



Outlook

WINTER 2009

New Steps to Accelerate Pace of Research



Dr. Nicole Calakos at our Think Tank

The Bachmann-Strauss portfolio of strategic research investments for this year fall into three categories: basic discovery grants that explore the causes of dystonia and Parkinson’s disease, translational research grants that help to move studies from the laboratory

bench to therapeutics that treat patients, and the establishment of a first-ever Dystonia Center of Excellence.

Twelve new grants, reviewed and selected by our Scientific Advisory Board at the end of 2008 for study in 2009, were approved by our Board of Directors at its November meeting. Characterized by Ted Dawson, MD, PhD, chair of the Bachmann-Strauss Scientific Advisory Board, as “an exciting body of work with tremendous possibility,” these grants provide seed money for innovative ideas to help find better treatments and cures for these devastating movement disorders. (See page 6).

In addition to this body of research, the first phase of our Antidystonia Drug Development Program is now under-

way with Ellen J. Hess, PhD as principal investigator. This new initiative was approved last year by our Board of Directors in a move to jumpstart the discovery of new drug treatments.

“Bachmann-Strauss has taken a bold step in funding the first large-scale drug discovery program,” said Dr. Hess, Professor of Pharmacology and Neurology, Emory University School of Medicine, Atlanta, GA. “At this stage, government funds simply wouldn’t be available for such a venture – it’s too new.”

The goal of the program is to establish a testing procedure to study genetic and non-genetic models of dystonia and to identify drugs that can either move directly into clinical trial or be put forward for product development by a biotechnology or pharmaceutical company.

Continued on page 2

Dystonia Center for Excellence Proposed

The generosity of an anonymous donor to our Foundation is the driving force behind the establishment of the first Dystonia Center of Excellence. Through a single, one-year gift of \$475,000, a new site will be funded that will bring together clinical care and research to catalyze major advances in scientific understanding and translate them into new treatments.

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Moving Forward



HELEN MILLER
EXECUTIVE DIRECTOR



BONNIE STRAUSS
FOUNDER AND PRESIDENT

Enabling advancement from theory to study to practice is one of the most powerful results of our Foundation's work.

Through the years we have come to understand that research is a very deliberate process but when science moves, it moves quickly and it is imperative to seize opportunities that arise.

Following the recommendation of the pre-eminent scientists who participate in our Think Tank, we will now give out grant awards twice a year to accelerate the pace of progress. Their expert advice has also helped to direct our funding which, this year, expands to drug development and moves to launch the first-ever site to house a Dystonia Center of Excellence.

Your ongoing support makes results like these possible. To each of you who contribute to our organization, and to the dedicated clinicians and scientists, we extend our appreciation and our best wishes for a happy and healthy New Year.

New Steps to Accelerate Pace of Research

Continued from page 1

Phase One will identify which models to test and develop the necessary methodology. To speed the pace of discovery, compounds already approved by the Food and Drug Administration (FDA) will be tested in rodent models to see if they alleviate dystonic symptoms. If successful, it is expected that the drugs will move to clinical trials in humans and it is hoped that the study will leverage additional large-scale government funding. Our Foundation will oversee the direction of the program and monitor its benchmarks.

Bachmann-Strauss early funding for the development of the first genetically-altered ("transgenic") mouse model resulted in the groundbreaking discovery of DYT1 dystonia that showed behavioral features similar to patients with early onset dystonia. Since then, over the past decade, we have funded studies on animal models that have shed light on the pathophysiology of DYT1 dystonia, on abnormalities in the brain that may result in dystonia, and on the impact of proteins.

The impetus for this new initiative and many of our strategic research investments is an outgrowth of our annual Think Tank, which brings together top scientists and clinicians in the field of movement disorders from around the globe to strategize on how best to advance the rate of discovery that can lead to new therapies and, one day, cures.



New genetic research is looking at the prevalence of dystonia in Amish families to help people like Dale Martin, shown at our DBS reunion with his wife, Miriam.

Grants Cycle Expanded

Effective this year our Foundation will, for the first time in its history, award grants twice a year, moving from an annual process.

This new direction is an outcome of discussion among the noted scientists who participate in our annual Think Tank and enables us to respond to the advances that are constantly being made.

"We must be more nimble to respond to new discoveries so we can help move them to the next level," said Ted Dawson, MD, PhD. "This biannual cycle will allow us to do just that."

Requests for Proposals (RFPs) will be issued to the scientific community beginning January 2009 so researchers can apply for grants. Information and application forms are available at www.dystonia-parkinson.org. The deadline for the RFPs is March 20.

Dystonia Center of Excellence Proposed

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Traditionally, clinical care and scientific research are conducted in two separate but parallel worlds. The result is that new insights and knowledge are often not translated into new therapies, which further delays the development of new treatments for people with dystonia.

