



Accelerating therapeutic
development for
Huntington's disease

Why CHDI reached out to C-Path

- or -

*How learning to play chess can inform efforts to bring effective
treatments for HD to patients*

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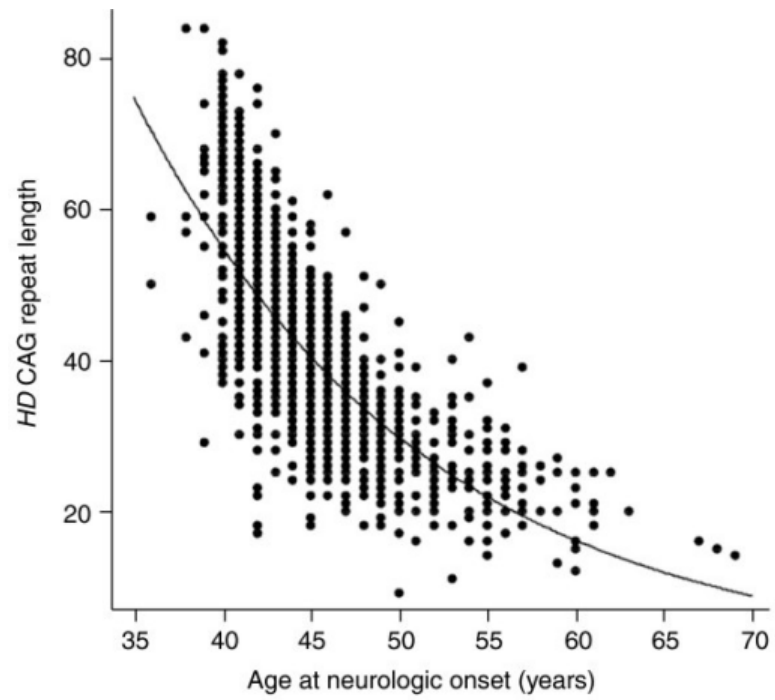
Admin

