Huntington's Disease Regulatory Science Consortium (HD-RSC)
Kick-Off Meeting

Sheraton Silver Spring Hotel
Silver Spring, MD
November 6-7, 2017

OVERVIEW AND OBJECTIVES

Critical Path Institute and CHDI Foundation welcome participants from the pharmaceutical industry, academic key opinion leaders, regulators and advocacy groups to the kick-off meeting to form the Huntington's Disease Regulatory Science Consortium (HD-RSC). The objectives of the meeting are to solicit input from stakeholders on HD-RSC plans and deliverables and to develop an understanding of 1) the value of a pre-competitive consortium model to advance regulatory science that can enable drug development in HD; 2) the critical importance of data contribution toward advancing regulatory science, and for the success of this consortium.

AGENDA

Day 1 - Monday, November 6, 2017
Magnolia Room

8:30 a.m. – 9:00 a.m.	**Coffee/Continental Breakfast**
9:00 a.m. – 9:20 a.m.	**The Vision: CHDI's Dedication to Successful HD Treatments** Robi Blumenstein, CHDI Foundation (*President*)
9:20 a.m. – 9:40 a.m.	**Consortia-Based Strategies in Neurodegenerative Diseases: Critical Path Institute's Track Record in Collaborative Efforts** Martha Brumfield, Critical Path Institute (*President and Chief Executive Officer*)
9:40 a.m. – 10:15 a.m.	**The Need: What Are the Key Challenges for Drug Development in HD?** ***Moderator****:* Jeff Carroll, Western Washington University (*Associate Professor, Department of Psychology*) - Mike Panzara, Wave Live Sciences (*Franchise Lead, Neurology*) - Scott Schobel, Roche (*Translational Medicine Leader*) - Louise Vetter, HDSA (*Chief Executive Officer*) - Juliana Bronzova, EHDN (*Science Director*) - Mark Gordon, Teva (*Senior Director, Clinical Development*)
10:15 a.m. – 10:35 a.m.	**Regulatory Impact for Huntington's Disease: FDA Perspectives** Billy Dunn, FDA (*Director, CDER*, *Division of Neurology Products*)
10:35 a.m. – 10:50a.m.	**Break**

10:50 a.m. – 11:05 a.m.	**Regulatory Impact for Huntington's Disease: EMA Perspectives** Manuel Haas, EMA (*Head of CNS, Evaluation Division*) (Remote)
11:05 a.m. – 11:25 a.m.	**Model Informed Drug Development** Issam Zineh, FDA (*Office Director, Office of Clinical Pharmacology*)
11:25 a.m. – 11:45 a.m.	**From Model-Based Clinical Trial Enrichment to Comprehensive Clinical Trial Simulation** Brian Corrigan, Pfizer (*Vice President, Global Head, Clinical Pharmacology*)
11:45 a.m. – 12:30 p.m.	**Lunch**

Developing a Comprehensive Quantitative Description and Understanding of Disease Progression in Manifest HD

12:30 p.m. – 12:50 p.m.	**Success in Sharing Data from HD Natural History Studies** Amrita Mohan, CHDI Foundation (*Director, Clinical Bioinformatics*)
12:50 p.m. – 1:30 p.m.	**HD Disease Progression Modeling: Aligning Data with Context of Use Applications** ***Moderator****:* Klaus Romero, Critical Path Institute (*Director, Clinical Pharmacology and Quantitative Medicine*) - Brian Corrigan, Pfizer (*Vice President, Global Head, Clinical Pharmacology*) - Bernhard Landwehrmeyer, Ulm University Hospital (*Professor of Neurology "Clinical Neurobiology," Department of Neurology*) - Kevin Krudys, FDA (*Lead Pharmacologist, CDER, Division of Pharmacometrics, Office of Clinical Pharmacology*) - Gerald Podskalny, FDA (*Medical Officer, CDER, Division of Neurological Products*) - Charles Venuto, University of Rochester (*Clinical Pharmacologist, Department of Neurology in the Center for Human Experimental Therapeutics*)
1:30 p.m. – 1:45 p.m.	**Break**
1:45 p.m. – 2:05 p.m.	**Rationale and Impact of Building a Comprehensive HD Clinical Database** Cristina Sampaio, CHDI Foundation (*Chief Medical Officer*)
2:05 p.m. – 2:25 p.m.	**Biomarkers as Tools to Enable Decision-Making in HD Drug Development** Eric Siemers, Eli Lilly (*Distinguished Medical Fellow, Alzheimer's Disease Platform Team*)
2:25 p.m. – 2:45 p.m.	**Clinical Outcome Measures in HD: Beyond UHDRS** Glenn Stebbins, Rush University (*Professor, Department of Neurological Sciences*)

