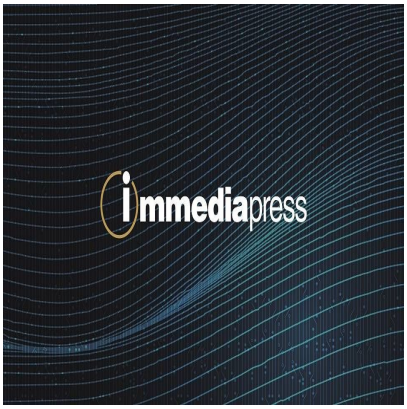

Skyhawk Therapeutics Releases Twelve-Month cUHDRS Subcomponent Results from its Phase 1/2 Clinical Trial of SKY-0515 in Huntingtonâ??s Disease

Descrizione

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Twelve-month findings showed favorable and consistent trends for participants receiving SKY-0515 across all four cUHDRS subcomponents, including Total Functional Capacity (TFC), Total Motor Score (TMS), Symbol Digit Modalities Test (SDMT), and Stroop Word Reading Test (SWRT)

Skyhawk also releases Clinician and Patient Global Impression (CGI and PGI) improvement of disease survey data, which shows that at twelve months no clinician or patient assessed disease progression â?? all saw either disease stabilization, or improvement

BOSTON, June 30, 2026 /PRNewswire/ â?? Skyhawk Therapeutics, Inc., a clinical-stage biotechnology company developing novel small molecule therapies designed to modulate RNA targets, announces cUHDRS subcomponent data from the twelve-month interim analysis of data from its Phase 1/2 clinical trial evaluating SKY-0515, an oral investigational treatment for Huntingtonâ??s disease (HD).

The twelve-month data, shared at the European Academy of Neurology, shows favorable and consistent trends for participants on SKY-0515 across all cUHDRS subcomponents, including Total Functional Capacity (TFC), Total Motor Score (TMS), Symbol Digit Modalities Test (SDMT), and Stroop Word Reading Test (SWRT).

At twelve months, participants receiving SKY-0515 demonstrated favorable mean change from baseline TFC score of +0.07, compared with an expected worsening of -0.87 points from propensity score-weighted analyses which use Enroll-HD natural history datasets.

At twelve months, participants receiving SKY-0515 demonstrated favorable mean change from baseline TMS score of -2.00, compared with an expected worsening of 2.21 points from propensity score-weighted analyses which use Enroll-HD natural history datasets.

At twelve months, participants receiving SKY-0515 demonstrated favorable stabilization of mean change from baseline SDMT score of -0.19, compared with an expected worsening of -1.78 points from

propensity score-weighted analyses which use Enroll-HD natural history datasets.

At twelve months, participants receiving SKY-0515 demonstrated favorable mean change from baseline SWRT score of +3.44, compared with an expected worsening of -3.13 points from propensity score-weighted analyses which use Enroll-HD natural history datasets.

Skyhawk also released today Clinician and Patient Global Impression (CGI and PGI) improvement of disease survey interim data, assessed by clinicians or their participants over twelve months. For HD participants for whom Huntingtonâ??s disease is expected to cause considerable disease worsening of over twelve months, no clinicians or participants assessed any worsening at twelve months and 65% of participants and 50% of clinicians assessed that there had been improvement.

Treatment with SKY-0515 resulted in dose-dependent reductions in mutant huntingtin (mHTT) protein in blood of up to 69% as well as reductions in PMS1 mRNA of up to 26%. 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.

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â??We are pleased to share this exciting subcomponent data from our cUHDRS assessments, including TFC and TMS data on functional and motor capacity and SDMT and SWRT cognitive test data,â?• said Sergey Paushkin, Head of R&D at Skyhawk Therapeutics.

â??And we are excited by the fact that participants and their clinicians, for whom Huntingtonâ??s disease is expected to cause considerable disease worsening, see no worsening at twelve months â?? and 65% of participants and 50% of clinicians assessed that there had been improvement. This further confirms for us the exciting possibility that SKY-0515â??s compelling and consistent effect on the critical biomarkers of mHTT and PMS1 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. There are more than 175 participants enrolled in Skyhawkâ??s SKY-0515 program, in five countries.

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 using the companyâ??s proprietary SKYSTARÂ® platform. SKY-0515 is designed to reduce both mHTT and PMS1 proteins.

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

About SKY-0515â??s Phase 1/2 Clinical Program SKY-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 SKY-0515â??s Phase 2/3 FALCON-HD (004-ANZ and 004-WW) Pivotal Program SKY-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.

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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 already.

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

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.

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