



Founder and President Bonnie Strauss

Striving for IMPACT

With the centennial of the modern foundation upon us, it's time to think about our impact in grant making, programs, and events. As you read this issue of Outlook, you'll see how your investment in our work continues to have significant meaning - enhancing the search to find better treatments and cures for dystonia and Parkinson's disease.

Strict review protocols and scientific integrity ensure that our funding goes to researchers with promising cutting-edge concepts. Moving from the lab to Capitol Hill, we're advocating for more government funding for a broad spectrum of dystonia programs. And, on the ground, volunteers, runners and cyclists, golfers and shoppers, too, are raising funds to support the Foundation's scientific grants.

Completing the agenda, our annual Think Tank and Symposium demonstrate the energy and intellectual capital driving our long-term vision and work, ensuring that Bachmann-Strauss funds scientific and clinical research that accelerates understanding of movement disorders and offers new approaches to treatment.

Indeed, over the past few months, our philanthropic endeavors - along with the hard work of our Board, volunteers, and staff - showcase the multifaceted efforts of a truly modern foundation. Your generous support sustains everything we do. Together, we are truly making a difference and improving the lives of dystonia and Parkinson's patients, one at a time. Please accept our grateful appreciation!

Bonnie Strauss
Founder and President

10th Annual THINK TANK Focused on Dystonia Triggers



Think Tank co-chairs Dr. Harry Paulson and Dr. David Standaert

In December 2011, the Foundation convened its tenth annual Think Tank on dystonia and Parkinson's disease. Co-chaired by Dr. Henry Paulson and Dr. David Standaert, this gathering brought together an international group of neurologists,

geneticists, and pharmacologists to share their knowledge and clinical expertise. The intensive day focused on relating the circuits- and systems-level mechanisms in the brain to the genes and proteins that trigger dystonia.

Historically, dystonia has always been considered a disorder of the basal ganglia. But recent evidence has pointed towards cerebellar circuits as well. Many researchers now believe that dystonia is caused by a disruption of a motor network that involved both the basal ganglia and cerebellum, rather than an isolated dysfunction of only one motor system, which brought upon this fascinating topic. Ted Dawson, MD, PhD, Chair, The Bachmann-Strauss Foundation Scientific Advisory Board said, "Targeting the cerebellum in future therapies has particular promise for treating patients and making them better."

After intense discussion, the outcome of the Think Tank presented a few essential conclusions. Although there was some disagreement over the speed with which the cerebellum theory is being advanced, it was agreed that the use of a primate model could be instrumental in increasing the understanding of neuronal connections that play important roles in dystonia. Hopefully, this will lead to better molecular targets and pharmacological treatments for dystonia and Parkinson's disease patients.

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Educating and Empowering Patients

Highlights from the 2012 Dystonia & Parkinson's Disease Patient Symposium

With an impressive line-up of neurologists, the Interactive Dystonia & Parkinson's Disease Patient Symposium, held on February 28, 2012, attracted over 200 patients and caregivers, making it the Foundation's most successful to date. Dr. Stanley Fahn, H. Houston, Merritt Professor of Neurology and Director of the Center for Parkinson's Disease and Other Movement Disorders at Columbia University, set the agenda with his opening remarks, focusing on ways to better educate and empower individuals.

The daylong Symposium included panel discussions with noted neurologists, discussing the latest research, treatments

and new discoveries, as well as interactive workshops and discussions with specialists in alternative, complementary therapies. Divided into two sessions, one devoted to dystonia and the other to Parkinson's disease, the Symposium offered participants a meaningful experience and showcased different therapies they might not have explored relating to specific movement disorders.

Topics included Myths and Misconceptions, Botulinum Toxin Therapy, How You Can Get Involved in Helping Research, and Genetics. And, in a large-group setting, Dr. Max Gomez, Emmy-winning medical reporter, moderated an audience question and answer session with neurologists.

Interactive classes were also held on Chair Yoga, Emotional Coping Strategies, Alexander Technique and Music, Movement and Dance.



Patients and caregivers connecting and educating one another.



Medical Reporter Dr. Max Gomez, moderates the panel discussion with Dr. Susan Bressman, Dr. Stanley Fahn and Dr. Steven Frucht.

As one participant said with a smile, "This was a great hands-on experience."

Allergan, In-Step Mobility Products, Inc., Lenox Hill Radiology and Medical Imaging Associates, P.C., Medtronic, Merz and Teva Neuroscience, generously supported the Patient Symposium. In addition to significant funding, the companies provided participants with information about their latest dystonia and Parkinson's disease drugs, treatment protocols and products.

Transforming the Scientific Landscape

Using explicit criteria and a stringent scientific review process, The Bachmann-Strauss Dystonia & Parkinson Foundation encourages research designed to transform the care and treatment of dystonia and Parkinson's disease. For 2012, a one-time special Request For Proposal (RFP) focusing on dystonia research was released by the Foundation. Following intense review, the Foundation's Scientific Advisory Board awarded two interrelated grants. The first grant was awarded to H.A. Jinnah, MD, PhD, Professor, neurology, human genetics and pediatrics, Emory University, and the second to Cristopher Bragg, PhD, Assistant Professor of Neurology, Massachusetts General Hospital. The Board believes these gifted researchers have the potential to develop bold new directions in dystonia treatment.



