March 1, 2017

Acceleron Reports Fourth Quarter and Year Ended 2016 Operational and Financial Results

- Luspatercept Phase 3 trials to complete enrollment in both the MDS ("MEDALIST") and beta-thalassemia ("BELIEVE") trials in 2H 2017 -

- Presented data from five abstracts on luspatercept and sotatercept at the American Society of Hematology Annual Meeting in December -

- Treated first patient in ACE-083 Phase 2 study for facioscapulohumeral muscular dystrophy -

- Outlined plans to initiate several new clinical trials in 2017 -

- Maintained strong balance sheet with $234.4 million in cash and investments to fund projected operating requirements into the second half of 2019 -

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Acceleron Pharma Inc. (NASDAQ: XLRN), a clinical stage biopharmaceutical company focused on the discovery, development and commercialization of innovative therapeutics to treat serious and rare diseases, today provided a corporate update and reported financial results for the fourth quarter and year ended December 31, 2016.

"Acceleron made tremendous progress in 2016 across our entire portfolio of potentially first- and best-in-class therapies for patients with serious hematologic, neuromuscular and fibrotic diseases without effective treatments," said Habib Dable, President and Chief Executive Officer of Acceleron. "The pace of recruitment continues to accelerate in both of our ongoing Phase 3 luspatercept trials, and we are highly confident that Celgene will complete enrollment in the second half of 2017. Furthermore, we and Celgene plan to expand the program by initiating new Phase 2 trials in non-transfusion dependent beta-thalassemia and myelofibrosis patient populations highlighting our confidence in the blockbuster potential of luspatercept. With encouraging preliminary results and multiple ongoing studies across all of our programs, we are well positioned for a transformational year in 2017."

DEVELOPMENT PROGRAM HIGHLIGHTS

Hematology

Luspatercept in myelodysplastic syndromes (MDS)

Luspatercept is being developed to treat anemia and reduce or eliminate the need for red blood cell transfusions in patients with MDS, a rare disorder in which bone marrow fails to produce enough healthy blood cells. Luspatercept is being developed as part of the global collaboration between Acceleron and Celgene.

- **Enrollment is robust in the MEDALIST Phase 3 clinical trial, with completion expected in the second half of this year**. The MEDALIST Phase 3 study is enrolling 210 patients to evaluate the efficacy and safety of luspatercept in patients with anemia due to lower-risk MDS with ring sideroblasts who require red blood cell (RBC) transfusions.
- **Results presented at ASH 2016 from an ongoing Phase 2 extension study in lower-risk MDS patients confirmed previously reported results showing durable safety, RBC transfusion independence** and erythroid response per the International Working Group's (IWG HI-E) criteria. Data were presented showing increases in hemoglobin sustained for as long as 22 months with treatment ongoing.
- **Initial data reported at ASH in first-line lower-risk MDS patients from an ongoing Phase 2 trial demonstrated encouraging response rates.** The Company plans to present additional results at medical conferences in 2017. Acceleron and Celgene continue to evaluate a clinical and regulatory strategy for luspatercept in this MDS patient segment.

Luspatercept in beta-thalassemia

Luspatercept is designed to treat severe, chronic anemia and reduce RBC transfusion-dependence in adults with beta-thalassemia, a rare genetic disorder that reduces the production of hemoglobin. Luspatercept is being developed as part of the global collaboration between Acceleron and Celgene.

- **Enrollment is robust in the BELIEVE Phase 3 trial, with completion expected in the second half of this year**. The BELIEVE Phase 3 study is enrolling 300 patients to evaluate the efficacy and safety of luspatercept in patients with anemia due to beta-thalassemia who require regular RBC transfusions.
- **The Company presented data at ASH 2016 from its ongoing Phase 2 extension study showing clinically meaningful reductions in RBC transfusion burden compared to baseline in transfusion dependent beta-thalassemia patients.** Data presented in non-transfusion dependent (NTD) beta-thalassemia patients showed durable increases in hemoglobin sustained for as long as 18 months with treatment ongoing.
- **Acceleron and Celgene plan to initiate a new Phase 2 trial in non-transfusion dependent beta-thalassemia patients by the end of the year.**

Luspatercept and sotatercept in myelofibrosis

Myelofibrosis is a malignant disease which results in fibrotic bone marrow, multiple cytopenias and severe anemia often requiring RBC transfusions. Luspatercept is being developed as part of the global collaboration between Acceleron and Celgene.

