



Measures of Progress

In this our 21st century, scientists and clinicians have at their disposal unprecedented knowledge and technological resources, yet cures for dystonia and for Parkinson's disease still remain elusive. Unquestionably, important strides have been made in these neurodegenerative diseases over the years, but how do we know if we are really making headway? How is progress evaluated?

According to Ted Dawson, MD, PhD, at The Johns Hopkins University School of Medicine, there are generally three ways in which the scientific community measures success: the ability to attract grants, the ability to leverage that funding into major government grants, such as from the NIH, and getting the results of scientific studies published.

"Patients and their families, understandably, want cures yesterday but that's not the way science works. It's a slow, measured process," explains Dr. Dawson, Director of the Parkinson's Disease and Movement Disorders Center, The Johns Hopkins University School of Medicine, and Chairman of the Bachmann-Strauss Scientific Advisory Board. "Everyone wants to discover a vaccine like the one that cured polio, but *eureka* moments come from unexpected places, and that's why small foundations like Bachmann-Strauss are so important – they're able to take risks and fund the kind of ideas that just may result in finding that magic bullet."

Early stage funding

Bachmann-Strauss follows a venture capitalist approach to grant making, providing seed money for innovative ideas. It's a strategy that has paid off well. Several researchers, who



received early funding from Bachmann-Strauss, have had notable success in attracting federal funding to take their research to the next level.

In a new twist, Guy Caldwell, PhD, has recently leveraged Bachmann-Strauss funding into a licensing agreement with QRxPharma to explore drug development for treating dystonia, Parkinson's disease and other neurological disorders. QRxPharma is a publicly-traded biotech company specializing in preclinical and clinical stage development of pain therapy products.

"If a company is willing to believe in an idea and champion it financially, it is a strong, direct measure of progress toward a therapy," Dr. Caldwell said.

Drs. Guy and Kim Caldwell, a husband and wife team at The University of Alabama, have centered their studies on the roundworm *C. elegans* as a model to accelerate gene and drug discovery. One piece of their research, which was the basis for this new relationship with QRxPharma, relates to a gene shown to suppress the harmful stress associated with the misfolding of proteins within cells. Many neurological diseases involve the toxic effects of misfolded proteins that kill critical nerve cells in the brain of afflicted patients.

Continued on next page

Key Advances

Breakthroughs in Parkinson's disease research date back to 1817 when the illness was first described. While there have certainly been many milestones, the determination in 1957 that Parkinson's is a disease of dopamine loss led to the development of rational therapies aimed at correcting this deficiency. In 1997, the first gene for hereditary Parkinson's disease was identified, which started the current renaissance in Parkinson's disease research.

Major breakthroughs in dystonia, the less prevalent and lesser-known neurological disease, have mainly occurred in the last 10 years since the finding that the DYT1 gene causes early onset torsion dystonia. Since then, there has been important progress in identifying other genes, molecules and therapies.

Parkinson's disease is estimated to affect one million Americans, while dystonia affects approximately 500,000 people in North America. The Bachmann-Strauss Dystonia & Parkinson Foundation is the leading organization actively looking at the interface between these two debilitating diseases.

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.

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Understanding differences

Mark Gluck, PhD, at the Rutgers University Center for Molecular and Behavioral Neuroscience, who has also received Bachmann-Strauss funding, suggests that one direct measure of progress is a deeper understanding of the heterogeneity, or differences, among patients. Why, for example, do two people with the same disease show different patterns of symptoms? Why do some patients respond well to a particular medication but others do not? Perhaps there are many different subtypes of Parkinson's and dystonia, and understanding them will lead to better patient-specific treatments.

"We've made big progress in sub-typing these broader disorders," Gluck says. "Why are we, today, a year closer to cures? I think we get closer when we understand the mechanisms of a disorder and what brain regions are involved. These are all building blocks to a cure."

Clinical and translational research

Danilo Tagle, PhD, program director in Neurogenetics at the National Institute of Neurological Disorders and Stroke (NINDS), part of the National Institutes of Health (NIH), says that these government agencies gauge progress with somewhat different metrics.

"Our ultimate measure is how close are we in terms of diagnostics, therapies and cures. Since our metric is geared toward improvements in the health of the American public, we look for progress made for translational or clinical research," Tagle explains. "They simply wouldn't be possible without the major strides in basic research, and we cannot lose sight of that." Translational research connects basic laboratory research to patient care and is known in medical and scientific circles as "bench to bedside".

There have been major strides in identifying disease genes, in diagnostics and in therapies, Dr. Tagle says, but, like Ted Dawson, he affirms the reality that progress in medical and scientific research can only be measured in incremental advances.

