

## New Collaboration Seeks to Speed Huntington's Disease Drug Licensing

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### Critical Path Institute launches new initiative to get HD drugs licensed as quickly as possible

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Recently the Critical Path Institute announced a new effort – the Huntington's Disease Regulatory Science Consortium, or HD-RSC. This collaboration with many international partners aims to speed the development of new HD treatments. What's all this about, and how could it help HD patients?

### What is C-Path?

The Critical Path Institute is likely new to most Huntington's disease community members, but they're not new to the fight against human diseases.



Regulatory agencies like the FDA and EMA need very specific evidence before drugs will get licensed. The HD-RSC will work to speed up this process.

In 2004, the US [Food and Drug Administration](#)(FDA) launched an effort to modernize and speed the development of new therapies. As a result of that effort, in 2005, the FDA and partners founded and funded the Critical Path Institute (or C-Path) with a goal of speeding the development and reducing the costs of bringing new drugs to patients who need them.

To do its work, C-Path brings together teams of organizations that are working on a specific problem. These **C-Path consortia** work to streamline drug trials in their area of interest, as well as developing new tools to improve the quality of trials.

As an example, C-Path has a consortium focused on Alzheimer's Disease which they call **Critical Path for Alzheimer's Disease, or CPAD**. This consortium of regulatory agencies, drug companies, researchers and advocates focuses on developing new tools to accelerate the development of new therapies for Alzheimer's Disease.

## What does C-Path do?

How do groups like CPAD work to improve or speed clinical trials? As an example, CPAD has developed a sophisticated computer model that tracks the progression of Alzheimer's Disease by compiling huge amounts of existing information about Alzheimer's Disease patients. Alzheimer's Disease clinical trials are done by a number of different individual organizations, and the data is not always shared between trials.

This is where C-Path comes in – they specialize at pulling together information about the disease stored with drug companies and academic researchers around the world. Based on real data from dozens of drug trials, C-Path's computer model allows organizations thinking of developing a new Alzheimer's Disease model to essentially do a practice run of their trial in a computer simulation.

This allows any organization with a new Alzheimer's Disease drug to plug in a few numbers about how well they think their drug will work, and get feedback about how likely a given trial design is to be successful. That can be enormously powerful for trying to figure out how many patients to enroll in a study, and how to divide the people in the study between the arms of the study.

C-Path's consortia have also worked on other critical roadblocks to rapidly completing trials. Several of their consortia have focused, for example, on helping researchers develop a new [biomarker](#) to simplify or shorten clinical trials.

There are a few kinds of biomarkers, but ultimately they are precise measurements that serve to tell us about how a disease is progressing in a human – say, a brain scan for HD. They can also report on how well a drug is doing based on changes that drug leads to in body chemistry or other measurements.

Because of their history and membership, C-Path understands how regulators, like the FDA and the European Medicines Agency (EMA), think about drug approval. This puts them in a great position to translate the science that researchers generate, into the measurements and outcomes that regulatory agencies need, to prove that drugs are safe and effective.

## C-Path's Newest Consortium: HD-RSC

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Recently, the official launch of C-Path's newest consortium was announced – the **Huntington's Disease Regulatory Science Consortium**, or HD-RSC. The consortium was launched in partnership with the CHDI foundation, a non-profit dedicated to rapidly developing meaningful treatments for HD.

The HD-RSC involves a huge range of players in the HD world beyond C-Path and CHDI, including drug companies (ten in fact, including Roche, Sanofi, Teva, Wave Life Sciences and others), regulatory agencies and patient advocacy organizations (including the HDSA, HSC and EHDN). These diverse organizations all have their own expertise and interests, but all of them are committed to developing new therapies for HD patients.

Last November, representatives from all these organizations came together in Silver Spring Maryland for a kickoff meeting. Your humble HDBuzz author attended to get a sense of what to expect from the HD-RSC. Over two days of meetings (program available at the link), dozens of participants talked about how best to speed clinical trials for HD.

Excitingly, the attendees included very high-ranking members of the FDA, including Eric Bastings (Deputy Director), and Billy Dunn (Division Director, Division of Neurology Products). These are the folks that are in charge of the review of new HD drugs being tested. Far from standing on the sidelines, the FDA attendees sat front and center in the first row, taking notes and asking a number of questions. It seems that these regulators get HD, and they're interested in trying to speed the safe development of new therapies for HD families. This consortium sums up the collaborative spirit of the HD community and our determination to make progress as quickly and efficiently as possible.

By the end of the meeting, the organizations comprising the HD-RSC had organized themselves into 5 working groups. Each of these smaller groups is focused on helping solve a specific problem – developing new biomarkers, for example.

Another exciting goal embraced by the HD-RSC is the problem of how to design trials of drugs for presymptomatic HD mutation carriers – meaning people who carry the HD mutation, but who don't yet have HD symptoms.

Stopping HD symptoms before they start is the goal for everyone working on HD, but it's complicated to understand how we would design a trial and monitor people who don't yet have any symptoms to measure. Excitingly, this working group suggests that some really savvy folks think this is an important problem and have agreed to spend time working on creative solutions.

## **Take home message for families**

We're entering a new phase in the fight against HD. Incredible drugs, designed specifically for HD, have moved from labs around the world into the clinic. The feeling many in the community had that “no one cares about HD” is being replaced by the realization that HD is a very hard, but solvable, problem.

Groups like the HD-RSC are great news for us because they mean that smart people are working hard, that they're collaborating and most importantly – that all these organizations feel that HD drug development is an exciting place to work. Stay tuned for exciting new ideas from the HD-RSC.