1983-2008

WE CELEBRATE 25 YEARS: LIVING OUR VISION OF A WORLD WITHOUT CMT

THE CMTA Report

JULY/AUGUST
2008

Information on Charcot-Marie-Tooth Disorders for patients, families, and the scientific community * www.charcot-marie-tooth.org

CMTA

Charcot-Marie-Tooth Association

OUR MISSION:

To generate the resources to find a cure, to create awareness, and to improve the quality of life for those affected by Charcot-Marie-Tooth.

OUR VISION:

A world without CMT.

EMAIL CMTA AT:
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Honor a Star, Be a Star Gala Held in State College, PA

Jim Chernega, recipient with his family of an Honor a Star, Be a Star award, shared his silent auction winnings with Amy de Silva's brother William. They both love the Pittsburgh Steelers and waged a bidding war on the autographed photo of Troy Polamalu and the other Pittsburgh memorabilia.

The first annual STAR™ gala was held on June 11, 2008, at the Nittany Lion Inn, State College, PA. Over one hundred guests enjoyed a VIP reception with College Hall of Fame Coach Joe Paterno, who graciously signed footballs, hats, and books for his fans. Following the reception, the attendees entered a room decorated in Penn State blue and white and were greeted by CMTA Executive Director Charles Hagins.

One of the presentations enjoyed by the audience was a film montage produced by Gatesman, Marmion, Drake, a public relations firm. The film focused on Amy de Silva, her singing, her difficulties in dealing with CMT, and on other patients discussing how CMT has affected their lives. Brief comments by Charles Hagins, Dr. Michael Shy, and Director of Program Services Pat Dreibelbis were also featured in the short film.

Chairman of the Board and President Patrick Livney talked about the STAR™ initiative and the CMTA's ambitious goal of raising $10 million over the period of the STAR™ program. He reminded listeners to always ask themselves what they can do to make the world a better place. He said, “We have the opportunity to make a positive difference for millions of children and adults afflicted with CMT and other neuropathies. You can be part of STAR™, which, I believe, will bring about the first treatments and the ultimate cure for CMT in 3 to 5 years. So, please spread the word, create awareness and, when considering your charitable contributions, remember the CMTA and the STAR™ initiative.”

(continued on page 2)

INSIDE:

LET'S DO MORE THAN 'WISH UPON A STAR'

A sage once said that "a goal without a plan is just a wish." The Strategy to Accelerate Research (STAR™), our international, collaborative research effort, has a plan in place to develop, for the first time, treatments for CMT and even a cure within five to ten years.

The goal is extraordinary, and that is to live in a world where adults, children, and entire families will one day not be affected by this debilitating neuropathy. Just imagine. Through the STAR™ initiative, we can actually envision a future without CMT. But realizing this dream is going to take more than wishing. The STAR™ will require more than $10 million of funding to support its initial three projects designed to test thousands of compounds through a high-throughput screening process that could lead to effective therapies.

How can you help? The CMTA is on a mission to see STAR™ through to the achievement of its ultimate goal—a cure for CMT. But it's going to take every dollar we can raise to do it. In my previous letter to you, I told you that this year, during the CMTA's 25th anniversary, we are offering a free year's membership worth $40 between May 1, 2008 and April 30, 2009, whether it's a renewal or a new enrollment. It's our way of saying thank you to those who are or will become a member of the CMTA family. Now, here's how you can make a commitment. Take that $40 and donate it back to the CMTA to help us keep the STAR™ shining brightly.

Some of you may believe that $40 can't possibly help. Believe me, little things can add up to a lot. Your gesture is meaningful. It says that you are with us in this extraordinary effort

—Charles F. Hagins
Executive Director, CMTA

HONOR A STAR

(continued from page 1)

A true highlight of the evening was the singing of "God Bless America" by Amy de Silva. She received a standing ovation for her stirring presentation of the classic made famous by Kate Smith.

Following a dinner of crab cakes and filet mignon, Dr. Michael Shy gave a brief talk on the STAR™ initiative and how its goal of excellence is in keeping with the spirit of excellence always demonstrated by Coach Paterno and his football teams.

The final portion of the evening was the recognition of the first recipients of the Honor a Star, Be a Star awards. The first recipients were the John Chernega family of State College, PA who gave the first large gift to the STAR™ campaign and got the ambitious research project going. The second recipient was the Livney Family Foundation, Chicago, IL, which, in 2006, proposed a $250,000 "Livney Challenge" and matched every dollar the organization was able to raise. The third recipient was the Scarduzio family, who ran golf tournaments for years and provided the first major source of research funding for the CMTA. Finally, the CMTA recognized Coach Joe Paterno for his work in making people in Pennsylvania so much more aware of the name Charcot-Marie-Tooth. His commercials on radio and TV significantly increased the inquiries to the CMTA's office by people in western Pennsylvania.

Following the silent auction, Roland Livney stood up and pledged $100,000 to STAR™ and was followed by Coach Paterno, who pledged $10,000 to help with the campaign. Not including those gifts, the dinner has grossed about $98,000 to date.

