November 7, 2017

Acceleron Pharma Reports Third Quarter 2017 Operational and Financial Results

-Presented vision and strategic focus in hematological, neuromuscular, and pulmonary diseases at September R&D Day-

-Obtained the rights to fund, develop, and lead global commercialization of sotatercept in pulmonary arterial hypertension; robust preclinical results show potential for first-in-class disease modifying approach-

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- 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, 2017.

"As we think about and plan to execute on our long-term vision and strategy, there were several significant corporate events in the third quarter. At our September R&D day, we outlined our core research and development focus in three disease areas of high unmet medical need: hematology, neuromuscular, and pulmonary disease. We announced that we gained rights to sotatercept, an internally discovered Phase 2 asset, for the development in pulmonary arterial hypertension. We also completed a successful equity offering that will provide sufficient funding through key inflection points in each of our clinical programs," said Habib Dable, President and Chief Executive Officer of Acceleron. "We and our partner Celgene continue to invest heavily in our luspatercept development plan with seven clinical trials expected to be ongoing in 2018. In neuromuscular diseases, ACE-083 continues to advance in the Phase 2 trials in FSHD and CMT, and we remain on track to launch a Phase 2 trial with sotatercept in the first half of 2018 as we work to grow our pulmonary franchise, and ultimately deliver transformative treatment options to patients in need."

Development Program Highlights

Hematology

Luspatercept:

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

Luspatercept is designed to treat chronic anemia and reduce red blood cell (RBC) transfusion burden in adults with rare blood disorders. Luspatercept is being developed as part of the global collaboration between Acceleron and Celgene.

- In addition to the ongoing MEDALIST and BELIEVE Phase 3 trials, Acceleron and Celgene continue to prepare for clinical trial expansion in new patient populations, including the COMMANDS Phase 3 trial in first-line, lower-risk MDS, regardless of ring sideroblast status, the BEYOND Phase 2 trial in non-transfusion-dependent beta-thalassemia, and the Phase 2 trial in myelofibrosis.
- Results from the Phase 2 trial of luspatercept for the treatment of anemia in patients with lower-risk MDS were recently published in *The Lancet Oncology*.
- Data from two clinical abstracts on luspatercept and sotatercept will be presented at the 59th American Society of Hematology (ASH) Annual Meeting and Exposition in Atlanta, GA on December 9-12, 2017.

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 debilitating focal muscle loss by utilizing the "Myostatin+" approach to inhibit multiple TGF-beta ligands.

- Enrollment and treatment are ongoing in Part 1 of the Phase 2 trial in patients with FSHD, one of the most prevalent forms of muscular dystrophy in adults.

- Enrollment and treatment are ongoing in Part 1 of the Phase 2 trial in patients with CMT disease, one of the most common inherited neurological diseases leading to focal muscle weakness.

ACE-2494:

ACE-2494 is a protein therapeutic designed to have a systemic effect on muscle mass and strength throughout the body by utilizing the "Myostatin+" approach to inhibit multiple TGF-beta ligands.

- The Company plans to initiate a Phase 1 healthy volunteer clinical trial this year and is actively evaluating a potential first indication.

Pulmonary Disease

Sotatercept:

Sotatercept is an activin receptor type IIA fusion protein that acts as a ligand trap for members in the TGF-beta protein superfamily involved in remodeling and regeneration of a variety of different tissues, including the vasculature and fibrotic tissue.

- Acceleron gained development and commercialization rights for pulmonary arterial hypertension (PAH).
- Preclinical results presented at R&D day show potential for sotatercept to be a first-in-class disease-modifying therapy that addresses fundamental molecular causes of disease in PAH.
- Preclinical results of sotatercept in PAH will be highlighted in an oral presentation at the American Heart Association Scientific Sessions 2017 in Anaheim, CA on November 14, 2017.

Key Corporate Priorities

Luspatercept

- Report top-line results from MEDALIST and BELIEVE Phase 3 trials in mid-2018
- Initiate the COMMANDS Phase 3 trial in first-line, lower-risk MDS in 1H 2018
- Enroll the first myelofibrosis patient in Phase 2 by YE 2017
- Initiate the BEYOND Phase 2 trial in non-transfusion-dependent beta-thalassemia by YE 2017

ACE-083

- Report FSHD Phase 2 results for cohort 1 in Part 1 in January 2018
- Report FSHD Phase 2 results for all dose-escalation cohorts in Part 1 in 2018
- Report CMT Phase 2 results from all dose-escalation cohorts in Part 1 by YE 2018

ACE-2494

- Initiate Phase 1 healthy volunteer trial in 2017

Sotatercept

- Initiate Phase 2 trial in PAH first half of 2018

Financial Results

- **Cash position** - Cash, cash equivalents and investments as of September 30, 2017 were $366.6 million. As of December 31, 2016 the Company had cash, cash equivalents and investments of $234.4 million. Cash, cash equivalents and investments include $187.6 million of net proceeds from a follow-on public offering of common stock in September 2017. In October 2017, the underwriters exercised the over-allotment option in the offering which resulted in additional net proceeds of $28.2 million. We believe that existing cash, cash equivalents and investments, including the net proceeds from the offering and the exercise of the underwriters' over-allotment option, will be sufficient to fund projected operating requirements into 2021.

- **Revenue** - Collaboration revenue for the third quarter was $3.0 million. The revenue is all from our Celgene partnership and is primarily due to cost sharing revenue of $2.9 million related to expenses incurred by the Company in support of our partnered programs.
- **Costs and expenses** - Total costs and expenses for the third quarter were $28.6 million. This includes R&D expenses of $21.1 million and G&A expenses of $7.5 million.
- **Net loss** - The Company's net loss for the third quarter ended September 30, 2017 was $25.5 million.

Conference Call and Webcast

The Company will host a webcast and conference call to discuss its third quarter 2017 financial results and provide an update on recent clinical development and corporate activities on November 7, 2017, at 8:00 a.m. EST.

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 refer to the "Acceleron Third Quarter 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 a Phase 2 trial of sotatercept planned 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)

	September 30, 2017	December 31, 2016
Cash and cash equivalents	$ 238,959	$ 20,950
Short and long-term investments	127,638	213,432
Other assets	14,680	13,265
Total assets	$ 381,277	$ 247,647
Deferred revenue	$ 3,838	$ 4,245
Warrants to purchase common stock	1,927	1,244
Other liabilities	16,971	16,561
Total liabilities	22,736	22,050
Total stockholders' equity	358,541	225,597
Total liabilities and stockholders' equity	$ 381,277	$ 247,647

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,	
	2017	2016	2017	2016
Revenue:				
Collaboration revenue	$ 3,014	$ 3,005	$ 9,776	$ 24,402
Costs and expenses:				
Research and development	21,059	17,102	64,387	49,492
General and administrative	7,533	6,411	26,735	19,029
Total costs and expenses	28,592	23,513	91,122	68,521
Loss from operations	(25,578)	(20,508)	(81,346)	(44,119)
Total other income (expense) net	86	(282)	791	6,374
Loss before income taxes	(25,492)	(20,790)	(80,555)	(37,745)
Income tax benefit	41	20	29	20
Net loss applicable to common stockholders - basic and diluted	$ (25,451)	$ (20,770)	$ (80,526)	$ (37,725)
Net loss per share applicable to common stockholders - basic and diluted	$ (0.65)	$ (0.55)	$ (2.08)	$ (1.01)
Weighted-average number of common shares used in computing net loss per share applicable to common stockholders	39,361	37,616	38,804	37,268

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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