

Down syndrome—a condition known for centuries but still with no cure or treatment for its associated learning disabilities. Scientists at Stanford have discovered a drug therapy that improves learning and memory in a research model of this genetic disorder, raising the possibility of revealing new intellectual abilities for those with Down syndrome. Imagine a child who could not distinguish shapes, who then learns to read. Imagine a young woman withdrawn to blank stares and social isolation, who then rides a bike and talks again. For the first time, there is hope.

Translating Discoveries in Down Syndrome Unlocking Great Potential



“Our studies have the potential to help children with Down syndrome soon, not at the end of a long research program many years down the road. I am energized by the potential to help children and adults and by the excitement of family members familiar with our work.”

Craig Garner, PhD, Professor of Psychiatry and Behavioral Sciences, Stanford University

Translating Discoveries in Down Syndrome Unlocking Great Potential

Down syndrome is an inherited genetic condition caused by an extra copy of chromosome 21 and leads to learning and memory impairment. It affects 1 in every 800 babies born. Just a few decades ago, many individuals with Down syndrome were institutionalized, left without educational or social opportunities. Improved treatment has produced remarkable increases in life expectancy and overall health for those with Down syndrome—yet there remain no approved therapies for their intellectual disabilities.

A few years ago, neuroscientists at Stanford University began a research program focused on causes of intellectual disability in Down syndrome. They discovered deficits in basic brain functions related to memory. Next came a remarkable finding from Dr. Craig Garner’s team: a simple drug treatment that improves learning and memory in a research model. Physicians, scientists, and families have responded with excitement and hope. A door has finally opened. In the next phase of study, Dr. Garner and other dedicated scientists and doctors at Stanford will conduct a clinical trial to confirm the treatment is safe and effective. The future for those with Down syndrome is brighter today than ever before.



“Dr. Garner is a passionate advocate for Down syndrome kids and their families. His research inspired me to try a promising, but unproven treatment for my son. I hope that a clinical trial will confirm Dr. Garner’s discovery as an effective drug treatment. It has such amazing potential.” Teresa Cody, mother of Neal

NEAL’S STORY

At the age of 8, Neal couldn’t read, write or distinguish shapes. Daily study sessions with his mother seemed to have no effect at all.” The main problem was he really couldn’t learn. It was absolutely amazing how much he could not remember. He did not ask questions. He could not verbalize,” said his mother, Teresa. “He used to pick up the pen and stare at the paper and you could see he had no idea which way to make the pen go to copy something.”

Based on Dr. Craig Garner’s work at Stanford, Neal’s mother devised a daily herbal therapy that contained compounds shown in Garner’s research to target the underlying cause of learning and memory problems. Now, a year later, Neal is reading at a second grade level and doing multiplication by hand.

Teresa and other parents are working with Dr. Garner to fund clinical trials to evaluate and document the safety and effectiveness of new drug-based therapies. A documentary of Neal’s progress, plus outreach to families in person and online, are bringing together the Down syndrome community and others as partners with Stanford scientists and doctors.

THE STANFORD ENVIRONMENT AND INNOVATION

Stanford’s location in the heart of Silicon Valley, the proximity of the School of Medicine to other disciplines on campus, and the integration of two world-class hospitals—Stanford Hospital and Clinics and Lucile Packard Children’s Hospital—all combine to create a unique environment for innovation and discovery. Evolving understanding of what causes learning and memory problems in Down syndrome is leading to drug treatments to benefit those with Down syndrome.

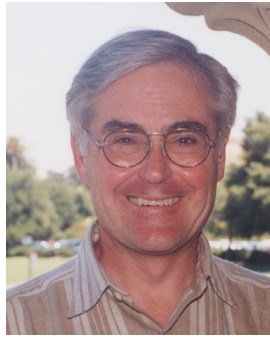
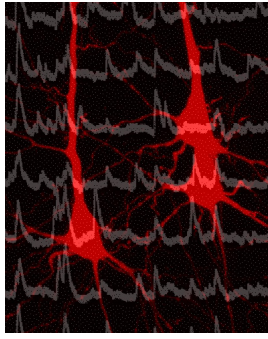
UNLOCKING GREAT POTENTIAL: FROM LAB TO CLINIC