Dr. H.A. Jinnah

The goal of Dr. Jinnah's project - the Dystonia Coalition iPSC Resource - is to develop a resource for the collection of skin samples for making fibroblast cultures for dystonia, to create stem cells from these fibroblasts to share with dystonia investigators, and to begin to examine the defects in these cells after they are converted into dopamine neurons.

Using newly developed technology to study neurons of different motor pathways, Dr. Jinnah explained that this technology involves taking a small skin sample from patients with dystonia, growing living fibroblasts from the skin, and then converting the fibroblasts into stem cells for making neurons. These stem cells can be used to generate a variety of different types of neurons. Thus, it becomes possible to have an unlimited quantity of different types of neurons for many different types of studies. As

these cells are made from skins samples of dystonia patients, they will contain the genetic defects responsible for the disorder.



Dr. Cristopher Bragg

Dr. Bragg's project - Generating Isogenic Dystonia iPSC Cell lines with Custom TALE Nucleases - will generate iPSCs to different genetic causes of dystonia by turning normal cells into cells with dystonia mutations with TALE nucleases. Essentially they will be able to create iPSCs to any genetic form of dystonia using TALE technology. Dr. Bragg will also collaborate with the Jinnah laboratory in developing and comparing the different dystonia iPSC models. Directly comparing TALE nuclease -generated iPSC lines to ones generated by reprogramming patient fibroblasts can provide a lot of useful information.

The funds provided by The Bachmann-Strauss Foundation will support efforts to derive iPSCs by different methods. The work will take place over the next two years and will proceed in parallel with Dr. Jinnah's lab. Dr. Bragg, explained, "Once our labs have derived iPSC models, it will be highly useful for us to coordinate functional analyses of the different models within the same assays, i.e., Dr. Jinnah's group could potentially differentiate all lines in the same platform and characterize morphologic features, while our group could perform transcriptional profiling on all lines."

In seeking proposals from extraordinary researchers like Drs. Jinnah and Bragg with the potential for innovative approaches in previously unexplored areas, the Scientific Advisory Board looks for projects that will lead to new tools or new directions for future treatments in dystonia and Parkinson's disease.

Seeking Better Dystonia Screening Drugs

Although no cure for dystonia exists, effective screening and treatment for dystonia helps to lessen the symptoms of muscle spasms, pain, and awkward postures. The goal is to improve the quality of life and functioning of patients with the fewest side effects possible.

The Bachmann-Strauss Scientific Advisory Board has announced that the Foundation will continue to fund the Anti-Dystonia Drug Discovery Program, headed by Ellen Hess, PhD, Professor, Department of Pharmacology, Emory University School of Medicine. "My general goal is to understand the pathomechanisms of dystonia by examining the underlying anatomical, physiological and biochemical substrates of the disorder by creating and manipulating mouse models. This strategy allows us to induce or ameliorate motor dysfunction in the context of an intact nervous system revealing potential targets for therapeutics," explained Dr. Hess.

For example, her team is currently using behavioral and cellular pharmacology to



Researcher and scientist Ellen Hess, PhD works to find better drugs for dystonia.

understand the cellular mechanisms that give rise to hyperactivity. Continuing her Bachmann-Strauss funded research studies, the objective of Dr. Hess's research is to identify drugs that can either move directly into clinical trial or be put forward for product development by a biotechnological or pharmaceutical company. The drug screening protocol created in the first

phase of her research has transitioned to the testing of new compounds to alleviate dystonia symptoms in mice.

Looking ahead, the Anti-Dystonia Drug Discovery Program plans to make drug screening more widely available to facilitate preclinical testing of novel anti-dystonia compounds.

Dystonia Advocates Push Congress To Fund Research



Dystonia advocates meet with Congressman Bill Owens to discuss the future of dystonia research.

Gathered on Capitol Hill in May for Dystonia Advocacy Day, over 100 advocates held informal conversations with members of congress and their staffs to promote changes on key issues that can ultimately benefit the dystonia community. Organized by DAN (Dystonia Advocacy Network), energetic, knowledgeable advocates discussed overall funding for the National Institutes of Health (NIH) for dystonia research; support for NIH's dystonia portfolio, including the Dystonia Coalition in the Rare Diseases Clinical Research Network, which recruits patients for clinical trails; inclusion of dystonia as a condition eligible for study in the Department of Defense (DOD) Peer Review Medical Research Program, as trauma can lead to dystonia, which is becoming increasingly prevalent among veterans; and support for patients' access to dystonia therapies.

Advocates wanted to ensure that the legislators fully understand and support funding for research and technology to improve the lives of those with dystonia through better therapies and ultimately a cure. Fabienne Michel said, "It meant so much to me to take part in Dystonia Advocacy Day. It made me hopeful to talk to our members of Congress and to put a face to this disorder. Letting them know that this can happen to anyone at any age and that their support in any capacity is so important. Dystonia Advocacy Day made me feel powerful against something that often renders me powerless."