While “centers of excellence” exist for Parkinson’s and Alzheimer’s diseases, as well as for the rarer Huntington’s and Wilson’s diseases, there are currently no designated centers of excellence for dystonia. Dystonia is estimated to affect 500,000 people in North America alone – more people than

muscular dystrophy, Huntington’s disease and Lou Gehrig’s disease combined.

“This tremendous contribution will enable a vital, much-needed resource to help people with dystonia,” said Bonnie Strauss. “Our appreciation and admiration is deep.”

Beginning in the first quarter of this year, the Bachmann-Strauss Foundation will extend a Request for Proposal (RFP) to the medical and scientific communities to begin the process that will lead to the establishment of a new site.

The proposed Dystonia Center of Excellence will bring together specialists from multiple disciplines so people with dystonia can get an improved level of care. This “seed grant” will also provide support for essential but underserved research, and include a training program for clinical and research fellows in order to attract the best candidates in the field and encourage them to continue work in this area. It is anticipated that the recipient of the grant will provide ongoing support for the Center beyond the first year.

Reunion Celebrates Outcomes for DBS Patients

For children and adults alike, the impact of dystonia can be so severe that their bodies twist and spasm, leaving them unable to walk, to sleep, to go to school or work. It has been ten years since the breakthrough surgery Deep Brain Stimulation (DBS) was found to alleviate the crippling effects of severe dystonia for many people with DYT1 dystonia. Bachmann-Strauss funding was instrumental in developing this concept.

This past November we hosted a gathering for people who had undergone DBS. Ranging in age from pre-teen to mid-50s, our guests and their families had the chance to meet one another and share experiences about the positive changes in their lives. This was particularly beneficial for children, who often don’t know others like themselves, or for people who are considering having the surgery.

Our thanks go to Allergan for their generous support of this event.



Clockwise from top: Michele Tagliati, MD, Carole Ann and Joe Peralta with their daughter, Allison, and Jeff Fagan with Bonnie Strauss.

Collaborations

TEAM Bachmann-Strauss Finishes Strong in ING NYC Marathon

Twenty-five runners in the world's most prestigious race raised \$128,000 this past November to support research for dystonia and Parkinson's disease.

As an official Charity Partner in the ING NYC Marathon, our Foundation was guaranteed 25 slots in the 2008 race, which was held on Sunday, November 2. TEAM Bachmann-Strauss was comprised of men and women from across the United States and the Bahamas.

Paul Harkins, who came from Michigan to run the 26-mile marathon, is newly involved in our organization. "I went looking for a charity that I could align myself with and came across Bachmann-Strauss," he said. "It was very easy for me to jump on board."

Harkins' motivation to raise funds was to help people like his father, who has Parkinson's disease. "It's been amazing," he said. "I started out wanting to run the marathon and inspire people to give. Somewhere along the line it turned around and their giving inspired my efforts."

The Harkins family brought in over \$25,000 – the most of anyone on the team; \$15,215 was the result of Paul's fundraising efforts, and the balance was matched by his brother and his father.

"We are so heartened by the team's generosity and enthusiasm," said Bachmann-Strauss Executive Director Helen Miller. "This year we had fewer slots in the marathon but our team came through and topped last year's fundraising record. They did a terrific job, which will fund two grants."

TEAM Bachmann-Strauss was initiated in 2006 by Brian Dugan, who led ten runners and raised over \$34,000. Last year, we were granted 30 slots in the race. Led by Team Captain Josh Sandbulte, the team raised nearly \$120,000.

Brian Dugan, who supported from the sidelines this year, is encouraging alumni to continue their involvement. To join his effort, contact Beth Pfeil – bpfeil@bsdpdf.org.

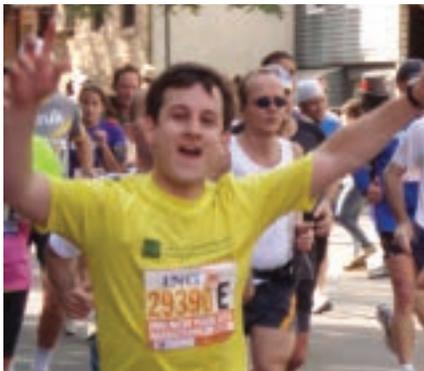


Photo by: Rachelle Robert



Pictured above, Stephen Welker.

At left, TEAM Bachmann-Strauss members at our pre-marathon dinner. Back row, from left: Samuel Sims, Kirk Zapp, Michael Hyland, Daniel Rosenbaum and Paul Harkins. Front row, from left: Meredith Kruse, Alice Stopkoski, Stephen Welker, and Kristen Rosenbaum.