- **Preliminary data reported at ASH 2016 from an ongoing investigator-initiated Phase 2 study with sotatercept showed that 36% (5/14) of evaluable myelofibrosis patients achieved an anemia response,** defined as a composite of RBC transfusion independence and hemoglobin response.
- **Acceleron and Celgene plan to initiate a Phase 2 trial in myelofibrosis with luspatercept by year-end.**

Neuromuscular Disease

ACE-083 in facioscapulohumeral muscular dystrophy (FSHD)

ACE-083 is designed to increase muscle mass and strength in target muscles for diseases that cause debilitating focal muscle loss.

- **First patient was treated in the ACE-083 Phase 2 study in patients with FSHD**, one of the most prevalent forms of muscular dystrophy.

Oncology

Dalantercept in Advanced Renal Cell Carcinoma

Dalantercept is being developed in combination with axitinib to further inhibit tumor angiogenesis.

- **Enrollment continues in randomized Part 2 of the Phase 2 DART study of dalantercept in combination with axitinib.** The primary endpoint of this trial, progression-free survival (PFS), is an event-driven assessment with preliminary data expected in the second half of 2017.

Preclinical Research

Acceleron continues its research on several molecules targeting musculoskeletal diseases, fibrotic disorders and other serious diseases.

- The Company plans to initiate a Phase 1 healthy volunteer clinical trial this year with ACE-2494.
- IND-enabling development work has begun to advance a new therapeutic candidate to the clinic in 2018.

OTHER CORPORATE UPDATES

- **Habib Dable appointed as President and CEO effective December 1, 2016.** Mr. Dable joined Acceleron from Bayer AG, where he most recently served as President of U.S. Pharmaceuticals.

NEWSFLOW AND CATALYSTS

Luspatercept

- Complete enrollment in the MEDALIST Phase 3 trial in 2H 2017
- Complete enrollment in the BELIEVE Phase 3 trial in 2H 2017
- Develop clinical and regulatory strategy in first-line lower-risk MDS in 2017
- Initiate new Phase 2 trials in myelofibrosis and NTD beta-thalassemia by YE 2017
- Additional Phase 2 extension study data to be presented at medical conferences in 2017

ACE-083

- Initial FSHD Phase 2 Part 1 dose-escalation results by late 2017
- Initiate a Phase 2 clinical trial in a second neuromuscular disease in 2017

Dalantercept

- Report topline results in the DART Phase 2 trial in renal cell carcinoma in 2H 2017

ACE-2494

- Initiate Phase 1 healthy volunteer study in 2017

Research

- Host an investor and analyst research day to discuss ongoing preclinical research and potential future disease areas in 2017

FINANCIAL RESULTS

- **Cash Position** - Cash, cash equivalents and investments were $234.4 million as of December 31, 2016. We believe that our existing cash, cash equivalents and investments will be sufficient to fund our projected operating requirements into the second half of 2019.
- **Revenue** - Collaboration revenue for the year was $27.8 million. The revenue is all from our Celgene partnership. It includes license and milestone revenue of $15.6 million and cost sharing revenue of $12.2 million related to expenses incurred by the Company in support of our partnered programs.
- **Costs and expenses** - Total costs and expenses for the year were $93.9 million. This includes R&D expenses of $68.6 million and G&A expenses of $25.3 million.
- **Other Income -** Other income, net was $9.1 million and includes a $7.3 million, non-cash, gain on marking the Company's common stock warrant liability to market and $1.8 million in interest income.
- **Net Loss** - The Company's net loss for the year ended December 31, 2016 was $57.0 million.

Conference Call and Webcast Information

The Company will host a live conference call and webcast to discuss its fourth quarter and full year financial results for 2016 and provide a corporate update on Wednesday, March 1, 2017, at 8:00 AM EST. Participants can access the live conference call by dialing 877-312-5848 (domestic) or 253-237-1155 (international) and refer to the "Acceleron Earnings Call."

The live webcast can be accessed on the Investors page of the Company's website at www.acceleronpharma.com.

