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HARNESS THERAPEUTICS

FAN1 Candidate selection
Patient group Q&A

What is HRN001 and how does it work?

HRN001 is Harness Therapeutics' investigational drug candidate for Huntington's disease. It is an antisense oligonucleotide (ASO) designed to selectively increase levels of FAN1, a DNA repair protein linked to slower disease progression. HRN001 uses Harness' proprietary MISBA® (microRNA site-blocking ASO) platform, which aims to increase FAN1 in a controlled way. By increasing FAN1, the therapy is designed to help address somatic CAG repeat expansion, a biological process now recognised as an important driver of Huntington's disease onset and progression. HRN001 is still in preclinical development and has not yet been tested in people.

What makes FAN1 such an important target for Huntington's disease treatment?

Genetic studies have identified FAN1 as one of the strongest modifiers of Huntington's disease onset discovered to date. Variations in the FAN1 gene have been associated with delayed disease onset, likely through effects on somatic expansion of CAG repeats in the huntingtin (HTT) gene. Because ongoing somatic expansion is believed to contribute to disease progression, increasing FAN1 activity represents a new investigational approach that researchers hope may influence the underlying biology of Huntington's disease.

Is HRN001 a gene therapy?

No. HRN001 is not a gene therapy and does not change or edit a person's DNA. It is designed to influence how a naturally occurring protein is regulated in cells. The effects of ASO medicines are not permanent and would require repeat dosing if proven safe and effective in future studies.

When will HRN001 enter clinical trials and what has been shown in preclinical studies?

Harness plans to progress HRN001 towards first-in-human clinical studies in 2027. Preclinical research has shown that HRN001 can increase FAN1 levels and slow somatic expansion in laboratory models of HD, along with favorable pharmacokinetic and tolerability characteristics. Further preclinical development will continue throughout 2026 to support potential clinical development. CSO Andy Billinton will present preclinical HD data at the CHDI Foundation Huntington's Disease Therapeutics Conference on the 24 February 2026. A clinical advisory board has been established to help guide future trial design and development

How is this approach different from other Huntington's disease therapies in development?

Many investigational therapies aim to lower levels of huntingtin protein directly. HRN001 takes a different approach by increasing FAN1, a DNA repair protein linked through genetics to delayed disease onset. Rather than targeting huntingtin itself, the programme focuses on somatic expansion, a key biological process believed to influence disease progression.

Who might be eligible for future clinical trials?

It is too early to define who may be eligible for clinical trials. Eligibility criteria are determined during clinical trial planning and will be shared publicly once studies begin. Early-phase trials typically focus on safety and may involve a small number of participants.

Why is this development important for the HD community?

There are currently no approved disease-modifying treatments for Huntington's disease. HRN001 represents an early-stage research programme exploring a new way to address somatic expansion, a fundamental driver of the disease. While still at an early stage, increasing FAN1 (a genetically supported target linked to delayed disease onset) may offer a potential disease-modifying strategy if future studies are successful.

What does this announcement mean for patients living with Huntington's disease today?

This announcement reflects an early step in the development of a potential new treatment approach. HRN001 has not yet entered clinical trials, and further research is needed before its safety or effectiveness in people can be determined. Harness plans to continue preclinical development with the goal of moving toward clinical studies in 2027.

How will the community be kept informed?

Harness is committed to sharing updates with the Huntington's disease community as the programme progresses. Information will be shared through scientific conferences, company communications and, if clinical trials begin, through public clinical trial registries and patient organisations.