Harness Therapeutics Nominates HRN001, a First-in-Class Drug Candidate for Huntington's Disease and Establishes Clinical Advisory Board

HRN001 is a potent and specific antisense oligonucleotide designed to drive controlled upregulation of FAN1, a genetically validated target in Huntington's disease

Clinical Advisory Board formed to support progression of HRN001 into first-in-human studies in 2027

Dr Andy Billinton, CSO will present preclinical data at the upcoming CHDI Foundation Huntington's Disease Therapeutics Conference

Cambridge, UK, 16 February 2026: Harness Therapeutics ('Harness'), a biotechnology company unlocking previously undruggable targets to transform the treatment of neurodegenerative diseases, today announces the nomination of HRN001 as its lead drug candidate for Huntington's disease and the formation of a clinical advisory board to support the programme's advancement towards clinical evaluation.

Huntington's disease (HD) is a devastating, inherited neurodegenerative disorder that leads to progressive cognitive, psychiatric and motor decline, with death often occurring within 15 years of symptom onset. Despite significant advances in understanding HD disease biology, there are currently no approved disease-modifying treatments available.

HD is caused by the expansion of CAG repeats in the huntingtin (HTT) gene. Ongoing somatic expansion of these repeats is now recognised as a key driver of disease onset and progression. FAN1 nuclease has emerged as one of the most compelling targets to suppress somatic expansion, demonstrating the strongest genetic association to disease onset in genome-wide association studies.

Our first-in-class candidate HRN001 is a potent and specific antisense oligonucleotide targeting FAN1, designed to drive controlled upregulation of this key DNA repair nuclease. It leverages Harness' proprietary MISBA® (microRNA site blocking ASO) platform, which enables precise upregulation of target protein levels without risk of over-expression.

HRN001 has demonstrated robust upregulation of FAN1 and slowing of somatic expansion in models of HD, as well as favourable PK and tolerability characteristics. Preclinical development will continue throughout 2026 to support clinical entry in 2027. Harness is exploring the potential of the MISBA® platform in other triplet repeat disorders and across a broader pipeline of neurodegenerative disorders.

To support the progression of HRN001 towards the clinic, Harness has established a clinical advisory board (CAB) comprising leading experts in the HD field, including:

Dr. Irina Antonijevic (Chair) – Chief Medical Officer, Trace Neuroscience
Dr. Anne Rosser – Professor of Clinical Neuroscience, Cardiff University
Dr. Jeffrey Long – Professor of Psychiatry and Biostatistics, University of Iowa Health Care
Dr. Ralf Reilmann – Founding Director and Chief Executive Officer, George-Huntington-Institute

Dr. Roger Barker – Professor of Clinical Neuroscience, University of Cambridge
Dr. Sarah Tabrizi – Professor of Clinical Neurology, University College London
Dr. William Gray – Professor of Functional Neurosurgery, Cardiff University

The CAB will provide strategic guidance on clinical development, trial design, and translational strategy as the programme advances towards the clinic.

Dr Andy Billinton, Chief Scientific Officer of Harness Therapeutics, will present the company's work in HD at the upcoming annual CHDI Huntington's Disease Therapeutics Conference in Palm Springs, California, 23rd-26th February, providing further insights into the scientific rationale and development progress for HRN001.

Dr Jan Thirkettle, CEO of Harness Therapeutics, commented: *"The nomination of HRN001 represents a pivotal milestone for Harness and underscores our commitment to the Huntington's disease community. By precisely upregulating FAN1, a target with compelling genetic validation in delaying disease onset, HRN001 represents a differentiated, first-in-class therapeutic approach for addressing somatic expansion, a fundamental driver of disease progression. The formation of a Clinical Advisory Board brings deep clinical and translational expertise to the programme. The CAB will work closely with Harness as we advance HRN001 towards the clinic and seek to deliver a truly disease-modifying therapy for patients and families living with Huntington's disease."*

Dr Irina Antonijevic, Chair of Harness Therapeutics' Clinical Advisory Board, added: *"FAN1 is one of the most compelling and consistently validated genetic modifiers of Huntington's disease identified to date, with a clear mechanistic link to somatic expansion and disease progression. Harness' approach with HRN001 offers a novel and highly targeted way to therapeutically modulate this pathway. We look forward to advising the Company, as it advances HRN001 toward the clinic, translating this promising science into a clinical programme that could meaningfully alter the course of this devasting disease."*

CHDI presentation details
Title: Upregulation of FAN1 with ASOs as a potential therapeutic strategy for triplet repeat disorders
Session: Somatic Instability/Mismatch Repair
Date: Tuesday 24 February
Presenter: Dr Andy Billinton, CSO of Harness Therapeutics

CAB biographies

Dr. Irina Antonijevic (Chair) is an experienced clinician and drug development leader, widely recognized for her leadership in advancing innovative therapies from early development through clinical stages, particularly in areas with high unmet medical needs such as neurodegenerative and psychiatric disorders. She currently serves as Chief Medical Officer at Trace Neuroscience, bringing over three decades of experience in clinical psychiatry, neurology, academic research, and therapeutic drug development. Dr. Antonijevic is a board-certified physician trained in both psychiatry and neurology, with deep expertise in translational medicine and clinical development strategies.

