



Cognition Research Update
Down Syndrome Research and Treatment Foundation

November 2nd 2013

Sarah Wernikoff
Chair, Board of Directors
DSRTF

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Our Mission

The mission of DSRTF is to stimulate and fund cognition research with **the goal of improving learning, memory, and speech** for individuals with Down syndrome in order that they:

- Participate more successfully in school
- Lead more active and independent lives
- Prevent or delay early cognitive decline (Alzheimer's Disease)

Our Strategy

Our Vision

- Not a cure, but development of safe and effective therapies to significantly improve Memory, Learning, Speech and Independence.

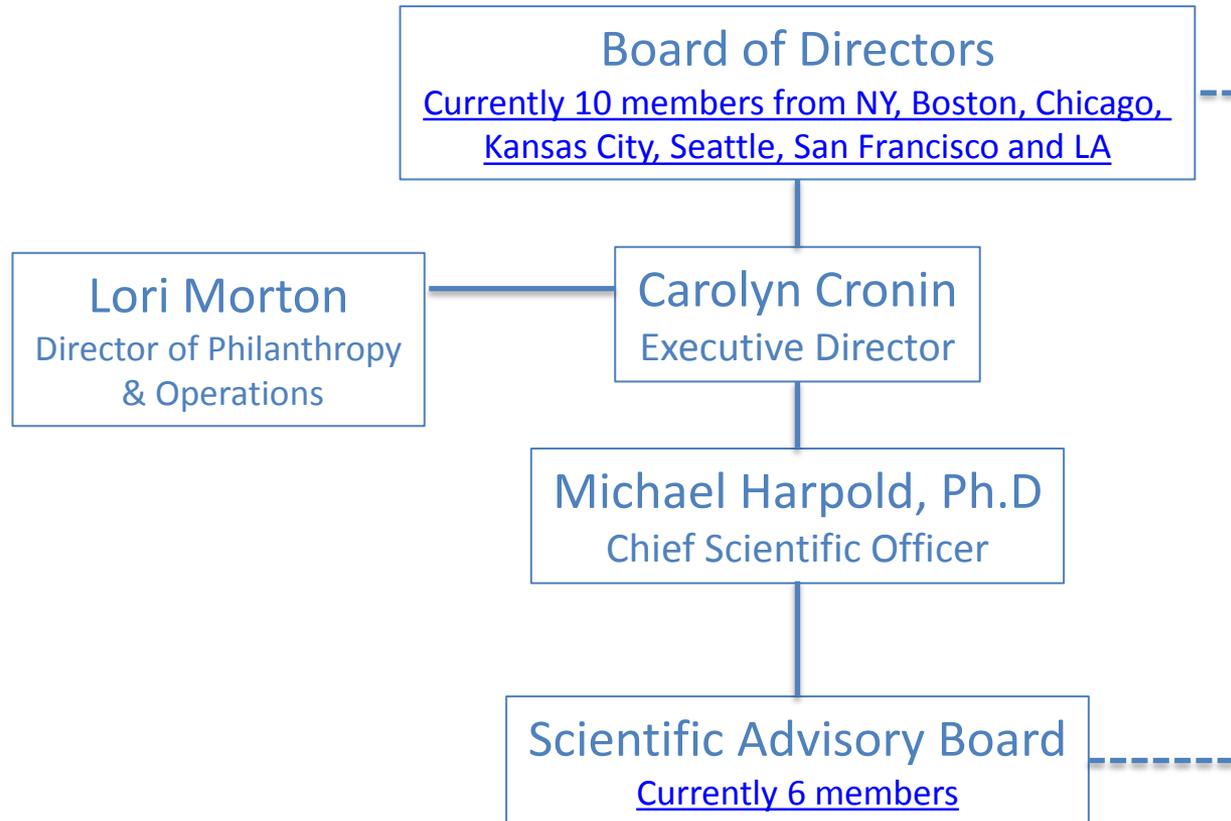
Our Values

- Narrow, scope & significant focus
- Collaboration and a multi-disciplinary approach
- Acceleration of translation from research to treatments

