May 8, 2017

Acceleron Pharma Reports First Quarter 2017 Operational and Financial Results

- Luspatercept MEDALIST and BELIEVE Phase 3 trials expected to complete enrollment in Q2 2017 -

- Presented results from ongoing Phase 2 trial with luspatercept at the 14th International Symposium on MDS -

- Announced plans to initiate luspatercept Phase 3 trial in first-line, lower-risk MDS in early 2018 -

- Announced plans to initiate ACE-083 Phase 2 study in Charcot-Marie-Tooth disease -

- Maintained strong balance sheet with $213.2 million in cash, cash equivalents 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 first quarter ended March 31, 2017.

"In the first quarter, we made significant progress across our entire pipeline. Our luspatercept program in MDS and beta-thalassemia continues to build momentum, as we look to complete enrollment of the MEDALIST and BELIEVE Phase 3 studies ahead of schedule, and expand into additional patient populations where there are limited treatment options, including first-line, lower-risk MDS and myelofibrosis," said Habib Dable, President and Chief Executive Officer of Acceleron. "As we continue to explore further expansion opportunities for luspatercept, we are also preparing to expand our wholly-owned pipeline into a second neuromuscular disease, with the expected initiation by mid-year of a Phase 2 study with ACE-083 in patients with Charcot-Marie-Tooth disease."

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 in the MEDALIST Phase 3 clinical trial is expected to complete in the second quarter 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 the 14th International Symposium on MDS from an ongoing Phase 2 study in first-line, lower-risk MDS patients demonstrated positive erythroid response and RBC transfusion independence rates.**
- **Acceleron and Celgene announced plans to initiate a third Phase 3 trial with luspatercept in early 2018.** The new Phase 3 trial will evaluate luspatercept treatment versus standard-of-care in the first-line treatment setting for lower-risk MDS patients.

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 in the BELIEVE Phase 3 trial is expected to complete in the second quarter 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.

Neuromuscular Disease

ACE-083 in facioscapulohumeral muscular dystrophy (FSHD) and Charcot-Marie-Tooth (CMT) disease

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

- **Enrollment and treatment are ongoing in Part 1 of the ACE-083 Phase 2 study in patients with FSHD**, one of the most prevalent forms of muscular dystrophy.
- **Announced plans to initiate a second Phase 2 clinical trial with ACE-083 in patients with Charcot-Marie-Tooth (CMT) disease,** one of the most common inherited neurologic diseases leading to focal muscle weakness, by mid-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.

NEWSFLOW AND CATALYSTS

Luspatercept

- Complete enrollment in the MEDALIST Phase 3 trial in Q2 2017
- Complete enrollment in the BELIEVE Phase 3 trial in Q2 2017
- Anticipate top-line data from both Phase 3 trials in mid-2018
- Initiate first-line, lower-risk MDS Phase 3 trial in early 2018
- Initiate Phase 2 trial in myelofibrosis by year-end 2017
- Initiate Phase 2 trial in non-transfusion dependent (NTD) beta-thalassemia by year-end 2017
- Additional Phase 2 results from ongoing trials to be presented at medical conferences in 2017

ACE-083

- Report initial FSHD Phase 2 Part 1 dose-escalation results by late 2017
- Initiate CMT Phase 2 Part 1 dose-escalation trial by mid-2017

Dalantercept

- Report top-line results in the DART Phase 2 trial in renal cell carcinoma by Q2 2017

ACE-2494

- Initiate Phase 1 healthy volunteer study in 2017

Corporate

- Host a Research and Development Day to discuss preclinical research and clinical development activities in 2H 2017

Financial Results

- **Cash position** - Cash, cash equivalents and investments as of March 31, 2017 were $213.2 million. As of December 31, 2016 the Company had cash, cash equivalents and investments of $234.4 million. We believe that existing cash, cash equivalents and investments will be sufficient to fund projected operating requirements into the

second half of 2019.

- **Revenue** - Collaboration revenue for the first quarter was $3.7 million. The revenue is all from our Celgene partnership and is primarily due to cost sharing revenue of $3.6 million related to expenses incurred by the Company in support of our partnered programs.
- **Costs and expenses** - Total costs and expenses for the first quarter were $29.6 million. This includes R&D expenses of $21.7 million and G&A expenses of $7.8 million.
- **Other income, net** - Other income, net for the first quarter was $0.5 million primarily due to interest income.
- **Net loss** - The Company's net loss for the first quarter ended March 31, 2017 was $25.4 million.

Conference Call and Webcast

The Company will host a live conference call and webcast to discuss its first quarter 2017 financial results and provide a corporate update on May 8, 2017, at 8:00 a.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 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)

	March 31, 2017	December 31, 2016
Cash and cash equivalents	$ 33,157	$ 20,950
Short and long-term investments	180,057	213,432
Other assets	15,915	13,265
Total assets	$ 229,129	$ 247,647
Deferred revenue	$ 4,108	$ 4,245
Warrants to purchase common stock	1,289	1,244
Other liabilities	15,412	16,561
Total liabilities	20,809	22,050
Total stockholders' equity	208,320	225,597
Total liabilities and stockholders' equity	$ 229,129	$ 247,647

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

Three Months Ended

	March 31,	
	2017	2016
Revenue:		
Collaboration revenue	$ 3,705	$ 18,201
Costs and expenses:		
Research and development	21,727	16,246
General and administrative	7,836	5,911
Total costs and expenses	29,563	22,157
Loss from operations	(25,858)	(3,956)
Other income, net	457	9,017
(Loss) income before income taxes	$ (25,401)	$ 5,061
Income tax provision	(6)	—
Net (loss) income	$ (25,407)	$ 5,061
Other comprehensive loss:		
Net unrealized holding gains on short-term and long-term investments during the period	25	245
Comprehensive (loss) income	$ (25,382)	$ 5,306
Net (loss) income per share applicable to common stockholders:		
Basic	$ (0.66)	$ 0.14
Diluted	$ (0.66)	$ 0.13
Weighted-average number of common shares used in computing net (loss) income per share applicable to common stockholders		
Basic	38,404	36,911
Diluted	38,404	38,666

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 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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