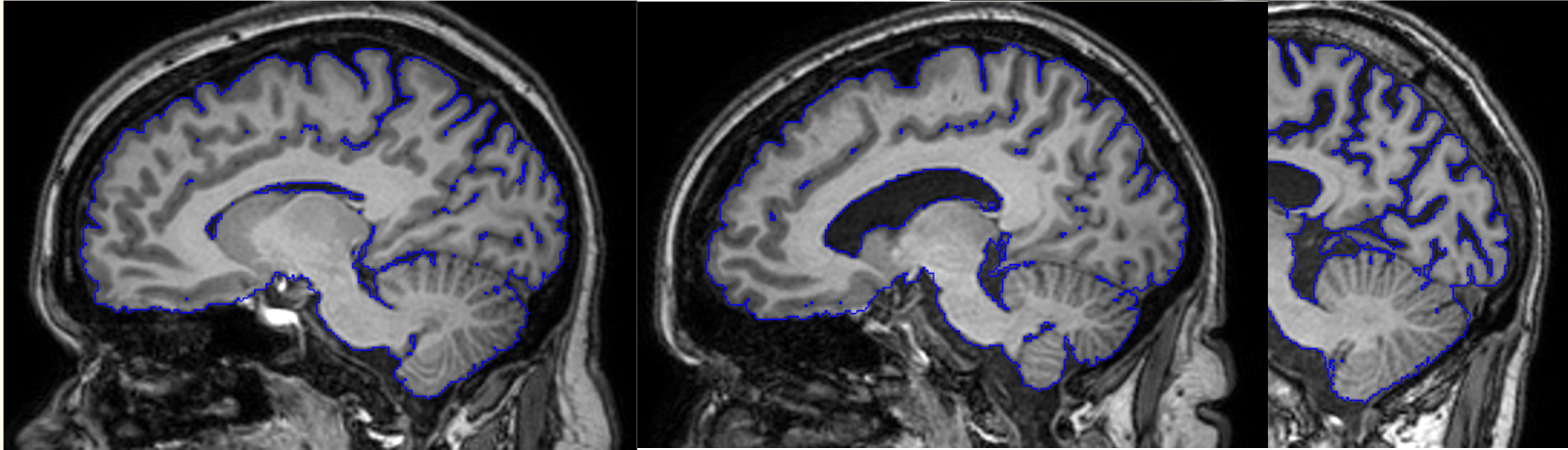
Ruth Basu

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Control

Pre Manifest

HD

TRACK **HD** 


**BOBBY FISCHER
TEACHES CHESS**

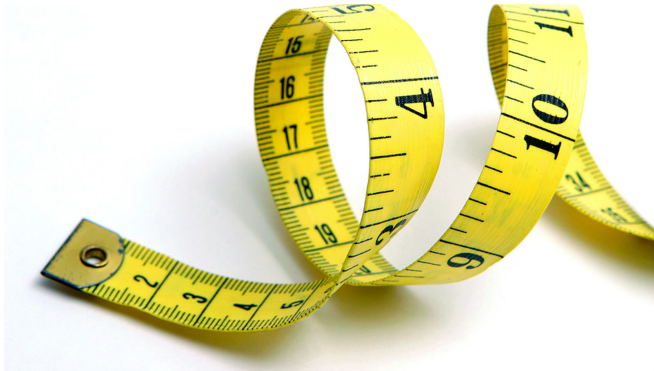
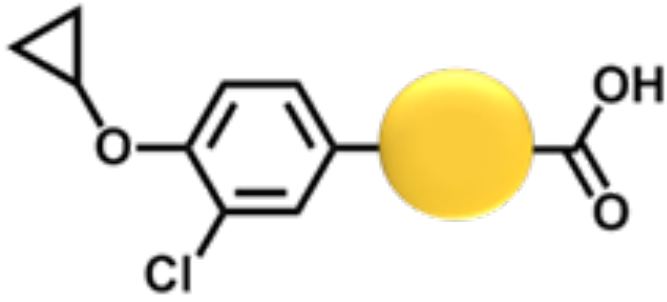




Control

Pre Manifest

HD



Estimates for a group that is all gene-expanded, with CAG lengths known

CAG age Model, 80% power, alpha = .05, 5% annual censoring

Magnitude of Therapeutic Effect

		10%	20%	30%	40%	50%
Years followed	3	19,509	4,690	1,997	1,071	649
	4	13,669	3,297	1,409	759	462
	5	10,321	2,496	1,070	578	353
	6	8,196	1,986	853	462	283

circa 2004

Guidance for Industry

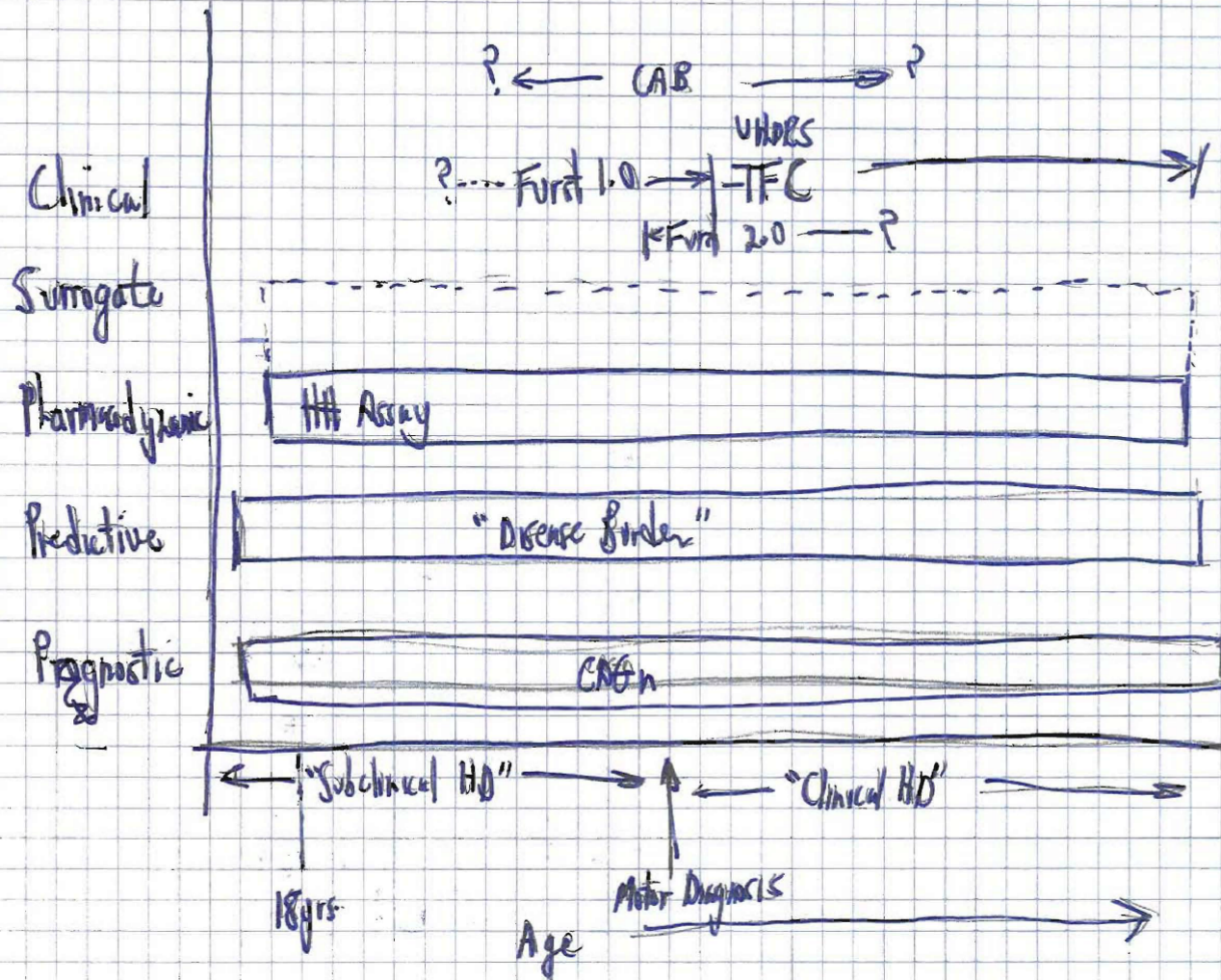
Qualification Process for Drug Development Tools