Executive Director Charles Hagins and Chairman and President of the Board of the CMTA Patrick Livney presented Coach Joe Paterno with the first Honor a Star, Be a Star award.

STAR™-FUNDED RESEARCH

PROJECT 1:
Develop a line of cells that mimic Schwann cells to use in a high-throughput screen (HTS) to identify treatments for CMT1A

PROJECT 1 PRINCIPAL INVESTIGATOR: PROFESSOR UELI SUTER, INSTITUTE OF CELL BIOLOGY, ETH ZURICH (SWISS FEDERAL INSTITUTE OF TECHNOLOGY), ZURICH, SWITZERLAND

Editor's note: The first phase of the STAR™ Initiative has begun in the laboratory of Professor Ueli Suter. Here he explains briefly his project for this year.

CMT1A is the most common form of CMT and is caused by Schwann cells over-expressing (making too much) peripheral myelin protein 22 (PMP22). Schwann cells are the cells that make the myelin insulation of the nerves in the peripheral nervous system. The extra PMP22 that they make in CMT1A patients disrupts the function of the myelin and causes peripheral neuropathy. The goal of the Strategy to Accelerate Research (STAR™) is to use a strategy called "High-Throughput Screens" (HTS) to identify treatments that will reduce PMP22 levels in Schwann cells and therefore treat CMT1A. To achieve this goal, STAR™ is funding three specific projects, the first of which is presented here.

For Project 1, Professor Ueli Suter will develop the cells to be used in the HTS. Professor Suter is uniquely qualified to perform these experiments. It was he who first "discovered" PMP22 in 1992, and it is because of his investigations that we know that overexpression of PMP22 causes CMT1A. He has great experience in the cell biology of PMP22 and has extensively studied PMP22 expression by culturing Schwann cells in tissue culture wells, a technique that is necessary for high-throughput screening.

Schwann cells are notoriously difficult to grow in tissue culture in part because they change the amount of PMP22 and other proteins they make over time. In addition, it is difficult to measure how much PMP22 protein Schwann cells make without performing laborious and time-consuming protein measurements, which are also a problem for HTS, which requires rapid measurements to screen high numbers of candidate medicines. Therefore, in Project 1, Professor Suter will utilize a more stable line of cells that are known as "MSC80" cells, which were created from Schwann cells by Professor Anne Baron van Evercooren in France. He will utilize genetic engineering to cause the MSC80 cells, to turn on a protein called luciferase whenever they would normally turn on PMP22. Luciferase is the protein used by fireflies to glow; therefore the cells will emit light every time PMP22 is expressed. The more PMP22 the MSC80 cells express, the brighter they will glow. This will allow the robots in the HTS tests described below to simply measure brightness as a measure of PMP22 levels. It is essential to generate these cells for the success of the HTS strategy. This is not a simple challenge. It will take 1 to 2 years to create these MSC80-luciferase cells to the point that they can be effectively used in the HTS.

Once the cells have been generated, they will be used in the HTS facility housed at the NIH Chemical Genomics Center under the direction of Dr. Jim Inglese. Using a robotic computerized system, Dr. Inglese and his colleagues will be able to rapidly test hundreds of thousands of candidate medications to determine if they reduce luciferase levels (brightness) in the MSC80 cells. Those compounds that reduce luciferase will be considered to be candidate medications that can reduce PMP22 levels, and they will be submitted for additional testing in laboratory models of CMT1A. Those medications that are also successful in laboratory models will then be considered for clinical trials in patients with CMT1A. We are very excited about these projects and hope to have multiple candidate medications in clinical trials within the next 3 to 5 years. ✻

Swimming the Chesapeake Bay 2008

On June 8, 2008, CMTA Board of Director Steve O'Donnell plunged into the choppy waters of the Chesapeake Bay to raise money to advance research for CMT. It has been Steve's mission for these seven years to bring awareness of CMT to people in the Maryland area and to generate funds to find therapies and a possible cure for CMT.

Steve was joined again this year by seven other swimmers representing TeamJulia, who were also working to raise funds for CMT research. TeamJulia swims in honor of eight-year old Julia Beron and is spearheaded by her mother Rachel Beron. Last year, TeamJulia raised approximately $65,000, and this year their contributions were up by more than 20%. A total of 325 organizations and individuals contributed to the cause.

The night before the swim, participants and friends enjoyed a dinner in the Annapolis harbor and listened to a CMT presentation by CMTA Executive Director Charles Hagins.

Julia Beron (second from right) and her girlfriends offered plenty of cheering and emotional support to the swimmers who braved the choppy Chesapeake Bay.

Early the next morning, everyone caravanned to the Chesapeake for the swim. The group was in one of the early groupings, which was fortunate because it was already 90+ degrees at 8:30 AM. The conditions this year were tougher than last year because of the extreme heat and very choppy waves. In spite of the conditions, everyone completed the course.

