12 June 2025

Dear HD community leaders,

Following your request to be informed of our research updates, we're happy to share that our gene therapy study has started and the first person was recently dosed. This Phase 1/2 clinical trial (NCT06826612) is evaluating the safety and preliminary efficacy of an investigational AAV gene therapy RG6662 (also known as SPK-10001) in adults with Huntington's disease (HD).

Start of new gene therapy clinical trial

We are grateful to the initial study participant and their family for helping us begin the essential work moving the drug into clinical testing. Progress in treatments for HD – and any disease – is reliant on families willing to volunteer for research. Thank you!

RG6662 is a gene therapy designed to lower huntingtin protein, including mutant huntingtin. It is delivered directly to specific brain structures most affected in HD via a one-time, neurosurgical procedure. The study will have multiple parts and progress over several years. The first part (Part A) will run in the United States and involve approximately 8 participants. Dosing and study progression will occur as safety milestones are met.

As with all early stage clinical research, our team will learn from each participant's experience, and we will adjust the study based on learnings. We look forward to sharing updates as research continues. We are conducting the study in partnership with Huntington Study Group (HSG). For more information (including study qualification and contact information), visit the study page on the HSG website.

Programme moving into Roche HD portfolio

Development of this gene therapy has thus far been led by Spark Therapeutics, a member of the Roche Group. Now moving forward, the Spark HD programme will fully transfer to Roche. Roche is pursuing multiple huntingtin-lowering approaches, and by integrating this gene therapy into our portfolio we strengthen our commitment to developing HD medicines.

We express deep appreciation to the many community members who have worked with us, shared their experiences, asked great questions, and helped to inform the design of our programme. The contributions have been very meaningful. The initiation of this study is an exciting milestone that was only possible with your partnership.

Sincerely on behalf of the Spark and Roche HD teams,

Jacose Bell
Director of Patient Advocacy, Spark

Mai-Lise Nguyen
Global Patient Partnership Leader, Roche

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