Arrowhead Pharmaceuticals Files for Regulatory Clearance to Begin Phase 1 Study of ARO-ANG3

October 15, 2018

— Arrowhead to host R&D DayOctober 16, 2018 to discuss ARO-ANG3 and its emerging pipeline of RNAi therapeutics

PASADENA, Calif.--(BUSINESS WIRE)--Oct. 15, 2018-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it has filed an application for approval to begin a Phase 1 clinical trial of ARO-ANG3, an RNAi-based investigational medicine targeting angiopoietin like protein 3 (ANGPTL3) being developed for the treatment of dyslipidemias and metabolic diseases. ARO-ANG3 will be the fourth therapeutic candidate to enter clinical studies that leverages Arrowhead's Targeted RNAi Molecule (TRiM™) platform.

Chris Anzalone, Ph.D., president and CEO of Arrowhead Pharmaceuticals, said: "The ARO-ANG3 program has moved rapidly through preclinical development and we are thrilled to now submit the regulatory filing ahead of schedule. After positive initial signs of tolerability and activity with ARO-AAT and ARO-HBV, our first two clinical programs leveraging the TRiM™ platform, we are moving into the ARO-ANG3 clinical program with confidence."

Pending approval, Arrowhead intends to proceed with AROANG1001, a Phase 1 single and multiple dose study to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamic effect of ARO-ANG3 in adult healthy volunteers and dyslipidemic patients. The study is designed to enroll up to 70 subjects.

An application was submitted to an ethics committee in compliance with the Clinical Trial Notification process of the Australian Department of Health and Ageing, Therapeutic Goods Administration.

As a reminder, Arrowhead will host a Research & Development (R&D) Day to discuss ARO-ANG3 and its emerging pipeline of RNAi therapeutics that leverage the Company's proprietary Targeted RNAi Molecule (TRIM™) platform onOctober 16, 2018 in New York City.

The R&D Day will feature presentations by Ira Goldberg, M.D. (NYU Langone Medical Center), who will discuss the current treatment landscape and unmet medical need for patients with dyslipidemia and hypertriglyceridemia.

Arrowhead's management team will provide an overview of the company's pipeline of RNAi-based therapeutics. Discussion topics will include:

- ARO-APOC3 for patients with hypertriglyceridemia. A CTA filing is planned by the end of 2018
- ARO-ANG3 for patients with dyslipidemia
- ARO-ENaC for patients with cystic fibrosis. A CTA filing is planned in 2019
- ARO-HIF2 for patients with renal cell carcinoma. A CTA filing is planned in 2019

This event is intended for institutional investors, sell-side analysts, investment bankers, and business development professionals only. Please RSVP in advance if you plan to attend, as space is limited. To reserve a seat, please click here to register.

A live and archived webcast of the event, with slides, may be accessed on the Events and Presentations page under the Investors section of the Arrowhead website.

About Arrowhead Pharmaceuticals

Arrowhead Pharmaceuticals develops medicines that treat intractable diseases by silencing the genes that cause them. Using a broad portfolio of RNA chemistries and efficient modes of delivery, Arrowhead therapies trigger the RNA interference mechanism to induce rapid, deep, and durable knockdown of target genes. RNA interference, or RNAi, is a mechanism present in living cells that inhibits the expression of a specific gene, thereby affecting the production of a specific protein. Arrowhead's RNAi-based therapeutics leverage this natural pathway of gene silencing.

For more information, please visit www.arrowheadpharma.com, or follow us on Twitter @ArrowheadPharma. To be added to the Company's email list and receive news directly, please visit http://ir.arrowheadpharma.com/email-alerts.

Safe Harbor Statement under the Private Securities Litigation Reform Act:

This news release contains forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. These statements are based upon our current expectations and speak only as of the date hereof. Our actual results may differ materially and adversely from those expressed in any forward-looking statements as a result of various factors and uncertainties, including the safety and efficacy of our product candidates, the duration and impact of regulatory delays in our clinical programs, our ability to finance our operations, the likelihood and timing of the receipt of future milestone and licensing fees, the future success of our scientific studies, our ability to successfully develop and commercialize drug candidates, the timing for starting and completing clinical trials, rapid technological change in our markets, and the enforcement of our intellectual property rights. Our most recent Annual Report on Form 10-K and subsequent Quarterly Reports on Form 10-Q discuss some of the important risk factors that may affect our business, results of operations and financial condition. We assume no obligation to update or revise forward-looking statements to reflect new events or circumstances.

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