TEAM members not pictured: Callan Blount, Joe Cullen, Jen Dekker, Shauna Harris, Eric Johnson, Michael Jones, Kristy Koenig, Gene Kwon, Robert Lefkowitz, Priscilla Mark, Christopher McLain, Ian Ogilvie, Danford Peterson, John Quigley, Ashley-Brooke Sandall, Jeffrey Solomon, and Ranan Wichler.

Collaborations

Jake's Ride Raises Over \$270,000

When Nancy and Jeff Silverman began to plan a bike ride that would raise money for dystonia research to help their son Jake, they had no idea that it would attract so much community attention and support.

Jake's Ride, held on October 5th, in the Short Hills/Millburn area of New Jersey was nothing short of terrific, raising \$270,096 – more than double their goal – to fund dystonia research through The Bachmann-Strauss Dystonia & Parkinson Foundation. All told, 750 people participated in the event by riding from one to 20 miles or by walking a one mile loop.

Eleven-year old Jake Silverman was diagnosed with childhood onset dystonia when he was in the fifth grade. He had Deep Brain Stimulation surgery last year and returned to school in September.

In addition to raising funds for research, the Silverman's wanted to do whatever they could to raise awareness of dystonia. Their success was evident when a middle school teacher asked her students, "how many of you know what dystonia is?", and every hand shot up.



Photos by: Herb August

Pictured with Jake Silverman are prize winners for fundraising and recruiting team members. From left: Ethan Stupp, Aashish Ahuja, Jake Silverman, Jared Seletsky, and Grant Gardner. Not pictured: Michael Beck and Mackenzie Goepel.



The Show Must Go On

Just days before our theater benefit of the Broadway preview of "Pal Joey", Christian Hoff injured his foot and had to relinquish his lead role.

Over 80 attendees to our theater benefit on December 3rd saw Matthew Risch, Christian's former understudy, in the show along with Stockard Channing and Martha Plimpton. Through the generosity of our guests, the event raised \$65,000 to benefit our organization.

Our best wishes for a speedy recovery go to Christian Hoff, our National Spokesman.

Rockers on Broadway, a one night benefit concert of the 15th anniversary of "The Who's Tommy", generously donated a portion of their proceeds to The Bachmann-Strauss Dystonia & Parkinson Foundation. Bachmann-Strauss National Spokesperson, Christian Hoff, was in the original Broadway cast of "Tommy".

2009 Grant Summaries

Nicole Calakos, MD, PhD *Duke University, Durham, NC*

Very little is understood about the cause and possible genetic contributions of late onset focal dystonias. Dr. Calakos' team will create a mouse model to study the effects of a novel TorsinA mutation at the cellular, neural circuit and behavioral levels, and to provide evidence for a genetic contribution to late-onset focal dystonias.

Mark A. Gluck, PhD* *Rutgers University, Newark, NJ*



Focusing on early-stage, recently diagnosed Parkinson's patients on and off dopamine-agonist therapy, this research will provide insights into the learning and

decision making deficits in Parkinson's disease and how they are altered by dopaminergic therapy. The long-term goal is to find better approaches to patient treatment which improve both motor and cognitive functioning.

Monica Holmberg, PhD *Umea University, Umea, Sweden*

Dr. Holmberg's studies are following a family in Northern Sweden with a form of late-onset primary dystonia. The disease gene is inherited in a dominant manner, meaning that only one copy of the gene is needed to cause the disease. This study aims to identify the exact gene responsible. Her previous studies have identified the chromosomal region where the disease gene is located.

Naomi Lubarr, MD *Beth Israel Medical Center, New York, NY*

The long term clinical outcomes of people above age 50 with DYT1 dystonia and their first degree relatives will be examined to explore the possible risk of parkinsonism or neurodegenerative disorders in DYT1 mutation carriers and to evaluate general neurological status, general med-

ical and mental health, as well as cognitive status and quality of life.

Jonathan Mink, MD, PhD *University of Rochester, Rochester, NY*

A significant proportion of children with Cerebral Palsy (CP) have dystonia. This study, to be conducted among children from four to 18 years old, is to determine whether the medication Tetrabenazine, newly approved by the FDA, can help them by improving upper limb dystonia to develop better hand function.

Martin Niethammer, MD, PhD *The Feinstein Institute of Medical Research, Manhasset, NY*

Genetic forms of primary torsion dystonia (PTD) typically begin in childhood or adolescence and are often generalized while the more common sporadic PTD begins in adulthood and is often focal. Building on previous studies, this research will be conducted to validate a new class of imaging-based biomarkers to assess sporadic forms of the disease and help evaluate new therapies.

Louis J. Ptacek, MD *University of California, San Francisco San Francisco, CA*



Efforts are aimed at understanding a rare episodic movement disorder called paroxysmal nonkinetic dyskinesia (PNKD), which has prominent dystonia and some similarities

with Parkinson's disease. Though rare, understanding molecular mechanisms relevant in a PNKD mouse model may be helpful for understanding PNKD and other dystonias.

