Acceleron and Fulcrum Therapeutics Announce Pulmonary Research and Discovery Collaboration Agreement

December 30, 2019

Fulcrum to receive $10 million upfront payment and be eligible for future milestone payments.

Cambridge, Mass. – December 30, 2019 – Acceleron Pharma Inc. (Nasdaq:XLRN), a leading biopharmaceutical company in the discovery and development of TGF-beta superfamily therapeutics to treat serious and rare diseases, and Fulcrum Therapeutics, Inc. (Nasdaq:FULC), a clinical-stage biopharmaceutical company focused on improving the lives of patients with genetically defined rare diseases, today announced they have entered into a collaboration and license agreement to identify small molecules designed to modulate specific pathways associated with a targeted indication within the pulmonary disease space.

"This collaboration brings together Fulcrum's skill in identifying drug targets based on modulation of genetic pathways associated with disease and Acceleron's deep expertise in TGF-beta superfamily signaling in an effort to generate potentially disease-modifying therapeutics," said Habib Dable, Chief Executive Officer of Acceleron Pharma. "With this agreement, along with the advancement of the Acceleron-discovered assets sotatercept—in Phase 2 trials in pulmonary arterial hypertension—and ACE-1334, we underscore our growing commitment to the development of novel therapies for patients with pulmonary diseases of high unmet medical need."

Under the agreement, Acceleron will have access to Fulcrum's unique, proprietary product engine and target identification platform with the potential to identify small molecules that control the expression of genes known to impact specific pathways associated with a pulmonary disease of interest. Acceleron and Fulcrum will collaborate on the identification of therapeutic targets and small molecule drug candidates for those targets. Acceleron will be responsible for all development and commercialization activities for any potential therapeutics identified via this platform. Fulcrum will receive a one-time, upfront payment of $10 million as well as reimbursement for relevant R&D costs. Fulcrum will also be eligible to receive research, development and commercial milestone payments of up to $295 million for a first product commercialized and up to a maximum of $143.5 million in additional milestone payments for all subsequent products commercialized. Fulcrum will additionally receive tiered royalty payments in the mid-single-digit to low double-digit range on net sales.

"We are very pleased to partner with Acceleron on this important research initiative," said Robert J. Gould, Ph.D., Chief Executive Officer of Fulcrum Therapeutics. "This collaboration builds on and extends the proven potential of our platform to identify therapies that can address the root cause of diseases, including our progress with losmapimod, currently in a Phase 2 clinical trial for FSHD and extensive pre-clinical and early stage research targeting other genetically defined diseases. This new opportunity to screen and identify pulmonary disease-specific therapies is another reflection of the broad potential applications of the Fulcrum platform in gene modulation."

About Acceleron

Acceleron is a biopharmaceutical company dedicated to the discovery, development, and commercialization of therapeutics to treat serious and rare diseases. Acceleron's leadership in the understanding of TGF-beta superfamily 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, Acceleron and its global collaboration partner, Bristol-Myers Squibb, are co-promoting newly approved REBLOZYL® (luspatercept-aamt), the first and only approved erythroid maturation agent, in the United States and are developing luspatercept for the treatment of chronic anemia in myelodysplastic syndromes and myelofibrosis. Acceleron is also advancing its neuromuscular program with ACE-083, a locally-acting Myostatin+ agent in Phase 2 development in Charcot-Marie-Tooth disease and is conducting a Phase 2 pulmonary program with sotatercept in pulmonary arterial hypertension.

For more information, please visit www.acceleronpharma.com. Follow Acceleron on Social Media: @AcceleronPharma and LinkedIn.

About Fulcrum Therapeutics

Fulcrum Therapeutics is a clinical-stage biopharmaceutical company focused on improving the lives of patients with genetically defined rare diseases in areas of high unmet medical need. Fulcrum's proprietary product engine identifies drug targets which can modulate gene expression to treat the known root cause of gene mis-expression. Fulcrum has advanced losmapimod to Phase 2 clinical development for the treatment of facioscapulohumeral muscular dystrophy (FSHD) and has completed extensive pre-clinical research for FTX-6058 for the treatment of sickle cell disease and beta-thalassemia.

Please visit www.fulcrumtx.com

Source: Acceleron Pharma Inc. and Fulcrum Therapeutics

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Acceleron Forward-Looking Statements

This press release contains forward-looking statements about Acceleron's strategy, future plans and prospects, including statements regarding the development and commercialization of Acceleron's compounds, the timeline for clinical development and regulatory approval of Acceleron's compounds, the expected timing for reporting of data from ongoing clinical trials, and the potential success of a collaboration with Fulcrum Therapeutics. 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 factors, risks and uncertainties, including, but not limited to, that preclinical testing of Acceleron's compounds and data from clinical trials may not be predictive of the results or success of ongoing or later clinical trials, that the results of any clinical trials may not be predictive of the results or success of other clinical trials, that regulatory approval of Acceleron's compounds in one indication or country may not be predictive of approval in another indication or country, that the development of Acceleron's compounds will take longer and/or cost more than planned, that Acceleron will be unable to successfully complete the clinical development of Acceleron's compounds, that Acceleron may be delayed in initiating, enrolling or completing any clinical trials, that Acceleron's compounds will not receive regulatory approval or become commercially successful products, and that Acceleron's collaboration with Fulcrum Therapeutics will not be successful or result in any successful development candidates. These and other risks and uncertainties are identified under the heading "Risk Factors" included in Acceleron's most recent Annual Report on Form 10-K, and other filings that Acceleron 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 Acceleron does not undertake and specifically disclaims any obligation to update any forward-looking statements.

Fulcrum Therapeutics Forward-Looking Statements

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 that involve substantial risks and uncertainties, including statements regarding whether the collaboration will yield any targets, potential milestone payments or royalty payments in connection with the collaboration and the potential benefits of the collaboration. All statements, other than statements of historical facts, contained in this press release, including statements regarding Fulcrum's strategy, future operations, future financial position, prospects, plans and objectives of management, are forward-looking statements. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "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. Any forward-looking statements are based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in, or implied by, such forward-looking statements. These risks and uncertainties include, but are not limited to, risks associated with each party's ability to perform its obligations under the agreement, the sufficiency of Fulcrum's cash resources to fund its foreseeable and unforeseeable operating expenses and capital expenditure requirements on its expected timeline and other important factors discussed in the "Risk Factors" sections contained in Fulcrum's quarterly and annual reports on file with the Securities and Exchange Commission. For a discussion of other risks and uncertainties, and other important factors, any of which could cause Fulcrum's actual results to differ from those contained in the forward-looking statements, see the "Risk Factors" section, as well as discussions of potential risks, uncertainties and other important factors, in Fulcrum's most recent filings with the Securities and Exchange Commission. In addition, the forward-looking statements included in this press release represent Fulcrum's views as of the date hereof and should not be relied upon as representing Fulcrum's views as of any date subsequent to the date hereof. Fulcrum anticipates that subsequent events and developments will cause Fulcrum's views to change. However, while Fulcrum may elect to update these forward-looking statements at some point in the future, Fulcrum specifically disclaims any obligation to do so.