Julia's father Herb wrote of his experience, "This past year has been an extremely emotional period of time for our family. We have opened ourselves up to a lot of people and we feel very fortunate to have so many friends who have supported us for the past two years. We only hope to increase our participation and cooperation with the CMTA, and we are already looking at expanding our team to include possible alternative locations for TeamJulia09!"

In a thank you letter to her supporters, Cathy Quartner Bailey, Rachel's sister, writes: "The swim was a once-in-a-lifetime experience. It was harder than I anticipated, and, while I knew I would somehow reach the finish line, I wasn't quite sure how. At one point, I think I even invented a new stroke—some combination of the crawl, breast stroke, and doggy paddle—as I tried to swim straight ahead, move forward, and breathe. To my surprise, even with adequate training, it took me at least a half mile just to get my breathing down, swim a straight line, and relax enough to enjoy. It was exactly at this point that I did a silent prayer, visualizing Julia's beautiful face and saying each one of your names to myself, and thanking you for your generous support of TeamJulia."

Between Steve O'Donnell and TeamJulia, this year's swim has so far generated $110,415 for the research fund. ✻

TeamJulia poses after a successful swim of the Chesapeake Bay. Rachel Beron (front, middle), was joined by seven friends who helped to raise money for the research fund of the CMTA.

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Patient/Family Conference Presented in Johnstown, PA

BY PAT DREIBELBIS

On May 29, 2008, approximately 85 people attended the CMT Conference at the Holiday Inn Downtown, Johnstown, PA. A highlight of the conference was the presentation by Dr. Michael Shy, who flew in from Detroit to discuss many aspects of CMT disorder, the research being undertaken, and the genetic inheritance patterns in CMT. He mentioned that possible new research might include RNA-induced interference, gene transfer strategies, ascorbic acid trials, and the use of progesterone antagonists. All of these strategies are happening outside of the very exciting STAR™ initiative, which he explained in detail. The sheer number of compounds that can be tested by high-throughput screening is amazing, numbering 350,000 in two weeks.

Dr. Shy also explained that six centers of excellence are being established around the country to provide uniform evaluations to CMT patients, provide a network for the distribution of information on the clinical symptoms of CMT, and be sites where clinical trials will ultimately take place on drugs discovered through the STAR™ three-phase program.

The centers are the University of Pennsylvania/Children's Hospital of Pennsylvania, the University of Washington, Johns Hopkins, University of Texas Southwestern, University of Rochester, NY, and Wayne State University, Detroit.

Another presentation by Jan Goodard, John P. Murtha Neuroscience and Pain Institute, centered around the study on Co-Enzyme Q-10 that is currently underway at the facility. The theory is that CO Q-10 will improve nerve communication and could possibly relieve weakness, fatigue, and pain, all issues in quality of life. The study takes place in two parts. In part one, 50% of the patients will be on a placebo and 50% will be on the CO Q-10. In part two, everyone will be given the CO Q-10. Participants cannot be on statin drugs or Coumadin, be pregnant, be under age 18, and they must be able to come to Johnstown every six weeks for evaluation. For more information, see the blurb on our website at www.cmtausa.org.

The final presentation before the panel discussion was a

Kerri Golden from the Occupational Therapy Department of Saint Francis University discussed the importance for CMT patients to take care of their hands.

The audience at the Johnstown, Pennsylvania, CMT patient/family conference listened to a presentation by Dr. William DeMayo on physical medicine and rehabilitation.

talk on occupational therapy by Kerri Golden from the Department of Occupational Therapy, Saint Francis University.

She told the audience that the most important question an OT can ask is "What are you able to do or what can't you do with your hands that you need or want to do?" Then, the OT will evaluate your range of motion, your strength and your coordination. After the testing, the OT will suggest adaptive equipment, joint protection and splinting, or possible exercises. It is always important to balance exercise with rest and to use adaptive equipment to supplement your existing abilities rather than to substitute for those skills.

The day ended with a panel of the presenters answering questions from the audience, followed by a hot buffet lunch. The conference was funded in part by a grant from the Pennsylvania State Department of Health. ✻

SHRINERS HOSPITALS FOR CHILDREN: ONE OF A KIND PEDIATRIC CARE

Nineteen of the 22 Shriners Hospitals specialize in caring for children with orthopaedic conditions, including clubfoot, limb deficiencies, scoliosis, and the effects of neuromuscular conditions, such as cerebral palsy, CMT, and spina bifida.

Children up to age 18 are eligible for care at Shriners Hospitals, if, in the opinion of their physicians, they can benefit from the specialized services available. Acceptance is based on a child's medical needs. A family's income and insurance status are not criteria for a child's acceptance as a patient.

Shriners Hospitals practice a "family-centered" approach to medical care and involve the patient's family in all aspects of treatment and recovery. The three-part purpose of all Shriners Hospitals is to provide specialty pediatric care, conduct medical research, and educate medical professionals.

