

Outlook

The Newsletter of The Bachmann-Strauss Dystonia & Parkinson Foundation, Inc. • Volume 2, Number 1 • Spring 2001

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New Directions for An Old Friend

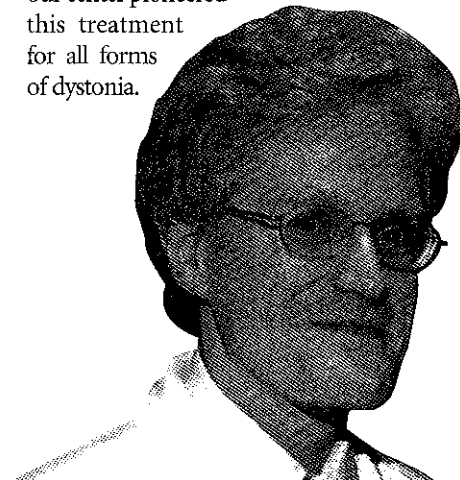
For the past six years, Dr. Mitchell F. Brin has served as Director of the Movement Disorders Program at Mount Sinai Medical Center, as well as a Scientific Advisory Board member for the Bachmann-Strauss Dystonia & Parkinson Foundation. In January 2001, Dr. Brin became Vice President for BOTOX® Neurology at Allergan, Inc., where he is now directing research teams and clinical programs striving to develop new neurotoxin products worldwide.

We are pleased to announce that Dr. Brin will continue to serve on both our Board of Directors and Scientific Advisory Board.

Before he took on his new role, Dr. Brin spoke with *Outlook* about his experiences at Mount Sinai and the opportunities his new position offers to advance dystonia treatment.

Q: What led you to focus on research and treatment of dystonia?

A: After completing my neurology residency, I began a fellowship with Dr. Stanley Fahn, who was just establishing a clinical center for treatment of dystonia. Understanding the genetics of dystonia became a passion for me, nurtured by outstanding experiences working with patients and their families. At the same time, Dr. Fahn had the foresight to explore botulinum toxin therapy for dystonia and, beginning in 1984, our center pioneered this treatment for all forms of dystonia.



Q: What have been the most significant changes in the understanding of dystonia since you began working in the field?

A: Our understanding of dystonia has expanded enormously in the past 20 years and, while we continue to just scratch at the surface, there have been many significant advances. We've seen dystonia established as a medical condition, not a psychiatric disorder. We've established botulinum toxin as an effective therapy for dystonia. Perhaps the most significant change has been progress in understanding the genetics of the disorder. Beginning in the late 1980s, led by Dr. Laurie Ozelius, we established linkage to chromosome 9, and ultimately discovered the gene for childhood-onset idiopathic dystonia and torsinA, the protein that, when mutated, causes the disease.

Q: What are you most proud of, as you consider your work at Mount Sinai?

A: I'm most proud of having built a training program that stimulates young scientists to develop an interest in dystonia. Many of them will go on to devote their professional careers to understanding and treating dystonia. My hope is that dystonia will never go undiagnosed or misdiagnosed, that patients will have prompt access to therapy, and that we'll have more trainees skilled in diagnosing and managing patients. I'm also very proud of having served and treated a large population of patients, whom I'll miss very much.

Q: What are you most looking forward to in your new position?

A: I'm looking forward to the opportunity to provide leadership in therapeutic development for a worldwide team. In all my new responsibilities, I will never lose my fundamental interest in finding the cause of dystonia and developing better therapies. Nothing would bring me greater pleasure than bringing to market a therapeutic that effectively ameliorates the symptoms of dystonia. ☆

Dr. Mitchell F. Brin, former Director of the Movement Disorders Program at Mount Sinai Medical Center, will continue as a member of the BSDPF Scientific Advisory Board, and its Board of Directors.

The Bachmann-Strauss Dystonia & Parkinson Foundation, Inc.

The Bachmann-Strauss Dystonia & Parkinson Foundation, Inc., was established in 1995 to raise funds to support research, provide treatment and promote medical and patient education. These efforts are designed to "make a difference" for those with dystonia and other movement disorders.

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Louis Bachmann (1916-2000)

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Executive Director

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New Board of Directors and Scientific Advisory Board Members

The Bachmann-Strauss Dystonia & Parkinson Foundation is pleased to announce the recent addition of three new Scientific Advisory Board members: Robert E. Burke, M.D., Joseph Jankovic, M.D. and Jose A. Obeso, M.D., and one new member of our Board of Directors, Billy Goldenberg.

