Michael M. Harpold
Chief Scientific Officer & Chair, Scientific Advisory Board

representing

LuMind RDS Foundation

United States of America

T21RS Founding Supporter





www.LuMindFoundation.org

LuMind Foundation (formerly Down Syndrome Research and Treatment Foundation - DSRTF) and Research Down Syndrome (RDS) are now one organization

Mission

To stimulate biomedical research that will accelerate development of treatments to significantly improve cognition, including memory, learning and speech, for children and adults with Down syndrome.

Creating New Opportunities for All Individuals with Down Syndrome

- To lead more independent lives
- To participate more successfully in schools & employment
- To prevent additional early cognitive decline with aging & Alzheimer's disease









Michael M. Harpold, PhD
Chief Scientific Officer & Chair, Scientific Advisory Board

The Research Strategy & Grants Program

Creating New Opportunities for <u>All</u> Individuals with Down Syndrome Through Cognition Research

Rapid success & validation of new Ds Research Strategy achieved

- Almost \$12 million in new research grants funding since 2004 High efficiency, unprecedented results - >45 researchers
- Defined multiple mechanisms involved in cognitive impairment & earlier onset Alzheimer's disease associated with Ds
- Identified and pursuing more than 9 new potential therapeutic drug targets
- Targeted grants to advance new therapeutic targets through drug R&D pipeline, including target validation, identification and evaluation of effective drug candidates

The Research Strategy & Grants Program

Creating New Opportunities for <u>All</u> Individuals with Down Syndrome Through Cognition Research

Rapid success and validation of new Ds Research Strategy achieved

 Focused on accelerating toward innovative, safe and effective clinical trials together with the development of effective new therapies and new opportunities for all individuals with Ds

** New clinical trials - Biopharma engagement **

Intellectual Disability in Ds

- Roche RG1662 Trials 2011-
- Balance Therapeutics BTD-001Trial 2012-

Alzheimer's Disease in Ds

- AC Immune ACI-24Trial 2013-
- Transition Therapeutics ELND005Trial 2013-
- Leveraged >\$12 million in additional research funding from NIH, universities & other foundations
- Proactive advisory and strategic dialogue with NIH & Congress to enhance comprehensive new Ds research, maximize synergy, and increase Federal funding
- Partnerships & collaborations in achieving breakthroughs