



## Acceleron Reports Third Quarter 2018 Operating and Financial Results

October 30, 2018

– MEDALIST and BELIEVE Phase 3 trial results in myelodysplastic syndromes (MDS) and beta-thalassemia, respectively, are expected to be presented at the 60<sup>th</sup> American Society of Hematology (ASH) Annual Meeting –

– COMMANDS Phase 3 trial in patients with lower-risk MDS who are treatment naïve has been initiated –

– Part 1 results from the ACE-083 Phase 2 trial in facioscapulohumeral muscular dystrophy (FSHD) and Charcot-Marie-Tooth disease (CMT) were presented at the World Muscle Society Annual Meeting –

– Pulmonary Arterial Hypertension (PAH) R&D Deep Dive Event scheduled for Friday, November 16<sup>th</sup> in New York City –

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Oct. 30, 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 third quarter ended September 30, 2018.

"2018 is proving to be a pivotal year for Acceleron, and with several key milestones approaching, we are well-positioned for future growth. Along with our global collaboration partner, Celgene, we expect to share results from the MEDALIST and BELIEVE Phase 3 trials of luspatercept with the global hematology community at the upcoming ASH meeting in December. With the recent initiation of the COMMANDS Phase 3 trial, we now have ongoing clinical trials with luspatercept in three additional patient populations. We remain committed to further exploring luspatercept's potential to be a platform treatment for a range of anemias," said Habib Dable, President and Chief Executive Officer of Acceleron. "Our neuromuscular and pulmonary teams have done a tremendous job in getting multiple Phase 2 trials underway for ACE-083 in patients with FSHD and CMT, and sotatercept in patients with PAH. Both programs will have multiple important inflection points over the next two years."

### 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 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, are expected to be presented at the 60<sup>th</sup> ASH Annual Meeting and Exposition in December 2018.
  - Acceleron and Celgene plan to submit regulatory applications for both MDS and beta-thalassemia in the United States and Europe in the first half of 2019.
- The COMMANDS Phase 3 trial in patients with lower-risk MDS who are treatment naïve has been initiated.
- Enrollment is ongoing in the BEYOND Phase 2 trial in patients with non-transfusion-dependent beta-thalassemia as well as the Phase 2 trial in patients with MF.

#### Neuromuscular Disease

##### ACE-083:

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

*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 involved in muscle formation.*

- Final results from Part 1 of each Phase 2 trial with ACE-083 in patients with FSHD and CMT, respectively, were presented at the 2018 World Muscle Society (WMS) Annual Meeting.
- Enrollment is ongoing in Part 2 of the Phase 2 FSHD trial, with preliminary 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:

*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 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 ligand trap for members of the TGF-beta superfamily that control the BMPRII signaling pathway, which is 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.*

- Multiple preclinical abstracts for sotatercept in PAH have been accepted for presentation at the American Heart Association Scientific Sessions on November 10-12, 2018.
- On November 16, 2018, the Company will host a PAH Research and Development Deep Dive event in New York City.
- Enrollment is ongoing in 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 study, called SPECTRA, in Q1 2019 to provide further understanding of sotatercept's impact on the disease.

#### **Financial Results**

- **Cash position** – Cash, cash equivalents and investments as of September 30, 2018 were \$319.8 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 third quarter was \$3.3 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 third quarter were \$33.4 million. This includes R&D expenses of \$24.7 million and G&A expenses of \$8.7 million.
- **Net loss** – The Company's net loss for the third quarter ended September 30, 2018 was \$29.0 million.

#### **Conference Call and Webcast**

The Company will host a webcast and conference call to discuss its third quarter financial results for 2018 and provide an update on recent corporate activities on October 30, 2018, at 4:30 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](http://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 Third 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](http://www.acceleronpharma.com). Follow Acceleron on Social Media: [@AcceleronPharma](https://twitter.com/AcceleronPharma) and [LinkedIn](https://www.linkedin.com/company/acceleronpharma).

#### **ACCELERON PHARMA INC.**

#### **CONDENSED CONSOLIDATED BALANCE SHEET**

(Amounts in thousands)

(unaudited)

	<b>September 30, 2018</b>	<b>December 31, 2017</b>
Cash and cash equivalents	\$ 112,427	\$ 100,150
Short and long-term investments	207,380	272,800
Other assets	18,310	16,227
Total assets	\$ 338,117	\$ 389,177
Deferred revenue	\$ —	\$ 3,702
Warrants to purchase common stock	2,031	2,236

Other liabilities	16,974	18,021
Total liabilities	19,005	23,960
Total stockholders' equity	319,112	365,217
Total liabilities and stockholders' equity	\$ 338,117	\$ 389,177

**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,	
	2018	2017	2018	2017
Revenue:				
Collaboration revenue	\$ 3,258	\$ 3,014	\$ 10,175	\$ 9,776
Costs and expenses:				
Research and development	24,667	21,059	74,027	64,387
General and administrative	8,653	7,533	23,756	26,735
Total costs and expenses	33,320	28,592	97,783	91,122
Loss from operations	(30,062 )	(25,578 )	(87,608 )	(81,346 )
Total other income, net	1,071	86	3,481	791
Loss before income taxes	(28,991 )	(25,492 )	(84,127 )	(80,555 )
Income tax benefit (provision)	12	41	(9 )	29
Net loss applicable to common stockholders- basic and diluted	\$ (28,979 )	\$ (25,451 )	\$ (84,136 )	\$ (80,526 )
Net loss per share applicable to common stockholders- basic and diluted	\$ (0.63 )	\$ (0.65 )	\$ (1.84 )	\$ (2.08 )
Weighted-average number of common shares used in computing net loss per share applicable to common stockholders	46,051	39,361	45,787	38,804

**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 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

Acceleron Pharma Inc.  
Todd James, IRC, 617-649-9393  
Vice President, Investor Relations and Corporate Communications  
Or  
Candice Ellis, 617-649-9226  
Manager, Investor Relations and Corporate Communications

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