Robert E. Burke, M.D., is Attending Neurologist, Columbia Presbyterian Medical Center and Alfred and Minnie Bressler Professor of Neurology and Pathology, Columbia University. He also is a member of both The Parkinson Study Group and Dystonia Medical Research Foundation.

Joseph Jankovic, M.D., is Professor of Neurology and

Director, Parkinson Disease Center and Movement Disorders Clinic, Department of Neurology, Baylor College of Medicine in Houston, Texas.

Jose A. Obeso, M.D., is Consultant Neurologist and Professor of Neurology, Director Unidad de Trastornos del Movimiento y Ganglios Basales, Área de Neurociencias, University Clinic and Medical School, University of Navarra, Pamplona, Spain.

Billy Goldenberg, has won more awards for his compositions for films, television, theater and ballet than we have space to list! He is currently at work on a new one-woman Broadway show for Bea Arthur. ☆

Annual Symposia for Patients and Their Families

Each year, the Bachmann-Strauss Dystonia & Parkinson Foundation sponsors a symposium on Parkinson's disease and another on dystonia. Both are designed to bring patients, families and caregivers up-to-date on the latest breakthroughs in medical research. This year's Parkinson's symposium, held at the Mount Sinai Medical Center on September 22nd, featured a presentation on new medical and surgical therapies for Parkinson's disease by Dr. Matthew B. Stern, Professor of Neurology at the University of Pennsylvania School of Medicine and Director of the Parkinson's disease and Movement Disorders Center of the University of Pennsylvania Hospital. Dr. C. Warren Olanow gave an update on current research, and Dr. Mitchell F. Brin detailed new research into the cause of Parkinson's.

The Dystonia Symposium on December first, also held at Mount Sinai, included presentations by P. Shashidharan, Ph.D. ("Molecular Basis of Childhood Onset Dystonia") Cynthia

Comella, M.D. ("Clinical Trials in a Multi-Center Environment") and Mitchell F. Brin, M.D. ("The Impact of Genetics in Clinical Applications").

Both symposia included hour-long question and answer sessions. Attendees praised the speakers for their informative, yet easily understandable, responses. ☆



Drs. P. Shashidharan, Cynthia Comella and Mitchell F. Brin at this year's Bachmann-Strauss Dystonia Symposium.

A Great Day for Golf — and for Fundraising

The Eighth Annual Dystonia Invitational (the Hedi Kravis Ruger Memorial Tournament) held last June at the Century Country Club in Purchase, the Golf Club of Purchase, and the Blind Brook Club, raised \$1.2 million for dystonia research. The warm, cloudy weather was just about perfect for the 244 golfers (together with 61 pros) who took advantage of it.

Three hundred attendees enjoyed cocktails and dinner later that evening. The late John M. Corrigan (see article, page 3, "An Unexpected Loss") a Trustee of the Bachmann-Strauss Dystonia & Parkinson Foundation, was honored at the dinner for his energetic work on our behalf.

James Niven of Sotheby's conducted a fast-paced, exciting auction. Among the more unusual items sold: an evening with Tom Wolfe, a stay at the Chateau "Magnol" in the Haute Medoc region of Bordeaux, and a personal song to be written by Marvin Hamlisch. ☆

SAVE THE DATE!

The Ninth Annual Dystonia Invitational will be held on **June 18, 2001**, at the same locations as last year. Mary and Jay Goldberg, and Loren Katzovitz are the Corporate Chairs; Bank of America Private Bank is a corporate sponsor. Dr. Mitchell F. Brin will be honored for his many outstanding contributions to the field. **Please hold the date!**

Introducing Our New Executive Director: Margie J. Walden

The Bachmann-Strauss Dystonia and Parkinson Foundation welcomes Margie J. Walden as our new Executive Director.

Prior to joining the Foundation, Ms. Walden worked with the American Institute of Chemical Engineers (AIChE), where she served as Manager of the Member Activity Groups Department. In addition to administering the Institute's awards program, Margie directed all services and publications for 110 local chapters, 165 student chapters, 15 technical divisions and 26 national committees. She also directed a number of large events and conferences for the Institute's leadership, significantly increasing attendance and revenue during her tenure. As liaison to the Institute's Minority Affairs Committee, Margie was honored for her role in implementing programs aimed at increasing diversity in the chemical engineering profession.