You can contact Shriners Hospitals at 1-800-237-5055, or in Canada, 1-800-361-7256.

Since it was established in 1922, Shriners Hospitals for Children has cared for more than 865,000 children. This year, the budget for the hospitals is $826 million. As has been true for 85 years, all treatment and services at Shriners Hospitals are provided at no charge to patients or their families. ✻

John Brown—the Agitator

BY ALAN PAPPALARDO

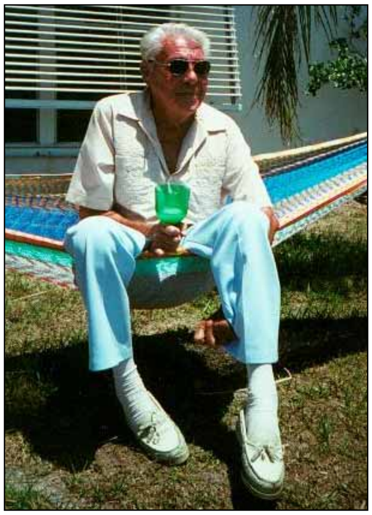
My first encounter with John Brown was when I received a hefty package in the mail. Not knowing what was inside, I opened it and found hundreds of meticulously preserved newspaper clippings, letters, and other personal artifacts. The amount was staggering! What I found out later through talking to John was that he had only sent a small fraction of his entire collection.

John Brown is modest. On many levels John is no different from any CMT patient: he was a clumsy child whose disease prevented him from playing sports and doing a handful of other tasks. While growing up on the Eastern seaboard, he developed a great love of the environment and animals. Through an exhaustive process of diagnosis, misdiagnosis, and some plainly ludicrous medical tests, John was diagnosed with CMT in 1955.

John Brown is an agitator. John used that word, quite accurately, to describe himself a number of times. Mr. Brown has devoted his life to helping those in need, from rallying the troops during a great snowstorm via CB radio to helping preserve our nation's aquatic resources through political action.

"My life-long concerns have been helping others by my involvement in community affairs. This outside activity helps relieve some of my pain from CMT."

Over the decades, John has made countless friends and has even ruffled a few feathers. Once, John was even imprisoned over a dispute concerning a homemade bench next to a small pond. Neighbors had made John and his wife a bench to sit on near their pond because his CMT kept him from standing for long periods of time. Because it was a "code violation," John was told to take the bench down. When he refused, it was off to jail for him!



Despite his CMT, John Brown has devoted his life to helping others in need.

For the most part, John uses endless communication, community coordination, and good old fashioned gumption to make people do the things they should be doing anyway. One thing I could never get John to do in all our conversations was to take credit for any of his countless achievements.

I asked John which of his accomplishments made him feel most proud. After some thought, John referenced something I had learned about in school. On the coast of the United States lies a 200-mile exclusive economic zone. For years foreign fishing fleets came and culled these waters, often without regard for the environment. John lobbied Washington, enlisted help from congressional heavyweights, and helped to pass what is law today, protecting those waterways.

Now, John says,"Every day presents a new challenge for me at 81 years of age and living alone." Still, while lakeside in Florida, John continues his good fight. John's modesty and determination are inspirations to anyone, not just those with CMT. While talking to him, I could not help but feel in awe as I was told one enchanting story after another. There was nothing I read or heard that has contradicted the fact that John W. Brown is extraordinary. While he may fight you on that, the evidence does not lie.

As many others have stated in the past, I thank you John Brown for your years of tireless work and being there when no one else was. A friend once wrote of John: "Don't Ever Give Up!!!—This is the motto of the John Brown I have come to know!" ✻

GIFTS WERE MADE TO THE CMTA

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Mr. & Mrs. B. Bullock
Ms. Janet L. Derr
Mr. & Mrs. Russell A. Falquet
Ms. Jane W. Flusche
Mr. & Mrs. Thomas E. Gebhardt
Ms. Sarah Girard
Granville Town & Country Club
Mr. & Mrs. Fred M. Groff
Mr. John D. Kessler
Mr. & Mrs. David Klauder
The Klauder Family
Mr. & Mrs. Magne
Mr. & Mrs. Ted Montague
Mr. Richard F. Neff, Jr.
Dr. & Mrs. Robert H. Owen
Janet & Nicole Padway & Family
Ms. Janet Shea
Mr. & Mrs. William L. Ullman
Ms. Barbara S. Vockel
Mr. Ryan Walseth
Mr. & Mrs. John B. Whitaker
Ms. Sara Jean R. Wilhelm

Joe Pinczuk
Mr. & Mrs. Frank Weiss

Larry Roman
Mr. & Mrs. Sam Bobrick
Mr. & Mrs. Allan Entous
Mr. Samuel Goldwyn, Jr.
Mr. & Mrs. Harry Jin
Dr. & Mrs. Dennis W. Saffro
Mr. & Mrs. Harvey A. Schneider
Mr. & Mrs. Hal Sender
Mr. Roy E. Tuckman