Kudos

The outstanding work conducted by Bachmann-Strauss funded researchers consistently attracts recognition.

Drs. Guy and Kim Caldwell, for example, were recently awarded the first HudsonAlpha Prize for Outstanding Innovation in Life Sciences for their groundbreaking research on diseases of the nervous system, especially in relation to Parkinson's disease. The Caldwells were also named 2008 recipients of the Blackmon-Moody Outstanding Professor Award at The University of Alabama – one of the highest honors bestowed upon faculty at the University. Guy Caldwell, PhD, is an Associate Professor of Biological Sciences; Kim Caldwell, PhD, is an Assistant Professor of Biological Sciences.

Mark Gluck, PhD, is heading a newly-established Center for Collaborative Research on Cognition and Parkinson's Disease at Rutgers University – Newark. The Center will use the experiences of patients and doctors worldwide to explore how memory, learning and other cognitive functions are impacted by Parkinson's disease in order to explore how the disease and medications used to treat it affect the workings of the brain.

Golf Invitational Raises Record \$1.8 Million



Bonnie and Kenneth Davis with Tom Strauss (center)



Taiki and his father, Masa Kasuga



From left: Jacob Spielberg with Ron Alterman, MD, Michele Tagliati, MD and Bonnie Strauss



Mark Standish, co-president of RBC Capital Markets and a member of the Bachmann-Strauss Board of Directors, was honored for setting the pace in leadership giving and for his ongoing commitment to our mission.

"I know that the effect of what I give, both my time and money, achieves amplified results. I hope that every dollar of our research seed money produces results that attract multiples of our investment in later stage research grants."



Event co-chairs Bennett Goodman and Douglas Ostrover



Golf carts at the ready

The rain held off all day for our 16th annual pro am golf tournament in mid June. Then in the evening, just as Broadway star Christian Hoff finished singing to the 280 guests assembled under our tent, the early summer storm hit full force. Within minutes there was water everywhere. Undaunted, our intrepid crowd literally rose to the occasion as we quickly evacuated the tent and moved inside Century Country Club to continue dinner and the evening's program.

Thanks to the support and hard work of our event co-chairs Bennett Goodman and Douglas Ostrover of GSO Capital Partners and the generosity of so many people, our 16th Annual Hedi Kravis Ruger Memorial Tournament generated \$1.8 million, which will go toward finding better treatments and cures for dystonia and Parkinson's disease.

We are grateful to Christian Hoff, to Dr. Max Gomez for serving as our emcee, to Sotheby's Vice Chairman Jamie Niven, who once again brought tremendous results from our auction, and to 13-year-old Jacob Spielberg, who spoke of his struggle with dystonia.

Christian Hoff Records Foundation PSA

Helping to bring awareness about dystonia, Tony Award winner and Bachmann-Strauss National Spokesperson Christian Hoff has recently recorded a public service announcement (PSA) on our behalf. "Few people have heard of it," Hoff says about dystonia in the touching PSA, "but a lot is being done."

The one-minute piece, which may be viewed at www.dystonia-parkinson.org, has been distributed to TV stations nationwide as well as to 110 Web sites, blogs and online social networks.



Christian Hoff entertains at our golf event

Christian Hoff will open on Broadway in December in the lead role of the new Broadway revival of the Rodgers and Hart musical, "Pal Joey". Hoff received a 2006 Tony Award for his portrayal of Tommy DeVito in the Broadway hit play "Jersey Boys" and a 2007 Grammy. He is leaving "Jersey Boys" at the end of September. In addition, he can be seen as Federal Prosecutor Thomas Grady on NBC's "Law and Order" and he is also starring as District Attorney Richard Blackman on ABC's "Ugly Betty".

See back page Save the Date to come to "Pal Joey" at our Theater Benefit on Wednesday, December 3.

When It Rains It Pours ...

... and we mean that in the most positive sense.

The outpouring of support from people across the country has been terrific and, as we embark upon more and more very special events in the coming fall season, that number is growing from every possible quarter.

People want to help – it's as simple as that. Our golf invitational achieved a phenomenal \$1.8 million this year. Children and teenagers are doing what they can to help other kids like Jake Silverman and Jacob Spielberg, who have dystonia. For a third year, a team of marathon runners are racing on our behalf, and people from across the country are adding their support.

We are faced by very clear imperatives. The day has come when government funding for health care, including money for research, is significantly diminished. That means the responsibility to pay for innovative research that will one day find better treatments and cures falls on all of us in the private sector.