Margie J. Walden, Bachmann-Strauss' new Executive Director.

Earlier, Margie served as Membership and Program Consultant to the Girl Scouts of the USA and as Executive Director of the Taiwan International Alliance, where she developed strategies to increase Taiwan's recognition in the international arena. In June 1996, Margie was elected to serve on the Executive Committee of Non-Governmental Organizations, in association with the U.N.'s Department of Public Information. She also serves on the Board of Directors of the Resource Center for the United Nations (RCUN).

"I am very pleased to join the BSDPF since I have experienced dystonia first-hand in my family. Working in support of medical research that focuses on finding a cure for this dreaded disease is a personal mission. I look forward to working with all of the Foundation's supporters to make this dream a reality," adds new Executive Director, Margie Walden. ☆

A Magical, Mystical Evening for Our Young Professionals

The Third Annual Young Professionals' event, billed as "a mystical evening of cocktails, hors d'oeuvres and dancing," was held this past July at the Astra Café, 979 Third Avenue. Benefit Co-Chairs Robin Fried and Felicia Hoffenberg were thrilled that ticket sales, a journal and a raffle brought in over



Young Professionals' "Mystical Evening" Co-Chairs Robin Fried (left) and Felicia Hoffenberg (right).

\$52,000 from the more than 325 young people who attended the spirited event. The BSDPF thanks all of the hard-working Committee members who made the evening such a resounding success — and — we look forward to the next Young Professionals' benefit, this summer. If you would like to be involved in helping with the benefit, please call us at (212) 241-5614 or e-mail us at: Bachmann.Strauss@mssn.edu. ☆



Q&A

Q: How widespread is dystonia?

A: About 300,000 Americans of all races suffer from dystonia, about one-third of them children — but only about 10% have been correctly diagnosed.

Q: Is there a cure for dystonia?

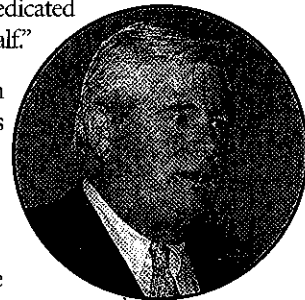
A: Researchers have not yet found a cure, but they have developed treatments that relieve symptoms. One of the most effective of these is Botulinum toxin — a deadly poison — but at carefully regulated dosages, a boon for thousands of dystonia patients.

An Unexpected Loss: John M. Corrigan

John M. Corrigan, a BSDPF Board member for over six years and Senior Vice President and Chief Information Officer of the Estee Lauder Companies, died suddenly this past September at the age of 58. In the words of his colleagues at Estee Lauder, "John always acted with a humanity and care which distinguished him as a professional and endeared him as a human being to all who knew him." Ed Straw, his supervisor, praised his intellect, his loyalty, his candor, his compassion, and his unique sense of humor. "He was selfless in his work ethic and dedication... We are better people because of him."

In a notice in the *New York Times*, Bonnie and Tom Strauss and Nancy Rackoff, the Foundation's Treasurer, wrote, "John showed a unique creativity and compassion as he worked tirelessly on behalf of the Foundation to raise funds for dystonia research. His special interest in our mission arose from the fact that his wife Toni has lived with dystonia for over 10 years. He was honored last June at the Eighth Annual Dystonia Invitational for his dedicated work on the Foundation's behalf."

John was born and raised in Jersey City. He received his B.S. in Political Science from Seton Hall University, and served for two years after graduation as an Officer in the U.S. Army Intelligence Corps. He worked as an executive for Chubb & Son (now the Chubb Corporation) for eight years. After 12 years at Revlon, he left as a Corporate Vice President to join the Eastbourne Consulting Group, where he served as President and CEO for three years. He worked at Estee Lauder for nine years. John is survived by his wife, Toni, and his children, Melissa, John and Mason, to whom all of us extend our heartfelt sympathy. ☆



John M. Corrigan,
Bachmann-Strauss Board
member and great friend.

The Tale of the Allergist's Wife: A Theatrical Evening Benefiting Parkinson's Research

On October 15th, 150 friends enjoyed a wonderful dinner at Jack Rose, followed by a hilarious performance (attended by 210 BSDPF supporters) of *The Tale of the Allergist's Wife*, the hit comedy written by Charles Busch and starring Linda Lavin, Tony Roberts and Michele Lee.