Jennie Torre
Mr. & Mrs. John Fitzgerald and Family

IN HONOR OF

Mr. & Mrs. Lawrence Battersby
Mr. & Mrs. Gerard McLoughlin

Mr. & Mrs. Joseph Brian
Mr. & Mrs. Edmund F. Capozzi

Jerry Donovan
Ms. Elaine K. Donovan

Floyd Fike
Mr. Richard Strand

Mr. & Mrs. Emil Klimah
Mr. & Mrs. Richard Betten
Ms. Heidi D. Conradt-Fish
Mr. & Mrs. Arthur E. Hartwig
Mr. James Hartwig
Mr. & Mrs. David L. Hartwig
Mr. & Mrs. Ernest Holtzman
Mr. Paul E. Klimah
Ms. Mary Ann Schneider
Ms. Mollie K. Siska
Ms. Linda J. Siska
Ms. Mary G. Slawinski
Mr. & Mrs. Robert Superek
Mr. Richard Thurau
Mr. & Mrs. John Thurau
Mr. & Mrs. Steve Voss
Mr. & Mrs. Stan Wilcinski

Jennifer Taub & Michael Kuch
Mr. & Mrs. Harvey Goodman

Donna Landucci-Ruiz
Mrs. Jill Landucci

Mi E. Kyung Lee
Mr. Melvin D. Cisneros

Patrick Livney
Mr. Wm. "Vasi" Vangelos

Joe Metzger
Dr. & Mrs. Douglas Boldon
Mr. & Mrs. B. Bullock
Ms. Janet L. Derr
Mr. & Mrs. Russell A. Falquet
Ms. Jane W. Flusche
Mr. John D. Kessler
Mr. & Mrs. Ted Montague
Ms. Janet Shea

Alex Ramsey
Mr. Earl Ramsey
Dr. Michael Shy
Mr. Wm. "Vasi" Vangelos

Belle Sohnen
Ms. Ray Weiler

Mr. & Mrs. Mathias Valentine
Mrs. Roseanne Christman
Ms. Anne Karacic
Dr. Joseph J. Karacic

While celebrating their 60th wedding anniversary, Chicago couple Emil and Arlene Klimah choose to forgo presents and, instead, collect donations for the CMTA. Throughout the night, almost $1,000 was collected. The CMTA congratulates them on such a successful union and wishes them many more happy years!

CMT IN THE NEWS

Newsmakers Spread Awareness of CMT

AN ARTICLE IN TRICITIES.COM by retired doctor Nat Ed Hyder, Jr. of Johnson City, TN, remarked on candidate David Davis, U.S. House of Representatives who is running for re-election. Davis is a strong supporter of veterans' affairs and is currently a member of the Homeland Security Committee in Congress. Of note is the fact that Davis inherited Charcot-Marie-Tooth disorder and required corrective surgery at Shriners Hospital many years ago. Even with his crippling condition, Hyder writes that Davis worked his way through college on his own and began his career in the respiratory therapy field.

THE CIRCLEVILLE HERALD printed an article called, "Local Men Offer Aid." The Circle of Caring, a volunteer organization associated with Berger Health System, works to help people remain in their homes and stay independent. One of their biggest programs is to build ramps for people who need them and have no means for the construction of the ramp. The group recently built a ramp for Circleville, Ohio, resident, John Weaver, who suffers from the genetic nerve disorder, Charcot-Marie-Tooth, which has caused him to lose nearly all feeling in his arms and legs. Weaver was recently presented with a power wheelchair, but his home didn't have a handicap ramp. "I was down to my last option until I got this help," Weaver said. Now that he has a power chair and the ramp, Weaver hopes to take his five-year-old daughter to the Columbus Zoo, something he's been waiting to do since she was born.

THE CAPITAL, a newspaper in Annapolis, MD, published an article entitled, "Annapolis doctors help Romanian teen walk again." A child from Romania named Andrei Tipa began to struggle to do things normal kids do when he was about 10. He had trouble running; his left foot began to weaken, and the muscles in his legs atrophied. Bit by bit, Charcot-Marie-Tooth was wresting his body's ability to function away from him. Through the Helping Hands Foundation, two Annapolis physicians recently performed surgery on Andrei's foot and ankle to help him walk better. "Romania doesn't have the technology to do the surgery my son needed," said his mother Clara, with the help of a volunteer translator. The Evangelical Presbyterian Church in Romania alerted Dr. Tom Harries when he was on a charity trip doing surgeries in Romania. Someone asked if we could help and he was flown over to examine him in the US and develop a plan to help him," said Dr. Edward Holt, a foot and ankle specialist, who, with his partner, Dr. Harries, performed the surgery at Anne Arundel Medical Center.

THE EXAMINER from Baltimore, MD featured an article about CMTA Board Member Steve O'Donnell. They remarked on the fact that he began to lose sensation in his hands and legs right after graduating from college. Despite that, Steve stated that he can still grip a baseball bat and hit the ball pretty well, even though running is tough for him. O'Donnell takes time out of his busy schedule to help raise money for research by swimming the Chesapeake Bay. O'Donnell said that they have found the genes that cause the disorder and now "we just have to find the money to get a way to cure it."

