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# Vertex Establishes Early-Stage Huntington's Disease Research Collaboration with CHDI Foundation, Inc.

-Vertex to receive up to \$4 million in funding for Huntington's disease research-

-Novel venture philanthropy model used in Vertex's Cystic Fibrosis R&D efforts to support new Huntington's disease research-

-Vertex's San Diego research and development site to lead Huntington's disease research efforts-

**San Diego, CA, June 17, 2008** — Vertex Pharmaceuticals Incorporated and CHDI Foundation, Inc. (CHDI) today announced a new collaboration aimed at developing assays for use in discovering novel compounds for the treatment of Huntington's disease, a genetic neurodegenerative disease that affects approximately 30,000 people in the United States. CHDI is a non-profit foundation committed to accelerating the discovery and development of new drugs that delay the onset or slow the progression of Huntington's disease.

"As a proven leader in the field of drug discovery and development, Vertex brings unique scientific experience to CHDI's network of researchers that will contribute to our mission to discover novel compounds for the treatment of Huntington's disease," said Robert Pacifici, Ph.D., Chief Scientific Officer for CHDI. "Vertex's approach, to directly target the protein folding aberrations that are involved in Huntington's disease, illustrates their commitment to tackle serious diseases in unconventional ways. We look forward to working closely with Vertex to potentially discover new therapies for this devastating disease."

As part of the collaboration, CHDI will provide Vertex up to approximately \$4 million of research funding through the first quarter of 2010. Under the agreement, Vertex will work closely with CHDI using those elements of CHDI's extensive network of academic researchers and Huntington's disease research assets that have direct relevance to this collaboration. Vertex will retain rights to all discoveries that are the result of the collaboration and will license related tools back to CHDI for use in further Huntington's disease research.

Vertex's collaboration with CHDI will be focused on two main areas: (1) errant protein folding and aggregation, a key cellular disruption that is believed to play a role in Huntington's disease, and (2) neurogenesis, a process that when disrupted, can result in Huntington's disease-related cognitive and physical impairments. Multiple neurodegenerative diseases, such as Alzheimer's disease, Parkinson's disease and Amyotrophic Lateral Sclerosis (ALS), are believed to be partially caused by protein mis-folding. In Huntington's disease, a mutation in the huntingtin gene is believed to affect how proteins related to the progression of the disease fold. The primary goal of the collaboration will be to design at least one cellular assay, or test, that will be used to investigate the impact of mutant huntingtin on protein folding, which will enable screening of small molecule compounds that may reverse those effects. Researchers at Vertex's San Diego-based research and development site will conduct the majority of the Huntington's disease research using protein folding technologies and capabilities unique to that facility.

"This research collaboration represents a promising opportunity for Vertex and CHDI to discover novel therapies for Huntington's disease and will allow Vertex to gain further experience in the underlying processes of other serious neurodegenerative diseases," said Peter Mueller, Ph.D., Executive Vice President, Drug Innovation and Realization, and Chief Scientific Officer at Vertex. "Vertex has demonstrated an ability to work collaboratively and productively with other non-profit disease foundations, and we look forward to this collaboration with CHDI. By applying Vertex's expertise in studying and modulating intracellular protein folding, we believe these new efforts in Huntington's disease may shed light on ways to proactively intervene in the disease processes and potentially slow or halt the progression of Huntington's disease."

## About Huntington's disease

Huntington's disease is a familial disease caused by a mutation in the huntingtin gene passed on from a parent to a child. Each child of a parent with Huntington's disease has a 50-50 chance of inheriting the mutation. As a result of carrying the gene for Huntington's disease brain cells fail and die leading to cognitive and physical impairments that, over the course of the disease, isolate and constrain the individual. Symptoms usually develop in midlife, but can also develop in infancy or old age. Once overt symptoms start, patients live about 15 to 20 years. One person in 10,000 is believed to carry a Huntington's disease mutation in the huntingtin gene. There is currently no way to stop, slow or reverse the course of Huntington's disease.

## **About CHDI Foundation, Inc.**

CHDI Foundation is a non-profit organization supporting an international network of research laboratories from academia and industry pursuing novel ways to treat Huntington's disease with drugs. Its virtual pharma approach is designed to bring the right collaborators together to identify and address critical scientific issues and move drug candidates to clinical evaluation as quickly as possible.

## **About Vertex**

Vertex Pharmaceuticals Incorporated is a global biotechnology company committed to the discovery and development of breakthrough small molecule drugs for serious diseases. The Company's strategy is to commercialize its products both independently and in collaboration with major pharmaceutical companies. Vertex's product pipeline is focused on viral diseases, inflammation, autoimmune diseases, cancer, pain and cystic fibrosis. Vertex co-discovered the HIV protease inhibitor, Lexiva, with GlaxoSmithKline.

Lexiva is a registered trademark of the GlaxoSmithKline group of companies.

Vertex's press releases are available at <u>www.vrtx.com</u>.

## Safe Harbor Statement

This press release contains forward-looking statements, including the statement that this collaboration could result in the discovery of novel compounds for the treatment of Huntington's disease, could shed light on ways to proactively intervene in the disease processes to potentially slow or halt the progression of Huntington's disease, or could result in the design of cellular assays useful in the drug discovery process for Huntington's disease. While the Company believes the forward-looking statements contained in this press release are accurate, there are a number of factors that could cause actual events or results to differ materially from those indicated by such forward-looking statements. Those risks and uncertainties include, among other things, the possibility that our research efforts will be unsuccessful for scientific or operational or other reasons, or that any assays ultimately designed under this collaboration will not prove useful in the Securities and Exchange Commission and available through the Company's website at <u>www.vrtx.com</u>. We disclaim any obligation to update the information contained in this press release as new information becomes available.

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