



**Attune Pharmaceuticals Announces Positive Phase 1 Results  
for ATN-249, an Oral Plasma Kallikrein Inhibitor  
for the Treatment of HAE**

**NEW YORK, Jan. 22, 2019** --(GLOBE NEWSWIRE) -- Attune Pharmaceuticals, a biotechnology company focused on the discovery and development of novel oral small molecule therapeutics for treatment of rare diseases, today announced positive results from the first in human studies evaluating ATN-249, a novel orally administered plasma kallikrein inhibitor for the treatment of Hereditary Angioedema (HAE). The data were presented by Ira Kalfus, M.D., chief medical officer of Attune, as a poster presentation at the Western Society of Allergy, Asthma and Immunology (WSAAI) 2019 Annual Scientific Session.

"We are very pleased that our first-in-human study showed orally administered ATN-249 achieves high blood plasma levels, with dose-dependent pharmacokinetics and a favorable safety profile," said Andrew McDonald, Ph.D., CEO of Attune Pharmaceuticals. "We expect to complete the multiple ascending dose trial shortly and plan to rapidly advance the program to a Phase 2 trial."

The randomized, placebo-controlled, Phase 1 single ascending dose clinical trial of ATN-249 in healthy volunteers successfully met all its objectives in assessing safety, tolerability and pharmacokinetics (PK) with results supporting further development as an oral plasma kallikrein inhibitor for the prophylactic treatment of HAE. Plasma levels of ATN-249 increased in approximate proportion to dose, and drug exposure was not affected by dosing with food. Once-daily dosing of ATN-249 was safe and well tolerated at all studied doses (50mg to 800 mg) in healthy volunteers. There were no moderate or severe adverse events with all adverse effects being mild and deemed not related to the drug, as well as no dose limiting toxicities.

Ira Kalfus, M.D., said, "The positive outcome of this Phase 1 trial is an important milestone in the development of ATN-249 for the treatment of HAE, a rare and potentially life-threatening disease. Current treatment options for HAE are limited to intravenous and subcutaneous options and are often associated with adverse events. ATN-249, as a potential effective and well-tolerated oral treatment, has the potential to significantly improve disease management and patient quality of life."

**About the Study**

The first in human study of ATN-249 was a double blind, placebo-controlled safety, tolerability, and pharmacokinetic single ascending dose study in 48 healthy volunteers. A total of 30 subjects received a single dose of ATN-249 ranging from 50 mg to 800 mg and 12 subjects received placebo. In the food-effect pharmacokinetic study, 6 subjects in the 100 mg cohort received ATN-249 under fasted conditions (period 1) and after a 7-day



washout received a second 100mg dose 30 minutes after the start of a high fat, high caloric meal (period 2).

### **About Hereditary Angioedema**

Hereditary angioedema (HAE) is a rare, potentially life-threatening disease characterized by acute skin and mucosal edema. It is caused by an autosomal dominant mutation of the SERPING1 or F12 genes, resulting in diminished C1 inhibitor levels and/or function. Dysregulation of the contact-kallikrein pathway mediated by dysfunctional C1 inhibitor causes upregulation of bradykinin production, leading to increased vascular permeability, recurrent abdominal pain, and mucosal swelling, which can be fatal with laryngeal involvement. Current treatments are limited by route of administration and adverse events, since all leading HAE drugs are administered intravenously or subcutaneously and may be associated with drug-specific adverse effects.

### **About ATN-249's Clinical Development Program**

ATN-249 is a novel, potent, selective, and orally-administered plasma kallikrein inhibitor for the treatment of Hereditary Angioedema (HAE). Preclinical studies in both biochemical and contact activation assays have demonstrated that ATN-249 is highly selective and potent at plasma kallikrein inhibition. Upon conclusion of the Phase I study in healthy volunteers, a Phase 2, four-week dose ranging trial to evaluate the safety, tolerability, pharmacokinetics, pharmacodynamics, and efficacy of ATN-249 as a preventative treatment to reduce the frequency of attacks in HAE patients is expected to begin.

### **About Attune Pharmaceuticals**

Attune Pharmaceuticals is a clinical-stage biotechnology company focused on the discovery and development of novel once-daily small molecule therapeutics for treatment of rare diseases. Attune Pharmaceuticals is currently advancing two programs, one in Hereditary Angioedema (HAE) and a second in complement-mediated diseases.

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