ADARx Pharmaceuticals Receives FDA Fast Track Designation for Onvuzosiran for the Treatment of HAE

August 24, 2026

SAN DIEGO, Calif. – August 24, 2026 – ADARx Pharmaceuticals, Inc. (ADARx), a late-stage clinical biotechnology company developing next-generation siRNA therapeutics, announced today that it has received Fast Track designation from the U.S. Food and Drug Administration (FDA) for onvuzosiran, an investigational small interfering RNA (siRNA) therapeutic candidate, for prophylaxis to prevent attacks of hereditary angioedema (HAE). Onvuzosiran is currently being evaluated in the Phase 3 STOP-HAE clinical trial (NCT06960213).

"We are pleased that the FDA has granted Fast Track designation for onvuzosiran, based on the data generated to date and reflecting the significant unmet need and treatment burden faced by people living with HAE," said Dr. Zhen Li, President and Chief Executive Officer of ADARx. "As we continue to progress the Phase 3 trial, this marks an important milestone for the program."

FDA's Fast Track designation is intended to facilitate and expedite the development and review of new drugs to treat serious or life-threatening conditions. To qualify, available clinical and non-clinical data need to demonstrate the potential to address unmet medical need. The benefits of Fast Track designation include opportunities for frequent meetings with the FDA to discuss trial design, development plans and data needed to support drug approval, as well as the ability to submit a New Drug Application (NDA) on a rolling basis, and eligibility for priority review, if relevant criteria are met. For more information on the Fast Track process, please visit the FDA's official website.

In October 2025, the FDA granted orphan drug designation to onvuzosiran for the treatment of HAE.

About HAE and Onvuzosiran
HAE is a rare autosomal dominant genetic disorder characterized by recurrent and unpredictable attacks of swelling that can be painful, debilitating, and life threatening. The underlying cause of HAE is an abnormality in the kallikrein-kinin cascade resulting in increased bradykinin, a potent vasodilator that increases vascular permeability, leading to the characteristic swellings of the disease. Uncontrolled plasma kallikrein activity is the critical component leading to increased bradykinin and the pathologic vascular permeability, vasodilation and tissue swelling in HAE attacks.

Onvuzosiranis an investigational siRNA therapy designed to inhibit prekallikrein (PKK) generation at the mRNA level and reduce the production of plasma PKK, thereby averting plasma kallikrein activation and bradykinin generation and potentially preventing HAE attacks. Compared to currently approved prophylactic treatments, onvuzosiranis 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. Onvuzosiranis currently being evaluated in the Phase 3 STOP-HAE clinical trial (https://stophae.com) and has received Orphan Drug and Fast Track Designations 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 siRNA therapeutics. We have developed technology designed 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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