STJOENEWS.COM REPORTED that 6-year-old Masan Payne will take his first trip to Disney World in July thanks to the Dream Factory. For Masan, who has Charcot-Marie-Tooth disorder, it will be the trip of a lifetime, a rare chance to just be a kid. For his mom, April, it's an opportunity for her family to get away from the pressures of living with Masan's disorder. The all-expense paid trip will last one week and enable the family to enjoy a "dream come-true."

THE NATIONAL INSTITUTES OF HEALTH (NIH) has awarded a two-year grant of $220,076 to Williams College Assistant Professor of Biology, Lara D. Hutson to support her work on Charcot-Marie-Tooth disease. iBerkshires.com reports that

Lara's research will use zebrafish as a model system to investigate disease mutations in two small heat-shock proteins, HPS27 and HSPB8, which can cause CMT or the closely related disease distal hereditary motor neuropathy. The results of these studies will help to better understand the events at the cellular level that lead to axonal degeneration in CMT.

RHODE ISLAND CATHOLIC reported on a fundraiser to benefit the search for a cure for CMT, on Friday, July 25, 2008. A neighborhood committee of friends has formed and is planning the fundraiser at the West Valley Inn, in West Warwick, RI. Grace Caldarone, 7, and her mother Marybeth, both of whom have CMT, are the reason the friends have gotten together to raise money for CMT research. The event is a dinner/dance from 6-10 PM and all proceeds will benefit the CMTA. Representatives from the CMTA, as well as neurologist Dr. Louis Weimer, Columbia University, will make brief presentations at the event.

THE HERALD NEWS, Joliet, IL, featured an article on Silver Cross Field, a small town's "field of dreams." Members of the Miracle League of Joliet find that baseball provides two things, a way to bond and a needed reprieve. That treasured bond between father and son formed on ballfields across America is almost indescribable. The Miracle League ensures that every child, regardless of ability or disability, can know the feeling.

Alvin Sizemore is thankful to the league. His son, Lawson, 5, has Charcot-Marie-Tooth disorder, which hinders his ability to use the muscles in his hands and feet the way other kids can. But the disorder can't touch Lawson's passion for baseball. All he wants to do is play baseball, and the Miracle League lets him do that. The games are played on a specialized rubber field so that the surface is soft enough to protect its participants from unwanted bumps and bruises.

The games last only two innings, but every player gets a hit and a chance to take the field. Score is not kept, and it is impossible to find any losers on Saturday mornings, almost as hard as it is to find disabled children. Lawson's mother, Marleen, said, "When you walk in the gates of the Miracle Field, you don't see a disability on any child, you just see kids playing baseball. It takes some of the pressure off the family, to see a child doing something he loves and having fun doing it." ✻

CMT1A CLINICAL TRIAL UPDATE

As readers of *The CMTA Report* are well aware, the CMTA and MDA are co-sponsoring the first large-scale multi-center trial for CMT1A in North America. Based on positive results in the CMT1A mouse model, patients are being treated with high-dose ascorbic acid (vitamin C). The first patients were evaluated in April 2007. We are pleased that approximately two thirds of the total number of patients have already been enrolled in the study. We are hoping to complete enrollment within the next few months. We encourage families with CMT1A to consider being part of this study. It is only by completing clinical trials that we will know whether compounds like ascorbic acid really have an effect on slowing disease progression in CMT1A. It is also only by completing clinical trials that we can develop the expertise to best treat and investigate additional patients and medications for CMT. We thank all of you for your help. We can't do this without you.

—The CMT1A Clinical Trial Team (Wayne State University, the University of Rochester, and Johns Hopkins University)

For more information about the study and the study centers, contact:

Lisa Rowe
Wayne State University
Detroit, MI
313-577-1689
Email: lrowe@med.wayne.edu

Patty Smith
University of Rochester
Rochester, NY
585-275-0581
Email:
patty_smith@urmc.rochester.edu

Lora Clawson
Johns Hopkins University
Baltimore, MD
410-614-4346
Email: lclawson@jhmi.edu

SUPPORT GROUP NEWS

> Our network of support groups is continuing to grow, but we still need volunteers for states without a group. Please consider starting a group in your area.

California – San Francisco

The June support group meeting was cancelled due to poor air quality. The wildfires in the region were nerve wracking. The next meeting will be a picnic on September 6th from 1:30 to 5:00 PM. The upcoming family patient conference in San Francisco on November 8th will be discussed. Dr. Michael Shy from the Wayne State CMT Clinic and the genetic counselors from the clinic, as well as an orthotist from Detroit, will join other presenters in making this a very special event.