Dr. Anne Rosser is Professor of Clinical Neuroscience at Cardiff University and an Honorary Consultant Neurologist at the University Hospital of Wales, leading the South Wales clinical service for Huntington's disease. She co-leads the Cardiff University Brain Repair Group, a multidisciplinary research team focused on developing cell-based and regenerative therapies for neurodegenerative diseases. Dr. Rosser previously served as Chair of the European Huntington's Disease Network (EHDN) and is currently Chair of the Scientific Committee of the Lister Institute of Preventive Medicine.

Dr. Jeffrey D. Long is Professor of Psychiatry and Biostatistics at the University of Iowa Health Care / Carver College of Medicine where he applies statistical modeling to neurodegenerative diseases, with a special emphasis on Huntington's disease. He develops and refines disease progression indexes, critical for understanding natural disease trajectories and planning clinical trials. Dr Long also serves as Lead Biostatistician for NIH-funded longitudinal studies, such as REDICT-HD, and plays a key role as Head Biostatistician in the Data Coordinating Center of the NeuroNEXT clinical trials network.

Dr. Ralf Reilmann is Founding Director and Chief Executive Officer of the George-Huntington-Institute (GHI) in Münster, Germany, a private research institute devoted to clinical care and research in Huntington's disease. Under his leadership, the Institute participates in key Huntington's observational studies including ENROLL-HD and clinical trials exploring disease-modifying therapies and gene therapy approaches. Dr. Reilmann is a board-certified neurologist, widely recognized for his commitment to improving trial outcomes, patient care, and global collaboration in Huntington's research.

Dr. Roger Barker is Professor of Clinical Neuroscience at the University of Cambridge and Honorary Consultant Neurologist at Addenbrooke's Hospital, Cambridge. He serves as Lead Academic Scientist for the Alzheimer's Research UK Drug Discovery Institute in Cambridge and Lead Consultant for the East of England Huntington's Disease Service. His research focuses on clinical characterization of neurodegenerative disorders and the development of new therapeutic approaches, especially cell- and gene-based treatments. Dr. Barker has led and coordinated major research programmes, including European transplant projects and stem cell trials for Parkinson's and Huntington's disease.

Dr. Sarah Tabrizi is Professor of Clinical Neurology at University College London, internationally recognised for her research in neurodegenerative diseases, particularly Huntington's disease. She holds several prestigious roles across UCL's neuroscience community and the UK's top clinical research institutions recognised with many major awards. Her work has transformed the Huntington's field, establishing frameworks for disease staging, biomarker development, and therapeutic trials that serve as models for other neurodegenerative conditions. Dr. Tabrizi's bench-to-bedside approach integrates deep biological insight with clinical trials to bring hope to patients with previously untreatable conditions.

Dr. William Gray is Professor of Functional Neurosurgery at Cardiff University and Honorary Consultant Neurosurgeon at the University Hospital of Wales. He brings dual expertise in clinical neurosurgery and neuroscience research, especially focused on disorders like epilepsy, Parkinson's disease, Huntington's disease, and frontotemporal dementia. He serves as Director of the Advanced NeuroTherapies Centre (ANTC), developing and delivering emerging therapies directly to the brain, is the Academic Lead in Neurosurgery in Wales, and Head of the Brain Repair and Intracranial Neurotherapeutics (BRAIN) Unit. Dr. Gray is the UK Chief Investigator for the uniQure AMT-130 gene therapy trial in Huntington's disease and Principal Investigator for gene therapy trials in frontotemporal dementia and Parkinson's disease. He also co-leads the European Huntington's Disease Network ATMP Working Group and chairs its Surgical Delivery Task Force.

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About Harness Therapeutics

Harness Therapeutics is a biotechnology company focused on unlocking previously undruggable targets to transform the treatment of neurodegenerative diseases. Its MISBA® platform technology enables precise upregulation of target protein levels by modulating mechanisms controlling protein synthesis. Harness' recently announced MISBA® Duo technology enables the design of single

constructs which simultaneously upregulate one target whilst downregulating a second. Using its deep understanding of post-transcriptional regulation and sophisticated neuron-based models, Harness' approach allows drugging of potentially disease-modifying targets, which would not be addressable using gene therapy or other modalities.

Its lead candidate in Huntington's Disease, HRN001, targets FAN1 nuclease, a key protective protein, shown to slow disease progression. Its pipeline also includes programmes addressing compelling first in class targets for ALS, Alzheimer's Disease, and Parkinson's Disease.

Harness has assembled a world-class team, supported by leading experts in neurodegeneration and RNA biology, and is based in Cambridge, UK. Its leading life science investors include the foundational investors Takeda Ventures and SV Health Investors' Dementia Discovery Fund, alongside Epidarex Capital and Ono Ventures Investment.

For more information, please visit https://www.harnesstx.com/ and follow us on LinkedIn.

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