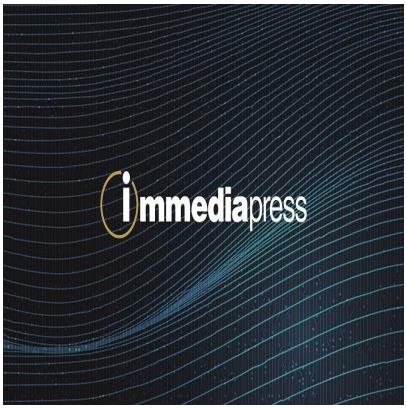

Skyhawk Therapeutics Announces Expansion of its Global Pivotal FALCON-HD Clinical Trial for SKY-0515 in Huntingtonâ??s Disease to the United States, Canada and the United Kingdom

Descrizione

COMUNICATO STAMPA â?? CONTENUTO PROMOZIONALE

More than ten countries and more than twenty sites are now participating in the Companyâ??s global pivotal trial for Huntingtonâ??s disease, with over 175 patients enrolled.

SKY-0515â??s Phase 1/2 enrollment is complete and it has been generally well tolerated with twelve months of patient data. SKY-0515 has shown 69% reduction in mHTT, 26% reduction in PMS1, and cUHDRS improvements from baseline instead of natural history declines.

BOSTON, July 14, 2026 /PRNewswire/ â?? Skyhawk Therapeutics, Inc., a clinical-stage biotechnology company developing novel small molecule therapies designed to modulate RNA targets, announces it has received additional regulatory approvals to open its Phase 2/3 FALCON-HD (004-ANZ and 004-WW) pivotal trial, with an IND acceptance in the United States and CTA acceptances in each Canada and the United Kingdom.

â??We are excited to now begin enrolling patients in our pivotal trial for Huntingtonâ??s in the United States, the United Kingdom and Canada,â?• said Sergey Paushkin, head of Skyhawk R&D. â??Clinician and Patient assessments done at twelve months of treatment support what the SKY-0515â??s compelling and consistent effects on critical biomarkers and cUHDRS scores demonstrateâ??the exciting possibility that SKY-0515 may offer Huntingtonâ??s patients a type of therapy they have long deserved, in a convenient daily pill.â?•

SKY-0515 has demonstrated excellent central nervous system exposure and has been generally safe and well tolerated across dose levels studied.

Huntingtonâ??s disease is a rare, hereditary, and ultimately fatal neurodegenerative disorder affecting more than 40,000 symptomatic individuals in the United States, with hundreds of thousands more impacted worldwide. There are currently no approved therapies shown to slow or halt disease progression.

SKY-0515 is an orally administered investigational small molecule RNA splicing modifier developed by Skyhawk using the companyâ??s proprietary SKYSTARÂ® platform. SKY-0515 is designed to reduce both mHTT and PMS1 proteins and has demonstrated that it does so effectively.

Skyhawkâ??s SKYSTAR platform has already generated additional novel therapies targeting rare neurological diseases with no approved disease-modifying treatment. The company plans to take several of these programs into clinical development by the end of 2027.

About SKY-0515â??s Phase 2/3 FALCON-HD (004-ANZ and 004-WW) Pivotal ProgramSKY-0515â??s FALCON-HD pivotal program (NCT06873334 and NCT07378644) is a randomized, double-blind, placebo-controlled, dose-ranging study evaluating the pharmacodynamics, efficacy and safety of SKY-0515.

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

FALCON-HD 004-WW plans to enroll up to an additional 400 participants with Stage 2 and early Stage 3 HD across more than 40 sites worldwide and is actively treating patients at a number of these sites presently.

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

About SKY-0515â??s Phase 1/2 Clinical ProgramSKY-0515â??s Phase 1/2 clinical trial is a first-in-human study designed to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of SKY-0515 in healthy volunteers and participants with early-stage Huntingtonâ??s disease (HD), as well as to assess biomarkers including mutant HTT protein and PMS1 mRNA, and efficacy endpoints including cUHDRS and its subcomponents Total Functional Capacity (TFC), Total Motor Score (TMS), Symbol Digit Modalities Test (SDMT), and Stroop Word Reading Test (SWRT).

The Phase 1/2 trial consists of three parts. Parts A and B evaluated SKY-0515 in Healthy Volunteers. Part C is a randomized, double-blind, placebo-controlled parallel-group study evaluating two dose levels of SKY-0515 in people living with Huntingtonâ??s disease with early-stage HD (HD-ISS Stage 1, Stage 2, or mild Stage 3) over 84 days, followed by a twelve-month blinded extension period during which all participants receive active treatment at either a low or high dose.

Enrollment in the Phase 1/2 study is complete.

About Skyhawk TherapeuticsSkyhawk Therapeutics is a clinical-stage biotechnology company leveraging its proprietary SKYSTARÂ® platform to discover and develop small molecule RNA-modulating therapies for the worldâ??s most intractable diseases. For more information, visit www.skyhawktx.com.

Skyhawk ContactMaura McCarthyHead of Corporate Developmentmaura@skyhawktx.com

View original content to download multimedia:https://www.prnewswire.co.uk/news-releases/skyhawk-therapeutics-announces-expansion-of-its-global-pivotal-falcon-hd-clinical-trial-for-sky-0515-in-huntingtons-disease-to-the-united-states-canada-and-the-united-kingdom-302825078.html

â??

immediapress/pr-newswire

Categoria

1. Comunicati

Tag

1. ImmediaPress

Data di creazione
Luglio 14, 2026
Autore
redazione