HD Regulatory Science Consortium (HD-RSC)

A consortium aimed at accelerating treatments for Huntington’s disease

Critical Path Institute / CHDI Foundation
The overall goal of this initiative is to create a regulatory science strategy for HD, offering additional incentive and de-risking for HD therapeutic development by all stakeholders. The HD Regulatory Science Consortium (HD-RSC) will provide a forum and structure to bring together the necessary participants from the HD community for data contribution and tool development, leading to efficiencies in development of new therapies.
HD-RSC Proposed Governance Structure

Critical Path Institute
- Biotech Companies
- Pharmaceutical Companies
- Huntington Study Group
  European HD Network (EHDN)
- Philanthropies

CHDI Foundation
- Government Agencies
- Regulatory Agencies
- Academic Advisors/Universities
- Patient Organizations

Executive Director
- Co-Director

COordinating Committee

Working Group 1
- HD Data
  (CDISC Standards, data management and integration)

Working Group 2
- HD Modeling
  (Model-based clinical trial enrichment platform)

Working Group 3
- Biomarker

Working Group 4
- Clinical Outcomes Assessment

Working Group 5
- HD Science Forum
  (White papers, points to consider, etc.)
C-Path Consortia

Fifteen global consortia collaborating with 1,450+ scientists and 84 organizations

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<th>Consortium</th>
<th>Focus</th>
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<td>Coalition Against Major Diseases</td>
<td>Focusing on diseases of the brain</td>
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<td>Coalition For Accelerating Standards and Therapies</td>
<td>Data standards</td>
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<td>Critical Path for Parkinson’s Consortium</td>
<td>Enabling clinical trials in Parkinson’s Disease</td>
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<td>Critical Path to TB Drug Regimens</td>
<td>Accelerating the development of TB drug regimens and diagnostics</td>
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<td>Duchenne Regulatory Science Consortium</td>
<td>Duchenne Muscular Dystrophy</td>
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<td>Huntington's Disease Regulatory Science Consortium</td>
<td>Expediting approval of Huntington’s therapeutics</td>
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<td>International Neonatal Consortium</td>
<td>Neonatal clinical trials</td>
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<td>Multiple Sclerosis Outcome Assessments</td>
<td>Drug Effectiveness in MS</td>
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<td>Polycystic Kidney Disease Outcomes Consortium</td>
<td>New imaging biomarker for PKD</td>
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<td>Patient-Reported Outcome Consortium</td>
<td>Assessing treatment benefit</td>
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<td>Electronic Patient-Reported Outcome Consortium</td>
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<td>Predictive Safety Testing Consortium</td>
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<td>Pediatric Trials Consortium</td>
<td>Developing effective therapies for children</td>
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<td>Type 1 Diabetes Consortium</td>
<td>Qualifying biomarkers for type 1 diabetes</td>
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<td>Transplant Therapeutics Consortium</td>
<td>New drug development tools for transplantation</td>
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- Biomarkers
- Clinical outcome assessment instruments
- Clinical trial simulation tools
- Data standards
- In vitro tools
Back up slides
HD-RSC Governance Founding Principles

• This HD initiative is uniquely positioned to address the current gaps in HD drug development and assist industry as they deliver new therapies to patients in need

• The goals of the consortium are to achieve consensus in advancement of HD drug development tools; data sharing is key to success

• Members are expected to provide in kind contributions in terms of sharing data and knowledge/expertise

• Confidentiality is fundamental to the principles of members when signing onto the HD-RSC membership agreement
Roles & Responsibilities of CPP Coordinating Committee

• Recommend subject matter expertise and contribute input as to the prioritization of consortia activities
• Review progress of workgroups, including recommending participants
• Review and provide input on manuscripts for publication and regulatory submissions (e.g. white papers, briefing packages, etc.)
• Assist with communication and coordination with relevant HD initiatives (e.g. NINDS HD biomarkers)
• Recommendation of HD-RSC Co-Chairs for the various working groups
### Proposed Initial Focus Areas and Working Groups

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<th>Working Group 1</th>
<th>Working Group 2</th>
<th>Working Group 3</th>
<th>Working Group 4</th>
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- **Working Group 1**
  - CDISC data standards for HD.
  - Identify and acquire key HD datasets.
  - Aggregate HD clinical data into C-Path Online Data Repository (CODR).
  - Enable stakeholder access to the integrated HD Database as defined by data contributors.

- **Working Group 2**
  - HD disease progression model in manifest HD.
  - Model-based clinical trial enrichment tool for clinical trial design.
  - EMA and FDA regulatory acceptance paths.
  - HD-RSC modeling publication strategy.

- **Working Group 3**
  - Prioritize leading candidate HD biomarkers.
  - Seek input from FDA and EMA on HD candidate biomarkers.
  - Develop and deliver on a HD-RSC biomarkers publication strategy.

- **Working Group 4**
  - Identify expectations for reliable and clinically-meaningful outcome measures acceptable for use in HD clinical trials.

- **Working Group 5**
  - Prepare points to consider white paper focused on advancing therapies in pre-manifest HD.
All Proposed HD-RSC Working Groups are Data-Driven

- Working Group 1: HD Data (CDISC Standards, data management and integration)
- Working Group 2: HD Modeling (Model-based clinical trial enrichment platform)
- Working Group 3: HD Biomarkers
- Working Group 4: HD Clinical Outcomes Assessments
- Working Group 5: HD Science Forum (White papers, points to consider, etc.)