Tremendous progress has been made in Parkinson's disease and in dystonia but, as is the case with most diseases, we still look forward to the day when there are cures. There is reason to be hopeful. The work being done by our grantees – and their commitment – is outstanding and fruitful. The support we receive from all of you enables us to maintain and accelerate the momentum.

Thank you all so much.



HELEN MILLER
EXECUTIVE DIRECTOR



BONNIE STRAUSS
FOUNDER AND PRESIDENT

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Shared Goals and Collaborations Strengthen Efforts

Community Joins Creative Fight Against Dystonia



Shortly before 11-year-old Jake Silverman was scheduled to undergo brain surgery last year, his friends said they wanted to get their heads shaved too. This touching idea mushroomed into "Kids Cuts for a Cure" – a fundraiser that generated more than \$5,000 for dystonia research.

"It was such a great kick off to Jake's surgery," said Nancy Silverman, Jake's mother. "A lot of haircutters at the salon, *The House*, volunteered their services, parents' got manicures, and people donated money."

Family friends and residents of the New Jersey community of Millburn/Short Hills responded in force, raising funds through a yoga class, a lemonade stand, jewelry sales and the upcoming Jake's Ride. As word spread, others including town and school officials, teachers and nurses, came forward wanting to do even more. Through the leadership of David Gardner, "Jake's Ride for Dystonia Research" has blossomed. To be held on Sunday, October 5, the bike ride has already generated \$97,000.

"Everyone who heard about Jake wanted to help," said Nancy Silverman. "The good in people really comes out, and they realize that what happened to Jake could happen to anyone."

Jake was diagnosed with childhood onset dystonia when he was in the 5th grade. He missed the following year of school because he was too ill. Now, following Deep Brain Stimulation surgery, he's steadily recovering and hoping to return to classes in September.



Photo By: Herb August

Jake Silverman, left, with his friends at "Kids Cuts for a Cure"

Clips for a Cure Gain Nationwide Support



A bright green carabiner, specially developed to help fund more research into dystonia, has become so popular that it has sold in every state in the nation, generating more than \$30,000.

Thirteen-year-old Jacob Spielberg is the mastermind behind these "Clips for a Cure," which are ideal to attach to luggage or for use as a keychain. Jacob and his friend Carly joined forces in an all-out effort to benefit The Bachmann-Strauss Dystonia & Parkinson Foundation. The popular clips sell for \$5 each and may be purchased at www.dystonia-parkinson.org.

TEAM Bachmann-Strauss Set to Run in Marathon

For a third year, a team of men and women will go all out in the famed ING New York City Marathon to benefit dystonia and Parkinson's disease. As an official charity partner of the marathon, our organization has a guaranteed 25 slots in the 26-mile race, which will be held this year on Sunday, November 2.

With a goal of \$150,000, TEAM Bachmann-Strauss is off to a great head start, already raising \$27,000 by mid-August. Last year, under the leadership of Josh Sandbulte, our team of marathon runners raised nearly \$120,000. Brian Dugan launched this benefit for our organization in 2005 in honor of his mother who has Parkinson's disease.

To add your support, go to www.teambachmannstrauss.kintera.org



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

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goes
here

SAVE THE DATE

Saturday, October 4, 2008

Parkinson's and Dystonia Symposium

Lighthouse International

111 East 59th Street, New York City

Reservations required.

Free symposium gives patients, families and health care providers the latest information about treatments and research.

Sunday, October 5

Jake's Ride

Glenwood School in Millburn, NJ

Bike ride in honor of Jake Silverman. Go to www.jakesride.org

Thursday, October 30

For Young Professionals

Westside Loft, 336 West 37th Street (8th and 9th Ave.)

7 – 10 p.m.

Food and wine tasting – with music and a silent auction

Sunday, November 2

ING New York City Marathon

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

Wednesday, December 3

Theater Benefit

8 p.m. "Pal Joey" starring Christian Hoff, Stockard Channing and Martha Plimpton.

Pre-theater dinner 5:45 p.m.

Monday, June 22, 2009

The Hedi Kravis Ruger Tournament

17th Annual Dystonia & Parkinson's

Pro-Am Golf Invitational

Century Country Club in Purchase, NY

For reservations or more information, call 212.682.9900 or go to www.dystonia-parkinson.org

DID YOU KNOW...

Dystonia is a neurological disorder that causes uncontrollable, painful spasms in one or more parts of the body. An estimated 500,000 Americans suffer with dystonia and one third of all patients are children. Dystonia affects more people than Muscular Dystrophy, Huntington's disease and Lou Gehrig's disease.