



August 3, 2017

Acceleron Pharma Reports Second Quarter 2017 Operational and Financial Results

- Completed full enrollment of luspatercept MEDALIST and BELIEVE Phase 3 trials -

- Presented updated luspatercept Phase 2 results in patients with MDS and beta-thalassemia receiving treatment for up to two years -

- Treated first patient in ACE-083 Phase 2 study in Charcot-Marie-Tooth disease -

- Upcoming R&D Day on September 19th to highlight Acceleron's pipeline and strategic focus -

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 second quarter ended June 30, 2017.

"The second quarter, and 2017 as a whole, have been marked by tremendous progress and operational execution across our hematology and neuromuscular programs," said Habib Dable, President and Chief Executive Officer of Acceleron. "Over 560 patients were enrolled ahead of estimated timing in our MEDALIST and BELIEVE Phase 3 trials combined, and our partner Celgene continues to invest in new opportunities for luspatercept. Our recent Phase 2 luspatercept results continue to support our confidence in achieving long-term, clinically meaningful outcomes for patients. In addition, our wholly-owned muscle compound ACE-083 has recently expanded its Phase 2 development program into a second neuromuscular disease. We look forward to outlining our long-term vision and growth strategy at our upcoming R&D day in September."

Development Program Highlights

Hematology - Luspatercept

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

Luspatercept is designed to treat the condition of 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.

- | **Completed patient enrollment in the MEDALIST and BELIEVE Phase 3 clinical trials.** The MEDALIST Phase 3 study enrolled 229 patients to evaluate the efficacy and safety of luspatercept in patients with anemia due to lower-risk MDS with ring sideroblasts who require regular RBC transfusions. The BELIEVE Phase 3 study enrolled 336 patients to evaluate the efficacy and safety of luspatercept in patients with anemia due to beta-thalassemia who require regular RBC transfusions.
- | **Results presented at EHA from an ongoing Phase 2 study in lower-risk MDS show increases in hemoglobin, reductions in RBC transfusion burden and RBC transfusion independence.** Patients demonstrated a clinically meaningful increase in hemoglobin for up to 26 months in this ongoing clinical trial.
- | **Results presented at EHA from an ongoing Phase 2 study in beta-thalassemia demonstrate increases in hemoglobin and decreases in RBC transfusion burden** sustained for up to 24 months in this ongoing clinical trial.
- | **Acceleron and Celgene continue preparations for additional luspatercept clinical trials in new patient populations**, including the COMMANDS Phase 3 trial in first-line, lower-risk MDS, the recently initiated Phase 2 trial in myelofibrosis, and the BEYOND Phase 2 trial in non-transfusion-dependent beta-thalassemia.

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.

- | **Enrollment and treatment are ongoing in Part 1 of the Phase 2 trial in FSHD**, one of the most prevalent forms of muscular dystrophy in adults.
- | **Achieved pipeline milestone with first patient treated in the ACE-083 Phase 2 study in patients with CMT disease**, one of the most common inherited neurological diseases leading to focal muscle weakness.

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.

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 early 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
- | Present additional Phase 2 MDS and beta-thalassemia study results at medical conferences in 2017

ACE-083

- | Report FSHD Phase 2 results for cohort 1 in Part 1 in late 2017
- | 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 study in 2017

Research & Development

- | Acceleron to host R&D Day on September 19, 2017 in New York City

Financial Results

- | **Cash position** - Cash, cash equivalents and investments as of June 30, 2017 were \$194.0 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 second quarter was \$3.1 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 second quarter were \$33.0 million. This includes R&D expenses of \$21.6 million and G&A expenses of \$11.4 million. G&A expense includes a \$3.6 million, one-time, non-cash, charge due to modifications of a former executive officer's equity awards in connection with his change in employment status announced in May 2017.
- | **Net loss** - The Company's net loss for the second quarter ended June 30, 2017 was \$29.7 million.

Conference Call and Webcast

The Company will host a webcast and conference call to discuss its second quarter 2017 financial results and provide an update on recent clinical development and corporate activities on August 3, 2017, at 5:00 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. Individuals can participate in the conference call by dialing 877-312-5848 (domestic) or 253-237-1155 (international) and refer to the "Acceleron Second 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 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. 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. Acceleron is also advancing its ACE-083 clinical program in the field of neuromuscular disease, 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](https://twitter.com/AcceleronPharma) and [LinkedIn](https://www.linkedin.com/company/acceleronpharma).

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

	June 30, 2017	December 31, 2016
Cash and cash equivalents	\$ 30,726	\$ 20,950
Short and long-term investments	163,312	213,432
Other assets	14,425	13,265
Total assets	<u>\$ 208,463</u>	<u>\$ 247,647</u>
Deferred revenue	\$ 3,973	\$ 4,245
Warrants to purchase common stock	1,516	1,244
Other liabilities	14,442	16,561
Total liabilities	<u>19,931</u>	<u>22,050</u>
Total stockholders' equity	188,532	225,597
Total liabilities and stockholders' equity	<u>\$ 208,463</u>	<u>\$ 247,647</u>

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

	Three Months Ended June 30		Six Months Ended June 30	
	2017	2016	2017	2016
Revenue:				
Collaboration revenue	\$ 3,057	\$ 3,195	\$ 6,762	\$ 21,396
Costs and expenses:				
Research and development	21,598	16,138	43,327	32,390
General and administrative	11,370	6,712	19,203	12,618
Total costs and expenses	<u>32,968</u>	<u>22,850</u>	<u>62,530</u>	<u>45,008</u>
Loss from operations	(29,911)	(19,655)	(55,768)	(23,612)
Total other income (expense) net	248	(2,361)	705	6,656
Loss before income taxes	(29,663)	(22,016)	(55,063)	(16,956)
Income tax provision	(6)	—	(12)	—
Net loss applicable to common stockholders - basic and diluted	<u>\$ (29,669)</u>	<u>\$ (22,016)</u>	<u>\$(55,075)</u>	<u>\$(16,956)</u>

Net loss per share applicable to common stockholders - basic and diluted	\$ (0.77)	\$ (0.59)	\$ (1.43)	\$ (0.46)
Weighted-average number of common shares used in computing net (loss) income per share applicable to common stockholders	38,631	37,272	38,515	37,092

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," "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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