SKYHAWK THERAPEUTICS
FALCON-HD

An Update for the Huntington's Disease Community on SKY-0515

Dear friends in the Huntington's disease community,

We'd like to share an update on ongoing research into **SKY-0515,** an investigational medicine being studied for Huntington's disease (HD). A scientific press release was shared today, and this letter is meant to explain the news in a more community-focused, easier-to-read way.

We know how important it is for families and individuals affected by HD to receive clear, honest updates, and we appreciate you taking the time to read this.

What is SKY-0515?

SKY-0515 is an investigational, **once-daily oral medicine** that is currently only available through clinical trials. It is not approved for use in any disease or in any country.

The investigational medicine is designed to affect two biological factors involved in Huntington's disease:

- **Mutant huntingtin (mHTT),** the abnormal protein that causes HD
- **PMS1**, a protein that may be involved in processes linked to disease progression

Researchers are studying whether changes in these biological markers could be relevant to HD over time.

What was shared in today's announcement?

The announcement included results from a **nine-month interim analysis** of people with early-stage HD who are participating in a Phase 1 clinical study.

This study is focused mainly on safety and on understanding how the investigational medicine behaves in the body.

Here is what was observed so far:

Changes in biological markers

- People taking SKY-0515 showed **dose-dependent reductions in mutant huntingtin levels in blood**
- At the higher dose studied, average reductions of about **62% were seen**
- Reductions in **PMS1 mRNA of about 26%** were also observed

- The investigational medicine was shown to **reach the brain**, which is essential for treating HD

These findings suggest that SKY-0515 is interacting with its intended biological targets. At this stage, it is not known how these changes relate to symptoms or long-term outcomes.

What do we know about safety?

- SKY-0515 has been **generally well tolerated** in the study so far
- No significant safety concerns were identified in the data reported
- Safety continues to be closely monitored as the studies move forward

Were symptoms or function measured?

Researchers also collected **exploratory clinical assessments**, including a combined score called the Composite Unified Huntington's Disease Rating Scale (**cUHDRS**), which looks at movement, thinking, and daily function.

- Average scores were **generally stable over time,** with small changes observed
- After nine months, participants on SKY-0515 showed **small average improvements from baseline**
- Because this is an early-phase study with a limited number of participants, these findings should be interpreted with caution
- Larger and longer studies are needed to better understand whether SKY-0515 may have an effect on symptoms or disease progression.

What studies are happening now?

Phase 1 study

- Enrollment is complete
- Participants are continuing in a blinded extension period
- Additional data are expected in mid-2026

Phase 2/3 FALCON-HD study

- Currently enrolling in Australia, New Zealand and Georgia. Other countries will be added soon
- Participants will receive treatment or placebo for at least 12-18 months
- This study will further evaluate safety, biological markers, and clinical measures

More details are available at ClinicalTrials.gov NCT06873334 and www.FALCON-HD.com.

What does this update mean for the HD community?

These results represent early but meaningful progress in the clinical development of SKY-0515. The findings show that the medicine is having measurable biological effects, but it is still too early to know what this may mean for people affected with HD.

SKY-0515 remains an investigational treatment, and further research is needed before any conclusions can be drawn about its potential role in HD care. We remain committed to sharing updates transparently as the science evolves.

Stay Engaged

Over the coming months, members of the Skyhawk team will participate in community events, conferences, and webinars hosted by several patient advocacy organizations, including Huntington's Australia, Huntington's Victoria, Huntington's Disease Association of Auckland, Huntington's Disease Association of America, Huntington Society of Canada, Huntington's Disease Youth Organization, Factor-H, Association Huntington France and Huntington's Disease Association of England and Whales.

These organizations will share details about dates, times, and how to join through their usual channels, so we encourage you to follow them for the latest updates.

Thank you

We are deeply thankful to the individuals and families who take part in research studies, and to the advocacy organizations and community members who continue to support HD research. We know the community has been waiting a long time for progress, and we are grateful that you continue to engage with us despite the uncertainties. Thank you for placing your hope and trust in research.

We remain committed to sharing updates as the research progresses.

With appreciation,

The Skyhawk Therapeutics Team