SKY-0515-004-ANZ Clinical Trial

A resource for people with Huntington's disease, their families, and carers

ABOUT THIS INFORMATIONAL FLYER



This informational flyer has been produced by Skyhawk Therapeutics, in consultation with Huntington's Australia, to provide essential information about the **SKY-0515-004-ANZ clinical trial.**

Clinical trials can test more than just medicines. They may also study lifestyle changes, surgical procedures, and medical devices. However, this flyer focuses on medicine trials.

Why are clinical trials needed?

Clinical trials aim to answer key questions, such as:

- Does the new medicine work?
- Is it more effective than existing treatment options?
- Does it have any side effects?

These studies are crucial in helping healthcare professionals determine which medicines are best suited for individuals. However, it is important to know that participation in a clinical trial does not guarantee access to a better treatment.

Who conducts and regulates clinical trials?

Clinical trials may be conducted by governments, pharmaceutical companies, hospitals, and universities. They are strictly regulated by laws, ethical standards, and codes of conduct.

Before a trial can begin, it must be approved by the Ethics Committee of each clinical site involved.

Are there benefits of participating in a trial?

Taking part in a clinical trial may offer several benefits, including:

- Contributing to the development of new medications that may change the course of Huntington's disease for current and future generations.
- Access to medical experts and facilities.
- Ongoing medical monitoring by experts throughout the trial.

What happens before a clinical trial?

Eligibility criteria must be met in order to participate in a clinical trial. Having the disease does not automatically make someone eligible for a study. Similarly, if someone is not eligible for this study, they may be eligible for other studies.

What happens during a clinical trial?

The treatment process depends on the trial. Some medications are taken at home, while others require clinic visits. Participants may need regular assessments to monitor effects.

Trials can last months, so your commitment is crucial for reliable results, and early withdrawal may occur due to side effects. It's essential to follow instructions on dosage, timing, and food requirements for accurate results.

What happens after a clinical trial?

Depending on the results, there will be a decision about what to do next:

- To proceed to another trial phase.
- To stop the research.
- Or to seek regulatory approval for the medication to be available for use.

If you have more questions, please speak to a healthcare professional.

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Eligibility Criteria for SKY-0515-004-ANZ

Inclusion criteria

Participants must:

- Be ≥ 25 years old at the time of informed consent.
- Have genetically confirmed Huntington's disease (CAG ≥ 40 in the HTT gene).
- Meet the following screening criteria:
  - Total Functional Capacity (TFC) ≥ 10
  - Total Motor Score (TMS) ≥ 6
  - Independence Scale (IS) ≥ 70
- Adhere to local contraceptive regulations if applicable.
- Provide signed informed consent and comply with study requirements.
- If previously in a tominersen study, the last dose must have been at least 12 months before dosing with SKY-0515/Placebo.

Participants cannot join if they:

- Have other significant neurological or medical conditions.
- Have conditions that would interfere with assessments (e.g., implanted shunts).
- Have had active malignancy in the last five years (excluding specific skin cancers).
- Have a history of severe allergic reactions to the study drug.
- Are currently using drugs that affect HTT mRNA or protein levels.
- Have elevated liver function test results.
- Test positive for HBsAg, HepC, or HIV antibodies.
- Are pregnant, planning pregnancy, or currently breastfeeding.
- Have a history of substance abuse or other conditions that may interfere with participation.

About SKY-0515-004-ANZ

Skyhawk Therapeutics, a biotechnology company pioneering innovative treatments, advanced the fight against Huntington's disease with the development of SKY-0515 which is an investigational drug. With no approved treatments to reverse or slow disease progression, SKY-0515 offers a new approach by modifying RNA expression of the HTT gene to reduce the production of mutated huntingtin protein—targeting the root cause of the disease.

Developed using Skyhawk's SKYSTAR® platform, SKY-0515 is designed to cross the blood-brain barrier and reach peripheral tissues. It is currently in a Phase 1 clinical trial in Australia to assess its safety, tolerability, and effectiveness in lowering harmful huntingtin protein levels.