2:45 p.m. – 3:45 p.m.	**Outlining a Roadmap for Clinical Trials in Pre-Manifest HD** ***Moderator****:* Karl Kieburtz, University of Rochester (*Professor of Neurology*) - Steve Hersch, Voyager (*Senior Director, Clinical Development*) - Billy Dunn, FDA (*Director, CDER, Division of Neurology Products*) - Eric Bastings, FDA (*Deputy Director, CDER, Division of Neurology Products*) - Bernard Ravina, Voyager (*Chief Medical Officer*)
3:45 p.m. – 4:00 p.m.	**Break**
4:00 p.m. – 4:30 p.m.	**The Impact: Why This Matters to Patients** Charles Sabine, Patient Advocate (*Former Emmy-awarded NBC News journalist and high-profile spokesman for the global HD community*)
4:30 p.m.	**Conclusion of Day 1 Meeting**
6:00 p.m.	**Dinner at Copper Canyon Grill**

AGENDA

Day 2 - Tuesday, November 7
Magnolia Room

The objective of Day 2 is to create a forum for open and engaging dialogue with experts and regulatory agency representatives focused on proposed HD-RSC activities. The focus of all breakout sessions will be aimed at data-driven strategies to address the current issues in HD clinical drug development.

8:00 a.m. – 8:30 a.m.	**Coffee/Continental Breakfast**
8:30 a.m. – 9:00 a.m.	**Summary and Highlights from Day 1; Objectives for Day 2** Emily Gantman, CHDI Foundation (*Director, Strategic Projects and Planning*)
9:00 a.m. – 9:30 a.m.	**The Operations: How HD-RSC Will Work** Diane Stephenson, Critical Path Institute (*Acting Director, Huntington's Disease Regulatory Science Consortium* (*HD-RSC) and Executive Director, Critical Path for Parkinson's* (*CPP*) *Consortium*) Debra Hanna, Critical Path Institute (*Executive Director, Critical Path to TB Drug Regimens* (*CPTR) Consortium*)
9:30 a.m. – 9:45 a.m.	**Q&A**
9:45 a.m. – 10:00 a.m.	**Introduction to Breakout Sessions** Diane Stephenson, Critical Path Institute
10:00 a.m. – 10:15 a.m.	**Break**
10:15 a.m. – 12:00 p.m.	**Breakout Groups** Meeting participants will be asked to sign up to participate in one of the three breakout sessions at the ***registration desk***. ***Proposed Breakout Session Topics Include:*** **1) Model-based Strategies for Clinical Trial Enrichment: Focus on Biomarkers** **Room: Magnolia** ***Moderators***: - Jeffrey Long, University of Iowa (*Professor of Psychiatry and Biostatistics, Department of Psychology*) - Andrew Wood, CHDI Foundation (*Vice President, Clinical Neuroimaging Research*) - Jackson Burton, Critical Path Institute (*Assistant Director, Quantitative Medicine*)

2) **Clinical Outcome Assessment Measures in HD for Use in Trials**
Room: Persimmon 1
Moderators:
- Julie Stout, Monash University (*Director, Clinical Cognitive Neuroscience Laboratory*)
- Rebecca Fuller, CHDI Foundation (*Director, Clinical Outcomes*)
- Daniela Conrado, Critical Path Institute (*Associate Director, Quantitative Medicine*)

3) **Therapeutic Development in Pre-manifest HD: Opportunities and Challenges**
Room: Persimmon 2
Moderators:
- Blair Leavitt, University of British Columbia (*Interim Director, Centre for Molecular Medicine & Therapeutics and Professor, Department of Medical Genetics*)
- Cristina Sampaio, CHDI Foundation (*Chief Medical Officer*)
- Diane Stephenson, Critical Path Institute (*Acting Director, Huntington's Disease Regulatory Science Consortium* (*HD-RSC*)

12:00 p.m. – 12:45 p.m.	**Report Out Summaries from Breakout Session Moderators** Recommendations and themes from each of the breakout sessions will be communicated to the larger group (*15 minutes each*)
12:45 p.m. – 1:00 p.m.	**Meeting Summary and Conclusions** Cristina Sampaio, CHDI Foundation (*Chief Medical Officer*)
1:00 p.m.	**Meeting Adjourns** (*box lunches to go*)