This, our fourth theater benefit, raised \$80,000 for Parkinson's research at Mount Sinai's Movement Disorders Center.

Many thanks to the enthusiastic Benefit Committee for selling so many tickets! ☆

The President's View



We had a very successful 2000, raising more than \$1.6 million for medical research related to dystonia and Parkinson's disease. Researchers funded by the Bachmann-Strauss Dystonia & Parkinson Foundation (BSDPF) have made great strides this year. Dr. P.

Shashidharan at the Mount Sinai Medical Center has been successful in developing a transgenic mouse. This amazing achievement will help researchers learn much more about dystonia and may allow testing of therapies in the near future. The BSDPF is grateful to Dr. Shashidharan for his commitment and efforts in this successful development. None of this would happen without your support.

As you read on page one of the newsletter, Dr. Mitchell F. Brin has left Mount Sinai to direct neurotoxin research at Allergan, Inc. All of us at the BSDPF wish Mitchell great success and satisfaction in his new role, where he will continue, through the development of new experimental therapies, to work on behalf of people with movement disorders. We are confident that, with his discipline and dedication, he will be the driving force behind important pharmaceutical advances at Allergan.

I also want to warmly welcome our new Executive Director, Margie J. Walden. Margie's father has dystonia and she is quite knowledgeable about the disease. She is a welcome member to the BSDPF team. For this coming year, our major goal is to encourage and support advanced research in dystonia and Parkinson's disease. We also have added three new members to our Scientific Advisory Board and we will again be supporting research efforts at Mount Sinai and throughout the United States through the Dystonia Medical Research Foundation in Chicago.

It is a part of our ongoing commitment to support effective management and treatment for movement disorders, as well as to educate the medical community and patients about the symptoms associated with movement disorders. We plan to have a new publication for children available in the spring and a handbook for Parkinson's patients ready in the summer. We will also be holding symposia again in the fall.

Finally, we are in the process of planning our major fundraising event — The 9th Annual Dystonia Golf Invitational — on June 18, 2001. Many thanks to our Corporate Chairs Mary and Jay Goldberg, and Loren Katzovitz for offering their leadership. We also want to thank the Bank of America Private Bank for serving as a corporate sponsor. This year, we will be honoring Dr. Mitchell F. Brin. Join us for a great day of golf, lunch, dinner and a live auction.

Thanks again for all your support. I look forward to seeing each of you at one of our special events this year. ☆

Bonnie Strauss
President and Founder

research notes

Highlights of the research funded by The Bachmann-Strauss Dystonia & Parkinson Foundation at the Mount Sinai Medical Center include:

DYSTONIA

Transgenic Mouse Model of DYT1 Dystonia:

Research conducted by Dr. P. Shashidharan on one of the most severe types of dystonia results from the inheritance of a mutated DYT1 gene. A person inheriting a mutated copy of DYT1 has a 30-40% chance of developing childhood-onset primary dystonia. The mutated gene causes the creation of an abnormal form of torsinA, a protein whose function, normal or abnormal, is still unknown. *Foundation-supported research by Dr. Shashidharan has now succeeded in breeding a small group of mice that carry the mutated form of the human DYT1 gene.* This mouse model should make it possible to learn much more about how the mutant gene causes dystonia, and may allow testing of therapies as well.

To make the mouse model, our researchers first isolated the mutant gene, and then linked it to a group of other DNA sequences. These extra bits of DNA ensure that the gene is only "turned on" to make torsinA in the brain, exactly where normal DYT1 functions. (DYT1 is found in all the cells of the body, but only makes torsinA in the brain's neurons.) Our researchers injected this mixture into mouse embryo cells. After the mice were born, researchers examined a bit of DNA from the tails to ensure that they actually had incorporated the mutant DYT1 gene.

Eventually, our researchers will examine the brains of these mice for evidence that mutant DYT1 was turned on there. They will use the newly developed antibody that tags torsinA (described in the Spring 2000 issue of *Outlook*).

These mice will serve as the founders of a colony of DYT1 mice, allowing a wide range of investigations into the cellular basis of dystonia and the changes caused by mutant torsinA. The mice will also be observed and tested for symptoms of dystonia. Even if the mice do not develop symptomatic dystonia, they are likely to provide an invaluable animal model for examining the pathways taken by mutant torsinA, and for testing therapies aimed at controlling the cellular damage done by the protein. As in so many other disorders, the development of an animal model is a crucial giant step toward understanding dystonia and developing new treatments.

