



ADARx Pharmaceuticals Presents Onvuzosiran Data Supporting Potential for Sustained Attack-Free Rates with Reduced Treatment Burden in Patients with HAE

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- *Onvuzosiran achieved significant and durable reductions of plasma kallikrein levels with a favorable safety profile in Phase 1/2 –*
- *Positive results support ongoing Phase 3 clinical trial –*

SAN DIEGO, Calif. – February 27, 2026 – [ADARx Pharmaceuticals, Inc.](#) (ADARx), a late-stage clinical biotechnology company developing next-generation RNA therapeutics, today announced Phase 1/2 clinical data for onvuzosiran (ADX-324), an investigational small interfering RNA (siRNA) therapeutic candidate being developed for the treatment of hereditary angioedema (HAE). The Company also highlighted the clinical trial design of its ongoing Phase 3 STOP-HAE study. A poster presentation is being given at the 2026 Annual Meeting of the American Academy of Allergy, Asthma & Immunology (AAAAI) being held February 27 – March 2, 2026, in Philadelphia, PA.

In the Phase 1 study, onvuzosiran demonstrated deep and durable dose-dependent reductions in plasma kallikrein levels. The 300 mg dose showed a 93% reduction at the lowest point, with 80% reduction maintained through Day 169. In the ongoing Phase 2a study, patients achieved similarly deep kallikrein level reductions. Notably, HAE patients experienced no attacks when kallikrein levels were reduced by 80%, further supporting the potential of twice-yearly dosing to achieve high attack-free rates. In the Phase 1/2 study, onvuzosiran was well tolerated, and no serious adverse events were reported, nor were there any treatment-related discontinuations.

“While progress has been made in prophylactic HAE therapy, there remains significant need to further reduce attack frequency and ease the treatment burden that both impact quality of life for patients,” said Dr. Donald Fong, Chief Medical Officer of ADARx. “Plasma kallikrein is a clinically validated target in HAE, and onvuzosiran has demonstrated deep, sustained inhibition, a favorable safety profile, and the potential for less frequent dosing. By leveraging our highly differentiated siRNA platform, we believe onvuzosiran could meaningfully advance the long-term prophylactic treatment landscape, with the goal of delivering a best-in-class profile with semi-annual dosing.”

The Phase 3 STOP-HAE clinical trial is a randomized, double-blind, placebo-controlled study investigating onvuzosiran in preventing HAE attacks in adults with Type I and Type II HAE. The trial, which is currently enrolling patients, will also assess safety, pharmacokinetics, pharmacodynamics, and health-related quality of life measures. Approximately 90 patients with HAE will be enrolled and randomized to receive either onvuzosiran 300 mg every 6 months, onvuzosiran 240 mg every 3 months, or placebo during the study. Patients completing the trial will be eligible to enroll in a long-term open-label extension study.

About the Phase 1/2 Study ([NCT05691361](#))

The Phase 1 placebo-controlled single-dose escalation study was conducted in 43 healthy participants who received onvuzosiran (n=32) in 5 cohorts from 0.4 mg/kg to 6 mg/kg or placebo (n=11) and evaluated the safety, tolerability, pharmacokinetics and pharmacodynamics of onvuzosiran. The open-label Phase 2a part of the study is evaluating plasma kallikrein levels and safety with subcutaneous dosing of onvuzosiran every six months in patients with HAE (n=3).

About HAE and Onvuzosiran (ADX-324)

HAE is a rare genetic disorder characterized by recurrent, unpredictable attacks of swelling that can be painful, disabling, and life-threatening. These attacks result from dysregulation of the kallikrein-kinin system (KKS), which regulates blood pressure, inflammation, coagulation and pain. Prekallikrein (PKK) is a critical protein in the plasma kallikrein pathway that activates a second protein called plasma kallikrein, which, if present, produces bradykinin, a potent vasodilator. A dysfunctional KKS leads to excessive release of bradykinin which causes the swelling attacks in HAE.

Onvuzosiran is an investigational siRNA therapy designed to inhibit PKK generation at the mRNA level and reduce the production of plasma PKK, thereby averting bradykinin generation and potentially preventing HAE attacks. Compared to currently approved prophylactic treatments, Onvuzosiran is expected to decrease PKK to a greater degree, offering the potential for greater and more durable control of kallikrein activity, which is expected to result in a higher proportion of patients remaining attack-free with a less frequent dosing regimen. Onvuzosiran is currently being evaluated in the Phase 3 STOP-HAE clinical trial (<https://stophae.com>) and has received Orphan Drug Designation for the treatment of patients with HAE from the U.S. Food and Drug Administration.

(FDA).

About ADARx Pharmaceuticals

[ADARx Pharmaceuticals, Inc.](#) is a late-stage biotechnology company dedicated to transforming cutting-edge science into next-generation RNA medicines across a wide range of therapeutic areas. We have developed technology to control the expression of specific disease drivers with highly selective RNA targeted therapies with the goal of delivering life-changing treatments for patients with urgent unmet medical needs. ADARx is focused on advancing and expanding a deep pipeline of highly potent, durable and selective RNA-targeted therapeutic candidates, developing product candidates for the treatment of complement-mediated, genetic, cardiovascular, thrombosis, central nervous system and metabolic (obesity) diseases. In addition to our wholly-owned programs, we have entered into a collaboration and license option agreement with AbbVie to develop small interfering RNA (siRNA) therapeutics across multiple disease areas, including neuroscience, immunology and oncology. Follow ADARx on [LinkedIn](#).

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