March 2025: This Month in Huntington's Disease Research

With so much science going on in the Huntington's disease space, we're bringing you a monthly recap from March 2025 to cover all the biggest HD science news and research we covered this month. Enjoy!

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March 2025 was packed with groundbreaking discoveries in Huntington's disease (HD) research, and we're here to bring you the biggest highlights! From the cutting-edge CRISPR delivery system RIDE, which could rewrite the playbook on gene editing, to major advances in drug development, biomarker breakthroughs, and fresh insights into HD biology, this month was a whirlwind of progress. Scientists are pushing the boundaries of what's possible, inching closer to real solutions for HD families. March not only brought us scientific progress, but also community events, like HDYO's International Young Adult Congress and Factor-H's Gratitude Day, that raise awareness about HD, provide a platform of support, and bring the community together. If you missed any of the exciting updates, don't worry—we've got you covered. Dive into our March recap and catch up on all the HD news you need to know!

CHDI

Every year CHDI hosts one of the largest HD research conferences. The 2025 HD Therapeutics Conference showcased significant advancements in research and potential therapies for HD. Over three days, more than 400 scientific experts from around the world convened to discuss clinical trial updates, genetic modifiers, and innovative technologies.

Scientists, advocates, and families are coming together to drive progress in HD research. Read our March recap to see how collaboration is shaping the future!

Image credit: Jerry Turner, CHDI

Day 1: Progress in Clinical Trials

The conference began with updates on therapeutic trials targeting huntingtin (HTT), the protein responsible for HD. Researchers shared data on ongoing efforts to reduce HTT levels safely, including small molecule drugs and gene therapies. Discussions also focused on refining clinical trial design, selecting meaningful biomarkers, and ensuring that future trials are better equipped to detect therapeutic benefits.

Day 2: Genetic Modifiers and Disease Progression

The second day explored genetic factors that influence when HD symptoms begin and how the disease progresses. Scientists presented findings from large-scale genetic studies identifying key modifiers that may delay disease onset, offering promising new therapeutic targets. Advances in understanding DNA repair pathways and their role in HD progression were also highlighted, providing new directions for potential treatments.

Day 3: New Technologies and Future Directions

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The final day spotlighted cutting-edge technologies that could revolutionize HD research and treatment. Talks covered advances in gene editing, innovative drug delivery methods, and AI-driven approaches to analyzing HD progression. Researchers emphasized the importance of collaboration and continued innovation to translate these discoveries into real-world therapies.

The conference reinforced the growing momentum in HD research, with scientists and industry partners working together to turn breakthroughs into meaningful treatments for families affected by HD.

Wake up call: Sleep is impacted before Huntington's disease symptoms appear

A recent study, highlighted during Sleep Awareness Week, reveals that sleep disturbances can occur in individuals carrying the HD gene up to 15 years before the onset of other symptoms. Researchers observed that those less than 15 years from predicted symptom onset experienced fragmented sleep and increased nighttime wakefulness, while those more than 15 years away may not show significant sleep issues. These findings suggest

that early sleep disruptions may contribute to thinking and mood impairments associated with HD, highlighting the potential of sleep-focused interventions to improve quality of life and possibly slow disease progression.

Listening for Whispers: How a Tiny Protein Could Transform HD Research

From CRISPR breakthroughs to biomarker discoveries, HD research is accelerating faster than ever. Catch up on March's biggest scientific updates!

Image credit: Polina Tankilevitch

A recent 14-year longitudinal study has demonstrated that neurofilament light (NfL), a protein released by damaged brain cells, can serve as an early indicator of HD progression. Elevated NfL levels were detected in individuals carrying the HD gene many years before the onset of symptoms, correlating with disease advancement. This finding suggests that monitoring NfL through simple blood tests could revolutionize HD research by predicting symptom onset, enhancing clinical trial design, and enabling earlier therapeutic interventions to potentially slow or halt disease progression.

The Huntington's Disease Youth Organization's World Congress: Supporting Young People Affected by HD

The Huntington's Disease Youth Organization (HDYO) supports, educates, and empowers young people affected by HD. HDYO's recent biennial International Young Adult Congress provided a unique space for connection and learning. At the 2025 Congress in Prague, attendees engaged in workshops, heard from leading researchers, and shared personal experiences, fostering a strong sense of community.

HDBuzz gave two presentations, firstly setting the stage with HD research terminology 101, then diving into an overview of important research going on at the benchside and in the clinic. Key themes in other talks included mental health, genetic testing decisions, and

navigating family dynamics, with experts offering guidance tailored to young people facing HD. The event underscored the importance of youth involvement in research, advocacy, and peer support, reinforcing HDYO's mission to empower the next generation of HD families.

Gratitude Day

> **"Science is moving fast, and every step forward brings us closer to real treatments for HD families. "**

Factor-H is a nonprofit dedicated to supporting HD families in Latin America, where extreme poverty and lack of resources make the disease even more devastating. This month, HDBuzz caught up with Factor-H founder, Dr. Ignacio Muñoz-Sanjuán, in an interview that details the unique problems that people with HD there face, and how they help and support these vulnerable families.

The organization provides humanitarian aid, medical care, housing improvements, education, caregiver training, and legal advocacy to some of the most vulnerable HD communities in Venezuela, Peru, and Colombia. These same communities played a crucial role in the discovery of the HD gene in 1993, yet many still lack basic necessities. Factor-H also works to combat stigma, educate the public, and advocate for sustainable, long-term support.

A key initiative is Gratitude Day, held this year on March 23rd, which honors HD families' contributions to research while raising awareness of their ongoing struggles. The 2025 event included a live stream from Venezuela, candlelight vigils, and medical outreach, reinforcing the importance of global solidarity. Factor-H's mission highlights the need for both immediate relief and systemic change, ensuring that hope and humanity—not just hardship—define the future for HD families.

Molecular Surgeons for Huntington's Disease Catch a RIDE with CRISPR Advancements

A groundbreaking gene-editing technology called RIDE (Ribonucleoprotein Delivery) is showing promise for treating HD. RIDE uses CRISPR, a powerful tool to edit DNA, and delivers it precisely to targeted cells in the brain, overcoming key challenges like off-target effects and immune responses. Early tests in mice and monkeys have demonstrated that RIDE can effectively reduce harmful HTT protein levels in the brain, leading to improved behavior and motor function. This system offers lasting effects from a single injection and is designed to minimize the risks of unintended genetic changes. While still in early stages, RIDE's targeted approach brings new hope for HD treatments, with the potential for broader applications in genetic diseases.

March was an exciting month for HD research, filled with groundbreaking discoveries, inspiring community events, and promising advancements toward better treatments. We hope you enjoyed this recap and feel as energized as we do about the increased pace of progress being made. Science is moving fast, and every step forward brings us closer to real treatments for HD families. Stay tuned to HDBuzz for the latest updates in HD research, and as always, thank you for being part of this journey with us!

The authors have no conflicts of interest to declare.For more information about our disclosure policy see our FAQ...

GLOSSARY

NfL biomarker of brain health

longitudinal study A study where each participant is looked at several times over a time period - unlike a cross-sectional study, where each participant is looked at only once

clinical trial Very carefully planned experiments designed to answer specific questions about how a drug affects human beings

therapeutics treatments

biomarker a test of any kind - including blood tests, thinking tests and brain scans - that can measure or predict the progression of a disease like HD. Biomarkers may make clinical trials of new drugs quicker and more reliable.

CRISPR A system for editing DNA in precise ways

HTT one abbreviation for the gene that causes Huntington's disease. The same gene is also called HD and IT-15