The intellectual disabilities associated with neurodevelopmental disorders such as Down syndrome are complex and require brain studies across many disciplines. At Stanford, biologists, neuroscientists, geneticists, and doctors are working together to understand the nature of the problem and design treatments for individuals with Down syndrome. For example:

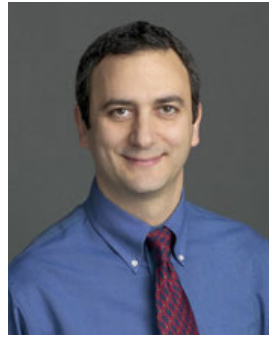
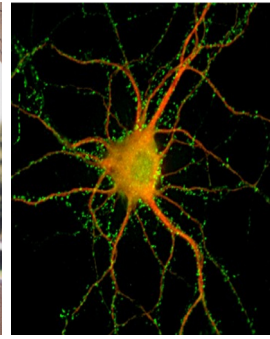
- Craig Garner, PhD, studies the genes and molecules that form and maintain connections between brain cells. These connections or pathways are composed of cells that excite and inhibit other brain cells. Garner’s research in Down syndrome revealed that the inhibitory brain cells signal too strongly, effectively acting like a parking brake that can not be released. This impaired the ability of the brain to retain what was learned. The Garner team discovered a drug therapy for Down syndrome that releases the parking brake and permits normal acceleration required for learning to occur.
- H. Craig Heller, PhD, an expert in sleep, behavior, and memory, conducts studies to test candidate drugs and doses. These experiments will determine treatment strategies for a clinical trial that is currently being planned.
- Antonio Hardan, MD, a clinical psychiatrist, is working with Dr. Heller, Dr. Garner and their colleagues to design clinical trials that will more rapidly translate research findings into therapies available to those with Down syndrome.



Craig C. Garner, PhD



H. Craig Heller, PhD



Antonio Hardan, MD

MESSAGE FROM DR. GARNER

We are inspired by the energy and hope of parents and community members who follow our research. Your input about daily challenges and successes provides clarity and reality to our efforts. We share your hope that through our combined efforts we can change the lives of millions of individuals and families around the world.

For more information about our research, visit the web site <http://garnerlab.stanford.edu/> or e-mail cgarner@stanford.edu



TO MAKE A GIFT

For questions about giving or making a gift online, contact:

Deborah Stinchfield
Associate Director of Development
650.234.0663
dstinchfield@stanford.edu

Checks should be made payable to Stanford University. Include a note indicating your gift is to support the Garner Down Syndrome Fund (DHAYQ). Mail your gift to:

Attn: Deborah Stinchfield
Stanford University
Office of Medical Development
2700 Sand Hill Road
Menlo Park, CA 94025

ADVANCING DISCOVERY AND TREATMENT THROUGH PHILANTHROPY

Individual philanthropy plays a critical role in advancing innovative projects to a stage where further development may be supported by funding sources such as the National Institute of Health. By garnering financial support from parents and the community, Stanford scientists and doctors are able to advance research to discover the underlying causes of intellectual disabilities of individuals with Down syndrome. This knowledge is utilized to design and conduct clinical trials to confirm the safety and effectiveness of promising drug-based therapies.

GIVING OPPORTUNITIES

Outright gifts of cash and securities provide critical resources needed now for basic science research, pre-clinical studies, and clinical trials.

- Basic science research funds provide needed capital for promising avenues of scientific breakthrough that lead to clinical trials and new treatments. These funds pay for laboratory supplies and equipment, salaries of post-doctoral fellows, and communication of research findings to scientists, doctors, and the Down syndrome community. Operating costs for the basic science component of this project are approximately \$500,000 annually.
- Philanthropy represents a critical bridge to complete pre-clinical studies and early phase trial requirements. To produce the treatment drug—and test it to confirm drug quality and safety—is anticipated to cost \$1 million.
- Planning for a phase I/II clinical trial for therapy in children and adults with Down syndrome is underway. The associated costs are estimated at \$30,000 per subject to supply drugs, measure intellectual skills, and conduct tests to confirm treatment safety. The cost of the multi-center trial is estimated at over \$3 million.

Every gift brings us one step closer to unlocking the mysteries of the underlying causes of learning and memory problems, and translating this knowledge into new treatments to benefit children and adults with Down syndrome.