Rachel Saunders-Pullman, MD, MPH *Beth Israel Medical Center, New York, NY*

Johann Hagenah, MD *The University of Luebeck, Luebeck, Germany*

Ultrasound of the brain (transcranial

sonography) has recently imaged structures involved in Parkinson's disease and dystonia and identified abnormalities not found on a routine MRI. Using transcranial sonography, this study will compare people with three specific genetic forms of dystonia to individuals without movement disorders to determine whether these genetic forms share features with PD and whether they have unique brain abnormalities.

David G. Standaert, MD, PhD *University of Alabama at Birmingham Birmingham, AL*

Antonio Pisani, MD *The University of Rome (Tor Vergata) Rome, Italy*

Anti-cholinergic drugs block the action of the chemical acetylcholine, and are one of the most commonly used treatments for dystonia. Building on a previous study, this research will study the brain cells

which make acetylcholine, and how they are affected by the DYT1 dystonia gene. Understanding how dystonia affects acetylcholine function may lead to new therapies.



Antonio Pisani, MD

Kathleen J. Sweadner, PhD *Massachusetts General Hospital Boston, MA*



Continuing her previous studies of a mutant mouse with one copy of the gene that causes Rapid Onset Dystonia Parkinsonism (RDP), this research centers

on the effect of RDP mutations on the protein: how they change its structure and its location in the cell. Understanding this enzyme's role in dystonia will help determine potential treatments.

Continued on next page

* This grant was made possible through the generosity of the Dekker Foundation.

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Bonnie Strauss

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Michele Tagliati, MD
*Mount Sinai School of Medicine,
New York, NY*

A randomized, blinded, cross-over study will be carried out to objectively evaluate the key dimensions of speech in Parkinson's disease patients. Coupled with Deep Brain Stimulation (DBS) manipulation, it is hoped that the evidence will provide new insights into the role of the basal ganglia in speech-motor control.

Howard J. Worman, MD
William T. Dauer, MD
Columbia University, New York, NY

Following their previous creation of cells that express a fluorescent version of the mutant torsinA found in patients with DYT1 dystonia, this study will screen thousands of different chemical compounds to identify those that reverse the abnormal subcellular localization of mutant TorsinA. The outcome will help find lead candidates for the development of drugs to treat DYT1 dystonia.

Propelling Dyskinesia Research

In 2009, Bachmann-Strauss will again be the Michael J. Fox Foundation's lead partner in studying dyskinesia—the involuntary, uncontrollable movements that are a frequent side effect of Parkinson's disease treatment and are distinct from the tremors commonly associated with Parkinson's disease.

This new grant will help fund the work of the Michael J. Fox Foundation Dyskinesia Working Group—a consortium of scientists collaborating to identify research projects that aim to tackle roadblocks that slow down the clinical testing of novel dyskinesia therapies.

Annual Symposium Provides Latest Information

An impressive roster of clinicians and scientists outlined the most current knowledge and answered questions from the audience at our November symposium on Parkinson's disease and dystonia.

This educational event, free to patients, families and health care professionals, covered possible overlaps between Parkinson's disease and dystonia, genetics in dystonia, cognitive deficits, causal factors of depression and treatments for Parkinson's disease, and current and emerging surgical therapies.



Effective treatment for Parkinson's disease includes exercise and dance. Tango has been shown to improve mobility and balance.

- Claire Henchcliffe, MD, DPhil

Our thanks go to: Ron Alterman, MD, Mount Sinai Medical Center; Ted Dawson, MD, PhD, Johns Hopkins University School of Medicine; Mark Gluck, PhD, Rutgers University – Newark; Claire Henchcliffe, MD, DPhil, Weill Cornell Medical College; Laurie Ozelius, PhD, Mount Sinai School of Medicine.



THE BACHMANN-STRAUSS
Dystonia & Parkinson Foundation, Inc.

Fred French Building
551 Fifth Avenue, Suite 520
New York, NY 10176

Phone: 212.682.9900
Fax: 212.682.6156
www.dystonia-parkinson.org

SAVE THE DATE

Monday, June 22, 2009

The Hedi Kravis Ruger Tournament

Mark your calendar now for the 17th annual
Dystonia & Parkinson's Pro-Am Golf Invitational
at the Century Country Club in Purchase, NY.

*For reservations or more information, call 212.682.9900
or email bpfeil@bsdpf.org.*



Photos by Ben Aasen except where noted

The Bachmann-Strauss Dystonia & Parkinson Foundation, Inc. was established in 1995 to find better treatments and cures for the movement disorders dystonia and Parkinson's disease, and to provide medical and patient information. An independent, nonprofit, 501(c)3 organization, its funding is made possible through the generosity of individual and corporate contributors.