Colorado – Denver Area

The meeting on June 28, 2008 featured a presentation by Linda Port from Canine Partners of the Rockies. CaPR trains dogs to provide assistance to people who have limited mobility, from individuals who use wheelchairs to those requiring a steady, four-legged partner to balance them as they walk. Leader Diane Covington also passed out the new wallet-sized card with the neurotoxic drug list provided by the CMTA. The next meeting will be August 30th and will feature Dr. Ronald Kramer, neurologist and recognized authority on sleep apnea.

Georgia – Atlanta Area

On June 21st, a group of 35 attendees heard Dr. Michael Shy from the Wayne State CMT Clinic discuss the types of CMT, its characteristics, and the work that the CMTA is doing to find treatments and ultimately a cure for CMT. The next meeting will be on August 23rd at 2:00 PM. The presenter will be Sean McKale, a leading orthotist from Wayne State who has tremendous experience in bracing CMT patients. October's meeting will be on the 25th due to a scheduling conflict at the school.

Illinois – Chicago

On June 21, 2008 the Chicagoland CMTA support group met in Hinsdale, Il. The guest speaker was Gene Bernardoni, an orthotics professional from the area firm Ballert Orthopedic. Gene presented on the use of orthotics with CMT patients and brought along a variety of leg-bracing examples. If you were unable to attend and would like Gene's contact information, please contact Alan Pappalardo. The next support group will be held on Saturday, September 27, 2008 at 2:00 PM. Location is to be announced. For any questions, please contact Alan Pappalardo (alan@charcot-marie-tooth.org).

Oregon – Portland

The first Support Group Meeting in the Portland area was held on June 19th, with an attendance of 10 people. It was a meet-and-greet meeting, and we also discussed the importance of proper nutrition and exercise. At the end of the meeting, we had a drawing for a couple of gift bags containing various items that people with CMT can use. We are in the process of seeking a different meeting location and will send out flyers for the next meeting with that information on them.

Pennsylvania – Philadelphia Area

The June meeting, originally scheduled for the 21st, was cancelled because of a lack of response from members. The next meeting, on August 16, 2008, will feature a presentation by National Support Group Liaison Elizabeth Ouellette. Elizabeth's son, Yohan, has CMT, and she has written many articles on issues surrounding parenting and CMT as well as innovative ways to manage pain. In addition to her presentation, we will have the postponed "pot luck" that day so that we can enjoy some fellowship and good food. Specific details regarding the meeting will be emailed and mailed out two weeks before the date. If you have questions, please call Pat at 1-800-696-2682, ext. 103 or email her at pat@charcot-marie-tooth.org. ✻

CMT Support Groups

Support Group Liaison: Elizabeth Ouellette, 1-800-606-2682, ext 107

California—Northern Coast Counties (Marin, Mendocino, Solano, Sonoma)
Place: Sutter Medical Center of
Santa Rosa
Meeting: Quarterly, Saturday, 1 PM
Contact: Louise Givens,
707-539-2163
Email: ladyblue123@att.net

California—San Francisco Bay Area/Santa Clara County
Place: San Mateo Library
Meeting: Quarterly
Contact: Elizabeth Ouellette,
650-248-3409 (C)
650-559-0123 (H)
Email: elizabetho@pacbell.net

Colorado—Westminster
Place: Capabilities, Westminster, CO
Meeting: 10 AM – noon, Last Saturday of
every other month
Contact: Diane Covington
303-635-0229
Email: dmcovington@msn.com

Florida—Tampa Bay Area
Place: St. Anthony's Hospital,
St. Petersburg, FL
Meeting: Second Saturday of Feb,
May, Nov, with other special
meetings throughout the year
Contact: Lori Rath, 727-784-7455
Vicki Pollyea
Email: rathhouse1@verizon.net
v_pollyea@mindspring.com

Georgia—Atlanta Area
Place: Cliff Valley School Library
2426 Clairmont Rd, NE
Meeting: Third Saturday of every
other month
Contact: Sue Ruediger, 678-595-2817
Email: susruediger@comcast.net

Illinois—Chicago Area
Place: Peace Lutheran Church,
Lombard, IL
Meeting: Quarterly
Contact: Alan Pappalardo,
800-606-2682, ext. 106
Email: alan@charcot-marie-tooth.org

Kentucky/Southern Indiana/ Southern Ohio
Place: Lexington Public Library,
Beaumont Branch
Meeting: Quarterly
Contact: Martha Hall, 502-695-3338
Email: marteye@mis.net

Michigan—Detroit Area
Place: University Health Center,
Wayne State U., School of Medicine
Meeting: Call for schedule
Contact: Lainie Phillips, 248-890-1529
Email: familiaphillips@sbcglobal.net

Minnesota—Benson
Place: St. Mark's Lutheran Church
Meeting: Occasionally
Contact: Rosemary Mills,
320-567-2156
Email: rrmills@fedtel.net

Minnesota—Twin Cities
Place: Call for location
Meeting: Quarterly
Contact: Bill Miller, 763-560-6654
Email: wmiller758@msn.com

