Press Release

Skyhawk Therapeutics Announces First U.S. Patients Dosed in Pivotal FALCON-HD Study Evaluating SKY-0515 in Huntington's Disease

More than 50,000 doses of SKY-0515 have now been administered to patients.

BOSTON, Nov. 5, 2026 - Skyhawk Therapeutics, Inc., a clinical-stage biotechnology company developing novel small molecule therapies designed to modulate RNA targets, today announced that the first U.S. patients have been dosed in the global pivotal FALCON-HD study evaluating SKY-0515 in Huntington's disease (HD).

Dosing in the U.S. began at the CenExel Movement Disorder Center in Colorado. Additional U.S. sites, including Beth Israel Deaconess Medical Center, are expected to begin dosing in the coming weeks.

More than 50,000 doses of SKY-0515 have been administered to date, across more than 30 study sites in 10 countries.

“The opening of U.S. sites in the pivotal FALCON-HD study is an important milestone for the U.S. Huntington’s disease community, where there are still no approved therapies shown to slow the progression of the disease,” said Samuel Frank, MD, Director of the Huntington’s Disease Society of America Center of Excellence at Beth Israel Deaconess Medical Center and a FALCON-HD investigator. “SKY-0515 is designed to lower both mutant huntingtin and PMS1, two proteins implicated in HD pathology and progression, and the Phase 1/2 results to date have been very encouraging. We look forward to beginning dosing at Beth Israel Deaconess before end of 2026 and to offering eligible people with HD in the U.S. the opportunity to take part in this study.”

“Bringing FALCON-HD to the U.S. is an important step toward our goal of delivering a once-daily oral, potentially disease-modifying treatment to people living with Huntington’s disease,” said Bill Haney, Co-founder and Chief Executive Officer of Skyhawk Therapeutics. “We are grateful to the participants, families and investigators whose commitment is making this study possible.”

There are currently no approved disease-modifying treatments for the more than 40,000 people in the U.S. living with symptomatic HD and hundreds of thousands affected worldwide.

About SKY-0515

SKY-0515 is an investigational, once-daily oral small molecule RNA splicing modifier being developed for the treatment of Huntington’s disease. SKY-0515 is designed to target two core mechanisms associated with HD— reducing both mutant huntingtin (mHTT) protein and PMS1. Mutant huntingtin is the primary protein responsible for HD pathology, while PMS1 is a key driver of somatic CAG repeat expansion associated with disease progression.

About FALCON-HD

The FALCON-HD pivotal program (NCT06873334 and NCT07378644) comprises two randomized, double-blind, placebo-controlled, dose-ranging studies evaluating the pharmacodynamics, safety and efficacy of SKY-0515. Eligible participants receive once-daily oral SKY-0515 at one of three dose levels or placebo.

FALCON-HD 004-ANZ enrolled 144 participants with Stage 2 and Stage 3 HD across sites in Australia and New Zealand. Enrollment is complete.

FALCON-HD 004-WW plans to enroll participants with Stage 2 and Stage 3 HD across more than 40 sites worldwide and is actively recruiting.

Additional information regarding FALCON-HD, including participating sites and eligibility criteria, is available at ClinicalTrials.gov and www.FALCON-HD.com.

About Huntington's Disease

Huntington's disease is a progressive, inherited neurodegenerative disorder caused by a CAG repeat expansion in the huntingtin gene. The disease leads to progressive deterioration in movement, cognition and function and is ultimately fatal. There are currently no approved therapies that have been shown to slow or stop the underlying progression of Huntington's disease.

About Skyhawk Therapeutics

Skyhawk Therapeutics is a clinical-stage biotechnology company developing novel small molecule therapies that modify RNA expression. The company's proprietary SKYSTAR® platform enables the discovery and development of orally administered small molecules designed to address RNA targets implicated in serious neurological and other diseases. Skyhawk is headquartered in Boston, Massachusetts. For more information, visit www.skyhawktx.com.

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