November 3, 2016

Acceleron Pharma Reports Third Quarter 2016 Financial and Operational Results

- Appointed Habib Dable as President and CEO effective December 1, 2016 -

- Five upcoming presentations at American Society of Hematology Annual Meeting in December -

- Advancing ACE-083 into Phase 2 study for facioscapulohumeral muscular dystrophy (FSHD) -

- Maintained strong balance sheet with $251 million in cash 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 third quarter ended September 30, 2016.

"In the third quarter, we continued to make strong progress across our pipeline of clinical and preclinical programs," said John Knopf, Ph.D., Chief Executive Officer of Acceleron. "Enrollment continues to go well in both of the ongoing luspatercept Phase 3 studies in patients with myelodysplastic syndromes and beta-thalassemia. We look forward to providing new and updated preliminary results from several Phase 2 studies at the upcoming American Society of Hematology annual meeting in December. Within our muscle program, we're on track to initiate the ACE-083 Phase 2 study in patients with facioscapulohumeral muscular dystrophy before year-end."

Continued Dr. Knopf, "I would also like to extend a warm welcome to the Company's newly appointed Chief Executive Officer, Habib Dable, who will assume this role on December 1st. Habib's global pharmaceutical operational and commercial experience will serve Acceleron well as we advance toward becoming a commercial-stage company."

THIRD QUARTER 2016 HIGHLIGHTS

Development Programs

Hematology

- **Enrollment is ongoing in the pivotal luspatercept Phase 3 clinical trials** for the treatment of myelodysplastic syndromes (MDS) patients (the MEDALIST study) and beta-thalassemia patients (the BELIEVE study).
- **Enrollment is ongoing in Phase 2 trial cohorts** evaluating luspatercept in a first-line treatment setting in low- or intermediate-risk MDS patients.
- **Data from five clinical abstracts on luspatercept and sotatercept will be presented at the 58th American Society of Hematology (ASH) Annual Meeting and Exposition** to be held in San Diego, California on December 3-6, 2016.

Neuromuscular Disease

- **Initiation of the ACE-083 Phase 2 study in facioscapulohumeral muscular dystrophy (FSHD) patients** expected to start by the end of the year.

Oncology

- **Enrollment continues in randomized part 2 of the Phase 2 DART study of dalantercept in combination with axitinib in patients with advanced renal cell carcinoma (RCC).** The primary endpoint of this trial, progression-free survival (PFS), is an event-driven assessment with preliminary data expected in the first half of 2017.

Preclinical Research and Development

- **Acceleron continues its research on several molecules targeting musculoskeletal diseases, fibrotic disorders and other serious diseases.** The most advanced molecule from the IntelliTrap™ platform, ACE-2494, continues to advance through IND-enabling activities. The Company plans to initiate a Phase 1 clinical trial with ACE-2494 in mid-2017.

Other Corporate Updates

- **Habib Dable appointed as President and CEO effective December 1, 2016.** Mr. Dable joins Acceleron from Bayer AG, where he most recently served as President of U.S. Pharmaceuticals.
- **John Knopf retiring as President and CEO.** Dr. Knopf is expected to apply his scientific expertise as an advisor to the Company.

Upcoming Events in Q4 2016

- **Present data at the ASH annual meeting from ongoing luspatercept Phase 2 studies** and an ongoing sotatercept Phase 2 investigator initiated trial in patients with myelofibrosis.
- **Initiate ACE-083 Phase 2 study in patients with FSHD,** one of the most prevalent forms of muscular dystrophy.
- **Provide sotatercept development plan update before year-end.**
- **Present at the 25th Annual Credit Suisse Healthcare Conference** on Monday, November 7, 2016 in Scottsdale, Arizona.
- **Participate at the Citi Global Healthcare Conference** to be held on December 7-8, 2016 in New York.

Financial Results

- **Cash position** - Cash, cash equivalents and investments as of September 30, 2016 were $251.0 million. As of December 31, 2015 the Company had cash, cash equivalents and investments of $136.0 million. The Company believes that existing cash, cash equivalents and investments will be sufficient to fund its projected operating requirements into the second half of 2019.
- **Revenue** - Collaboration revenue for the third quarter was $3.0 million. Cost sharing reimbursement revenue from the Company's Celgene partnership was $2.9 million and is related to expenses incurred by the Company in support of its partnered programs.
- **Costs and expenses** - Total costs and expenses for the third quarter were $23.5 million. This includes R&D expenses of $17.1 million and G&A expenses of $6.4 million.
- **Other expense, net** - Other expense for the third quarter was $0.3 million and includes a $0.8 million, non-cash, loss on marking the Company's common stock warrant liability to market.
- **Net loss** - The Company posted a net loss for the third quarter ended September 30, 2016 of $20.8 million.

Conference Call and Webcast

The Company will host a live conference call and webcast to discuss its third quarter 2016 financial results and provide a corporate update on November 3, 2016, at 5:00 p.m. EDT. 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 available approximately two hours after the event on the Company's website.

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. Follow Acceleron on Social Media: @AcceleronPharma and LinkedIn.

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

	September 30, 2016	December 31, 2015
Cash and cash equivalents	$ 31,016	$ 27,783
Short and long-term investments	220,007	108,198
Other assets	11,862	10,356
Total assets	$ 262,885	$ 146,337
Deferred revenue	4,380	4,794
Warrants to purchase common stock	12,161	17,187
Other liabilities	16,797	15,093
Total liabilities	33,338	37,074
Total stockholders' equity	229,547	109,263
Total liabilities and stockholders' equity	$ 262,885	$ 146,337

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,	
	2016	2015	2016	2015
Revenue:				
Collaboration revenue:				
Collaboration revenue	$ 3,005	$ 4,155	$ 24,402	$ 14,294
Costs and expenses:				
Research and development	17,102	13,335	49,492	42,261
General and administrative	6,411	5,433	19,029	14,796
Total costs and expenses	23,513	18,768	68,521	57,057
Loss from operations	(20,508)	(14,613)	(44,119)	(42,763)
Total other (expense) income, net	(282)	2,755	6,374	5,951
Loss before income taxes	(20,790)	(11,858)	(37,745)	(36,812)
Income tax benefit	20	—	20	—
Net loss applicable to common stockholders—basic and diluted	$ (20,770)	$ (11,858)	$ (37,725)	$ (36,812)
Net loss per share applicable to common stockholders - basic and diluted	$ (0.55)	$ (0.36)	$ (1.01)	$ (1.12)
Weighted-average number of common shares used in computing net loss per share applicable to common stockholders - basic and diluted	37,616	33,097	37,268	32,869

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," "believe," "continue," "could," "estimate," "expect," "forecast," "goal," "intend," "may," "plan," "potential," "project," "should," "strategy," "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 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 February 25, 2016, 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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