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. – 10:50 a.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**

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**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)**