Overview: History

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| 2003 | Decoding of human genome opens door to cognitive research |
| 2004 | Mouse model for DS developed by researchers in London; DSRTF is founded |
| 2005 | DSRTF awards first grant to Dr. William Mobley of Stanford University |
| 2006 | First drug target, the APP gene, identified by Dr. William Mobley and his team, linking cognitive impairment in DS to the cognitive decline experienced in Alzheimer's disease |
| 2007 | DSRTF establishes its Scientific Advisory Board , the only scientific board in the US dedicated exclusively to cognition research for DS |
| 2008 | DSRTF announces supporters had held over 50 events in over 20 states since the organization's founding |
| 2008-2010 | DSRTF expands grant awards to include Johns Hopkins University, UC San Diego, University of Arizona, University of Texas, and the VA Hospital of Palo Alto |
| 2011 | Roche Pharmaceuticals begins clinical trial of the first potential therapy designed to improve cognition and adaptive behavior in individuals with Down syndrome |
| 2013 | TODAY: Over \$9M in funding provided, 9 drug targets identified and 3 clinical trials underway |

Overview: Organization



Overview: Why Cognition Research?

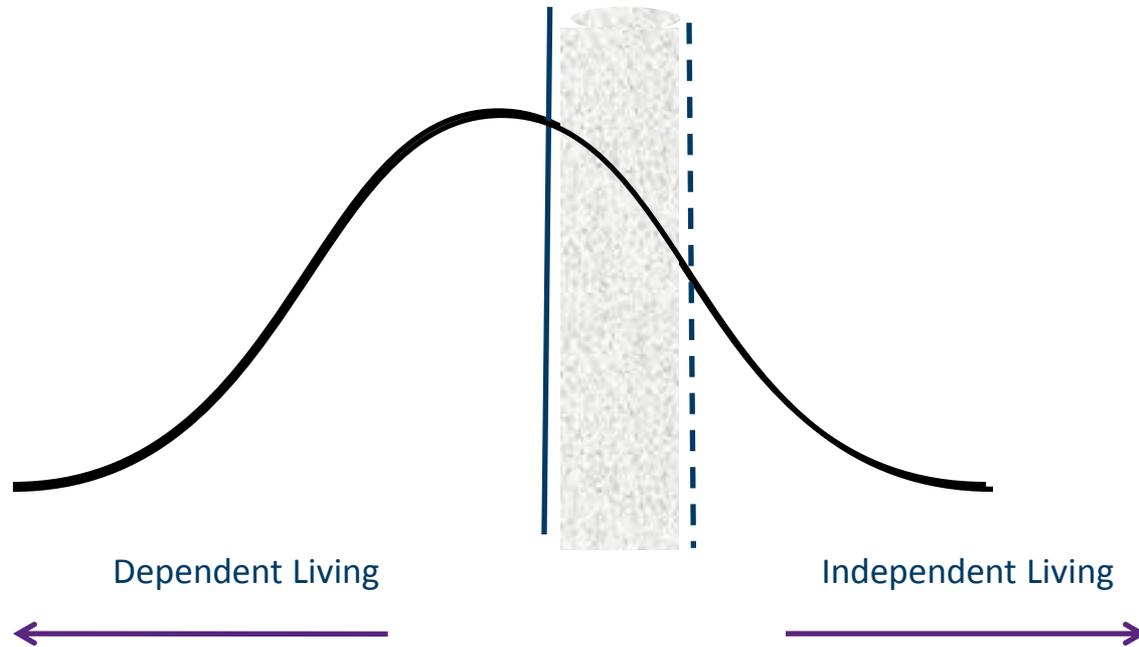
- Life expectancy of individuals with Down syndrome have more than ***doubled*** in the last 30 years – from 25 to nearly 60 years old.
- The majority of those with DS experience mild-to-moderate cognitive impairment, thus a small improvement = great impact to independence.
- Cognitive challenges are limiting throughout these longer lives, and typically increase with age:

Nearly 90% of individuals with DS develop the neuropathology of Alzheimer's disease by the age of 40.

- The decoding of the human genome in 2003 made this research possible, and last 8 years of progress tells us treatments are probable.