Structure of TorsinA:

The discovery of torsinA by Foundation-funded researchers has opened up entire new lines of research. To learn more about the protein and its role in causing dystonia, scientists are determining the fine details of the molecule's structure using a technique known as X-ray crystallography. Proteins are far too small to see under a microscope, but by bombarding them with X-rays and measuring how the rays bounce off, it is possible to determine the protein's structure. Analysis of the structure will help researchers make predictions about its role in the brain, and may provide clues to how mutant torsinA leads to dystonia.

Physiology of Dystonic Movements:

Researchers are investigating the electrical signals that control movements flowing to and from the central nervous system. Muscles normally send feedback to the brain and spinal cord. The strength of these sensory messages is adjusted within the spinal cord by other messages from the brain. Previous studies have shown that, in dystonia, this complex control system is deficient, at least while the muscle is not being used. Researchers now will determine if the same deficiencies occur during movement. Understanding the physiological basis of dystonic movements will help scientists further understand how dystonia develops.

PARKINSON'S DISEASE

Molecular culprits in Parkinson's Disease (PD):

Neurodegeneration in PD is believed to involve oxidative stress, in which vulnerable brain cells succumb to damage from high-energy molecules called free radicals. Our researchers are studying the pathways by which this damage occurs, and which of the cell's own proteins might be involved in exacerbating the damage done by the free radicals. Scientists will grow brain cells in culture, stress them, and determine which cell proteins respond. Knowledge of these pathways is an important step in designing therapies for PD.

TorsinA in PD Lewy bodies: One of the hallmarks of PD is the formation of protein tangles in the cells of the brain. Foundation-supported researchers recently discovered that, intriguingly, Lewy bodies contain torsinA, the protein associated with childhood-onset dystonia. They suspect that cell death in PD may begin with accumulation of proteins the cell is unable to break down or clear out. This hypothesis will be tested by studying mice, who make extra normal torsinA, to see if they develop Lewy bodies or other signs of PD. ☆



THE BACHMANN-STRAUSS
DYSTONIA & PARKINSON
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**In Support of
Movement Disorders
Research**

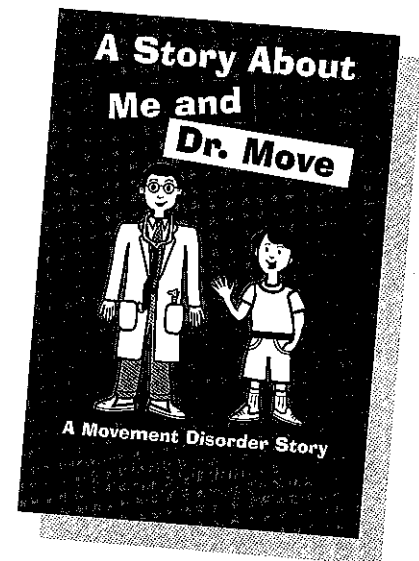
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Especially for Children: *A Story About Me and Dr. Move*

Rebecca (Beka) Serdars, the Intensive Care Unit nurse and dystonia patient we profiled in the last issue of *Outlook*, has written a booklet for children entitled, *A Story About Me and Dr. Move*. This warm and reassuring guide for young people with movement disorders tells the story of Beka's dystonia and the help she received from "Dr. Move." She explains exactly what a movement disorder specialist does, and helps prepare young readers for their own medical treatment.

The Bachmann-Strauss Dystonia & Parkinson Foundation plans to publish and distribute *Dr. Move* this spring. To order a copy, please call us at (212) 241-5614 or e-mail us at: Bachmann.Strauss@mssn.edu.

Thank you, Beka, for this very helpful and comforting guide! ☆



Q: How many people suffer from Parkinson's disease?

A: Approximately 1 million Americans suffer from Parkinson's. The disease is age-related; with the aging of the population, it is estimated that four times as many Americans will suffer from Parkinson's disease by the year 2040.

Q: Is there a cure for Parkinson's disease?

A: Not yet. But researchers have made important advances in our understanding of what happens in the brain of the Parkinson's patient. For example, the Parkinson's disease gene was first identified in 1998. Recent breakthroughs had led to new treatments such as deep brain stimulation and transplantation. And the discovery of a gene mutation in some Parkinson's disease families is an extraordinary advance that will, we hope, lead to other valuable discoveries.