-beta superfamily directly involved in the BMP signaling pathway, which is proven critical for maintaining healthy pulmonary vasculature. In multiple preclinical studies in PAH, sotatercept significantly decreased pulmonary vessel muscularization, improved pulmonary arterial pressures, and decreased indicators of right heart failure.

- The Company initiated the PULSAR Phase 2 trial in patients with PAH with preliminary results expected in the first half of 2020.
- The Company plans to initiate an exploratory imaging study in Q1 2019 to provide additional understanding of endpoints in anticipation of a potential pivotal trial in the future.
- In November 2018, the Company will host a PAH Research and Development Deep Dive event in New York City.
    - The event will include internal and external expert presentations to discuss disease background, the current treatment landscape, key disease pathways including BMP signaling, Acceleron's clinical development activities, and the latest sotatercept preclinical results.

Corporate Highlights

- Robert K. Zeldin, M.D., was appointed Chief Medical Officer (CMO). He brings 20 years of industry experience and joined from Ablynx where he served as Chief Medical Officer. Prior to Ablynx, Dr. Zeldin served in senior roles at Novartis, Merck, and the FDA Center for Biologics Evaluation and Research.
- Janethe Pena, M.D., Ph.D., recently joined the Company as Vice President of Pulmonary to lead the company's clinical development efforts in this area. Dr. Pena most recently served asVice President and Group Head of Pulmonology Clinical Development at Bayer Pharmaceuticals. At Bayer, she was responsible for the pulmonary portfolio, including leading clinical trials with riociguat (Adempas®) in different pulmonary hypertension indications and life cycle management for the program.

Financial Results

- **Cash position** – Cash, cash equivalents and investments as of June 30, 2018 were $332.3 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 second quarter was $3.7 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 second quarter were $33.6 million. This includes R&D expenses of $25.9 million and G&A expenses of $7.7 million.
- **Net loss** – The Company's net loss for the second quarter ended June 30, 2018 was $28.9 million.

Conference Call and Webcast

The Company will host a webcast and conference call to discuss its second quarter financial results for 2018 and provide an update on recent corporate activities on August 2, 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 Second 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 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, 2018	December 31, 2017
Cash and cash equivalents	$ 79,592	$ 100,150
Short and long-term investments	252,666	272,800
Other assets	19,283	16,227
Total assets	$ 351,541	$ 389,177
Deferred revenue	$ —	$ 3,702
Warrants to purchase common stock	1,689	2,236
Other liabilities	16,743	18,021
Total liabilities	18,432	23,960
Total stockholders' equity	333,109	365,217
Total liabilities and stockholders' equity	$ 351,541	$ 389,177

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,	
	2018	2017	2018	2017
Revenue:				
Collaboration revenue	$ 3,685	$ 3,057	$ 6,917	$ 6,762
Costs and expenses:				
Research and development	25,933	21,598	49,363	43,327
General and administrative	7,658	11,370	15,099	19,203
Total costs and expenses	33,591	32,968	64,462	62,530
Loss from operations	(29,906)	(29,911)	(57,545)	(55,768)
Total other income, net	979	248	2,410	705
Loss before income taxes	(28,927)	(29,663)	(55,135)	(55,063)
Income tax provision	(11)	(6)	(21)	(12)
Net loss applicable to common stockholders- basic and diluted	$ (28,938)	$ (29,669)	$ (55,156)	$ (55,075)
Net loss per share applicable to common stockholders- basic and diluted	$ (0.63)	$ (0.77)	$ (1.21)	$ (1.43)
Weighted-average number of common shares used in computing net loss per share applicable to common stockholders	45,789	38,631	45,654	38,515

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 will be unable to successfully complete the clinical development of the Company's compounds, that the Company 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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