A replay of the webcast will be archived on the Company's website and accessible approximately two hours after the event.

About Acceleron

Acceleron is a clinical stage biopharmaceutical company focused on the discovery, development and commercialization of innovative therapeutics to treat serious and rare diseases. Its pioneering research platform leverages the powerful biology behind the body's ability to rebuild and repair its own cells and tissues. This approach to drug discovery has generated four

therapeutic candidates that are currently in clinical trials. The Company's lead therapeutic candidate, luspatercept, is being evaluated in Phase 3 studies for the treatment of the hematologic diseases, myelodysplastic syndromes (MDS) and beta-thalassemia under a global partnership with Celgene Corp. Acceleron is also advancing clinical programs in the fields of oncology and neuromuscular diseases and has a comprehensive preclinical research effort targeting fibrotic and other serious diseases.

For more information, please visit www.acceleronpharma.com.

ACCELERON PHARMA INC.
CONDENSED CONSOLIDATED BALANCE SHEET
(Amounts in thousands)
(unaudited)

	December 31, 2016	December 31, 2015
Cash and cash equivalents	$ 20,950	$ 27,783
Short and long-term investments	213,432	108,198
Other assets	13,265	10,356
Total assets	$ 247,647	$ 146,337
Deferred revenue	$ 4,245	$ 4,794
Warrants to purchase common stock	1,244	17,187
Other liabilities	16,561	15,093
Total liabilities	22,050	37,074
Total stockholders' equity	225,597	109,263
Total liabilities and stockholders' equity	$ 247,647	$ 146,337

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

	Three Months Ended December 31,		Year Ended December 31,	
	2016	2015	2016	2015
Revenue:				
Collaboration revenue	$ 3,369	$ 3,804	$ 27,771	$ 18,097
Costs and expenses:				
Research and development	19,088	16,146	68,580	58,404
General and administrative	6,267	5,773	25,297	20,572
Total costs and expenses	25,355	21,919	93,877	78,976
Loss from operations	(21,986)	(18,115)	(66,106)	(60,879)
Other income (expense), net	2,742	(8,967)	9,116	(3,015)
Loss before income taxes	(19,244)	(27,082)	(56,990)	(63,894)
Income tax provision	(44)	—	(24)	—
Net loss	$ (19,288)	$ (27,082)	$ (57,014)	$ (63,894)
Other comprehensive loss:				
Net unrealized holding losses on short-term and long-term investments during the period	(83)	(199)	(205)	(220)
Comprehensive loss	$ (19,371)	$ (27,281)	$ (57,219)	$ (64,114)
Net loss per share - basic and diluted	$ (0.51)	$ (0.81)	$ (1.52)	$ (1.92)
Weighted-average number of common shares used in computing net loss per share - basic and diluted	37,914	33,268	37,430	33,303

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, including sotatercept, luspatercept, dalantercept, ACE-083, ACE-2494, the Company's IntelliTrap™ drug discovery platform, and the Company's TGF-beta superfamily program generally, the timeline for clinical development and regulatory approval of the Company's compounds, the expected timing for the reporting of data from ongoing trials, and the structure of the Company's planned or pending clinical trials. The words "anticipate," "appear," "believe," "continue," "could," "estimate," "expect," "forecast," "goal," "intend," "may," "plan," "potential," "predict," "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.

Each forward-looking statement is subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statement. Applicable risks and uncertainties include the risks that the Company's cash, cash equivalents and investments will be insufficient to fund operations into the second half of 2019, 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 data may not be available when the Company expects it to be, that the Company or its collaboration partner, Celgene, will be unable to successfully complete the clinical development of the Company's compounds, that the development of the Company's compounds will take longer or cost more than planned, that the Company or Celgene may be delayed in initiating, enrolling or completing any clinical trials, that the Company's drug discovery activities may not yield drug candidates for which the Company can commence clinical trials at the rate at which the Company currently anticipates or at all, and that the Company's compounds will not receive regulatory approval or become commercially successful products.

Other risks and uncertainties include those identified under the heading "Risk Factors" included in the Company's Annual Report on Form 10-K which was filed with the Securities and Exchange Commission (SEC) on March 1, 2017, 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 reflect the Company's current views 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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