*Additional copies are available from:
Office of Communication
Division of Drug Information, WO51, Room 2201
Center for Drug Evaluation and Research
Food and Drug Administration
10903 New Hampshire Ave.
Silver Spring, MD 20993*



DDT-HD

January 26, 2011



Validation of a Prognostic Index for Huntington's Disease

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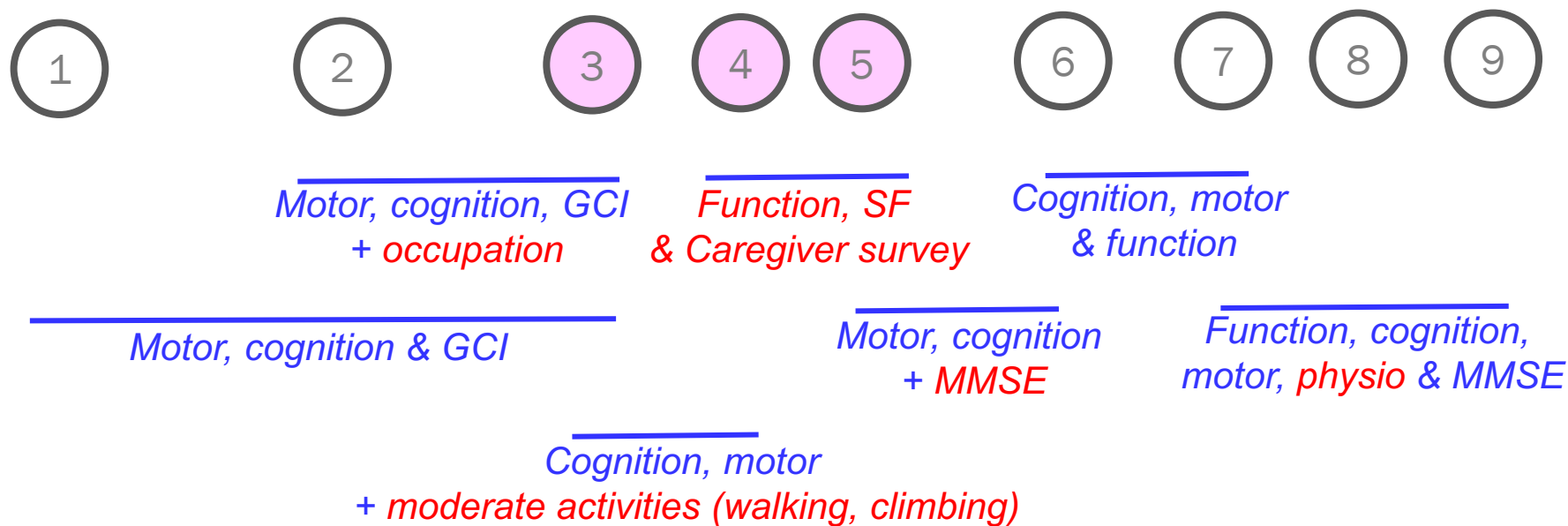
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ABSTRACT: Background: Characterizing progression in Huntington's disease is important for study the natural course and selecting appropriate partici-

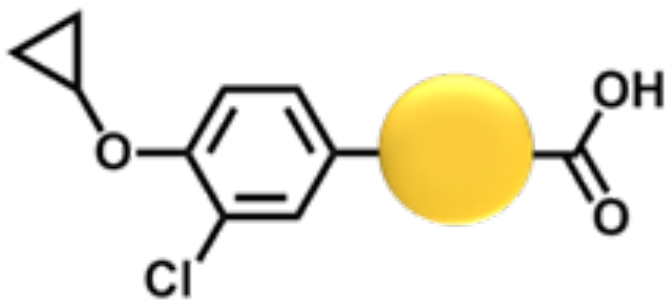
total motor score, Symbol Digit Modalities Test, base-line age, and cytosine-adenine-guanine expansion. External predictive performance was very good for the

Phenotype progression through States 1 - 9

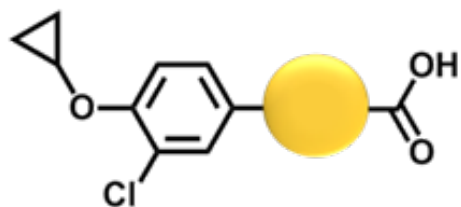


abc: new outcome measures

Distance between states proportional to empirical durations in years



A word on strategy... and hope



10^{20}



10^{120}