Jake's Ride

Hits \$1 million!

Celebrating the Foundation's largest community event, an estimated 1,000 local residents rode bikes, walked, or volunteered, during Jake's Ride for Dystonia Research on September 25, 2011, supported by 50 local sponsors. Achieving a new annual high, this event raised more than \$300,000.

From its modest beginning four years ago, when the Short Hills/Millburn, New Jersey, community came out to support 11-year-old Jake Silverman who has dystonia, Jake's Ride has now reached the \$1 million mark, funding 13 grants and one movement disorder fellowship.

The success of Jake's Ride for Dystonia Research in 2011 enabled the Foundation to award three research grants: C. Savio Chan, PhD, Northwestern University; Diane Ruge, MD, PhD, Institute of Neurology, London; and Ana Westeneberger, PhD, University of Luebeck, Germany; as well as a fellowship to Jeff Waugh, MD, Massachusetts General Hospital.

Mark your calendar - **Join Jake's Ride on September 30, 2012** to support dystonia research. The ride begins promptly at 9:00 am at Hartshorn Elementary School, 400 Hartshorn Drive, Short Hills, NJ 07078. Registration opens at 7:30 am. And, check out the details on Jake's Ride web site www.jakesride.org. Hope to see you all there!



NBC reporter Brian Thompson, Founder and President of the Foundation Bonnie Strauss, volunteer Ed Gewirtz and The Jets Flight Crew Cheerleader's participate in the ride's ribbon cutting.



Jake Silverman rides the 5 mile course and proudly crosses the finish line.



Guests supporting the fashion fundraiser.

Internationally Renowned Fashion Designer Hosts Bachmann-Strauss Gathering

With a unique and highly recognizable collection, using vibrant colors and patterns, Matthew Williamson, the UK's leading fashion designer hosted The Bachmann-Strauss Foundation's Young Leadership Council on March 22, 2012, at his New York emporium. Essentially a fundraising event, 20% of the proceeds from the evening's sales were given to the Foundation to fund medical research. Guests were invited to try on the designer's Spring collection. Bright, intricately detailed pieces set the pace for the event; generating \$15,000 in sales in just a few hours. Special thanks to Allison London, who chaired the event, fashionable shoppers, and Matthew Williamson for proving that fashion can make a difference!

A Quintessential Volunteer

America's story is the story of volunteers, everyday citizens who step forward to change lives and strengthen our work. For Pam Sloate, a dystonia patient, volunteering at Bachmann-Strauss is an opportunity for her to contribute to the Foundation's important work. Pam explains:

"I'm well aware of the critical activities undertaken by the Foundation to advance medical research and improve the lives of people like me with dystonia. I'm a current movement disorder patient at The Bachmann-Strauss Dystonia Center of Excellence at Beth Israel Medical Center. Dr. Susan Bressman, my clinician at the Center, is second to no one."



Pam Sloate

Diagnosed roughly 40 years ago, Pam said that dystonia affects her daily functioning. "It's a tremendous relief not to have to worry about the quality of my health care. I know I have access to the latest treatments and patient care is compassionate as well as state of the art," she continued.

With the constant challenges dystonia brings and the general lack of awareness, Pam finds that it's a great feeling to have people in her corner. Also, she has attended the Foundation's patient symposia, a tremendous resource that enables patients to meet others affected by dystonia.

Pam began her volunteer work at the annual Bachmann-Strauss Golf Invitational, where she felt welcome and was impressed by the commitment of Bonnie Strauss, the Foundation's Founder and President, and the entire staff. Pam wanted to show her appreciation and become more involved.

"The staff are caring individuals who go out of their way to let me know my efforts are appreciated, making my volunteer time immensely gratifying. Dystonia is a small community and really I'm just trying to do my part. I can't explain how it feels to give back," Pam said proudly. She took part in Dystonia Advocacy Day on Capitol Hill, along with Foundation staff, and felt it was an honor to represent the concerns of the dystonia community to national legislators.

The Foundation's Founder and President, Bonnie Strauss, has praised Pam for her dedication and excellent contribution. Bonnie said, "Pam's professional experience, her legal practice, nonprofit insights, and program administration background have really enhanced our team's resources. We're fortunate to have her volunteer with us."

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SAVE THE DATE

Sunday, September 30, 2012, Jake's Ride for Dystonia Research

Hartshorn Elementary School, Short Hills NJ. A bike ride fundraiser with routes ranging from one to 20 miles to help children and adults living with dystonia. To donate or register go to www.jakesride.org

Sunday, November 4, 2012, ING New York City Marathon

Come cheer on TEAM Bachmann-Strauss running to benefit our Foundation. Add your support at www.teambachmannstrauss.kintera.org

Your support is gratefully appreciated. To learn more or make a donation visit www.dystonia-parkinson.org or call 212-682-9900.

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.