Overview: Our Goal

Moving the Curve Toward Greater Independence



Our Results and Clinical Trials

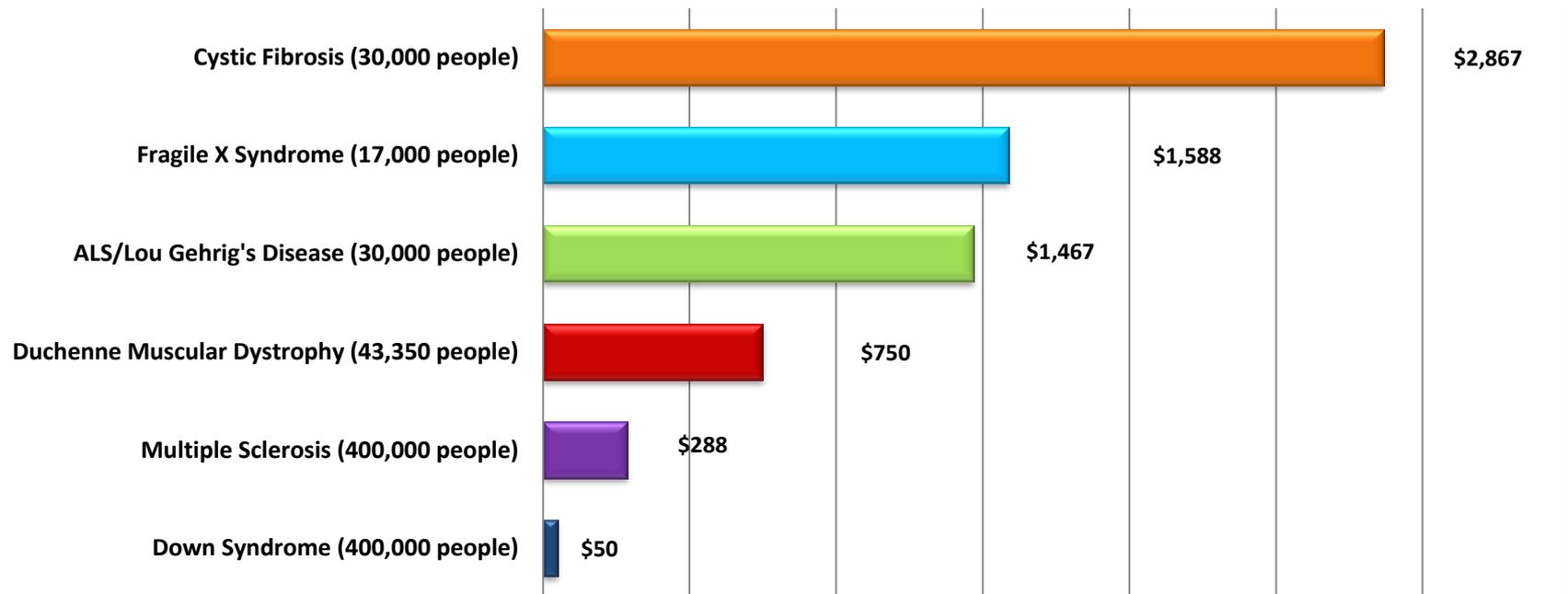
At DSRTF we are proud of our achievements during our initial 8 years.....

- Identification of 9 drug targets, leading to 3 drug candidates.
- Catalyst for the research that has lead to 3 clinical trials (ongoing) & a 4th clinical trial in planning
- Over \$9M in funding at the following research institutions:
 - Stanford University
 - VA Palo Alto Healthcare System
 - University of California San Diego School of Medicine
 - Johns Hopkins University School of Medicine
 - University of Arizona
 - University of Texas, Austin
- An international Scientific Advisory Board focused exclusively on identifying the most promising DS cognition research in the U.S.

Why we need YOU

Federal Funding is not enough.....

2012 NIH Research Dollars Per Diagnosed Person



What YOU can do:

- JOIN US: www.dsrtf.org
www.plus15.org
www.facebook.com/groups/dsrtf
www.facebook.com/plus15
- PARTICIPATE: Bay Area “Signs of Support Event”
*Signing Times Concert and Fundraiser
South San Francisco on 3/21/14*
- DONATE: 2013-2014 Challenge Grant
*All new donors over \$1K and events over \$50K will be
matched 1:1*