Mississippi/Louisiana
Place: Baptist Healthplex,
102 Clinton Parkway, Clinton, MS
Meeting: Quarterly
Contact: Flora Jones,
601-825-2258
Email: flojo4@aol.com

Missouri—St. Louis Area
Place: Saint Louis University Hospital
Meeting: Quarterly
Contact: Carole Haislip, 314-644-1664
Email: carole.haislip@sbcglobal.net

Nevada—Las Vegas
Place: West Charleston Library,
6301 West Charleston Blvd.
Meeting: Email for dates 1-3 PM
Contact: Mary Fatzinger
Email: cmt_suppgroup_lvnv@yahoo.com

New York—Greater New York
Place: NYU Medical Center/
Rusk Institute, 400 E. 34th St.
Meeting: Second Saturday, 12:30-2:30 PM
Contact: Dr. David Younger,
212-535-4314
Fax 212-535-6392
Website: www.cmtnyc.org
Email: bwine@acm.org

New York—Horseheads
Place: Horseheads Free Library on Main
Street, Horseheads, NY
Meeting: Quarterly
Contact: Angela Piersimoni,
607-562-8823

New York (Westchester County)/ Connecticut (Fairfield)
Place: Blythedale Children's Hospital
Meeting: Bimonthly, Jan, March, May,
Sept, and Nov; 3rd Saturday
Contacts: Beverly Wurzel,
201-224-5795
Eileen Spell,
732-245-0771
Email: craneomatic@verizon.net
espell@optonline.net

North Carolina—Triangle Area (Raleigh, Durham, Chapel Hill)
Place: Raleigh, NC
Meeting: Quarterly
Contact: Susan Salzberg,
919-967-3118 (afternoons)
Betsy Kimrey
Email: judae@bellsouth.net

Ohio—Greenville
Place: Brethren Retirement Community
Meeting: 4th Thurs. of
April, July and October
Contact: Dot Cain, 937-548-3963
Email: Greenville-Ohio-CMT@woh.rr.com

Oregon—Portland Area
Place: 1008 NE Divisioin, Suite B
Gresham, OR
Meeting: Quarterly
Contact: Debbie Hagen
Email: hagen84@yahoo.com

Pennsylvania—Johnstown Area
Place: John P. Murtha
Neuroscience Center
Meeting: Bimonthly
Contacts: J. D. Griffith,
814-539-2341
Jeana Sweeney,
814-262-8467
Email: jdgriffith@atlanticbb.net,
cjsweeney@ussco.net

Pennsylvania—Northwestern Area
Place: Blasco Memorial Library
Meeting: Call for information
Contact: Joyce Steinkamp,
814-833-8495
Email: joyceanns@roadrunner.com

Pennsylvania—Philadelphia Area
Place: CMTA Office,
2700 Chestnut St., Chester, PA
Meeting: Bi-monthly
Contact: Pat Dreibelbis or
Dana Schwertfeger,
800-606-2682
Email: info@charcot-marie-tooth.org

Virginia—Harrisonburg
Place: Sunnyside Retirement
Community, Sunnyside Room
Meeting: Bi-monthly, Second Sat. 1-3
Contact: Anne Long,
540-568-8328

Washington—Seattle
Place: U of Washington Medical
Center, Plaza Café—
Conference Room C
Meeting: Monthly, Last Saturday, 1-3 PM
Contact: Ruth Oskolkoff,
206-598-6300
Email: rosk@u.washington.edu

PRODUCT REVIEW:

Journey of the Wild Divine and the StressEraser

BY ELIZABETH OUELLETTE

Are you tired of feeling stressed, anxious, and worried? Do you suffer from chronic pain, high blood pressure, or muscle tension? Do you think the benefits of meditation sound great, but just the thought of taking 15 to 30 minutes out of your busy schedule to let go and relax makes you cringe? If you can relate to any of the above scenarios, I may just have some suggestions involving devices utilizing a technology called biofeedback, or biological feedback.

Biofeedback is a method which enables individuals to observe, monitor, and even alter internal bodily functions, such as heart rate, body temperature, muscle tension, and breathing. Just as a thermometer indicates fever and a blood pressure monitor reveals hyper- or hypotension, biofeedback techniques offer not only a unique glimpse into your physiological being, but also provide the tools with which to modify and regulate the autonomic and unconscious inner systems. In essence, the goal of biofeedback training is to harness the powers of the mind to enhance physical health and psychological well-being.

THE JOURNEY OF THE WILD DIVINE: THE PASSAGE

www.wilddivine.com—$159
amazon.com—$147.95

The Journey to the Wild Divine is a computer-based biofeedback adventure which draws on your potentially untapped, but innate, natural abilities to quiet the mind and calm the body. Upon inserting your fingertips into the three "Magic Rings" biofeedback sensors, vital physiological information is relayed back to you and to the Wild Divine biofeedback software. As you meander along the path of your computer journey, the feedback you receive from attempting to overcome the challenges of the game will lead to a more extensive awareness and mastery over the physical and spiritual self.

The Journey of the Wild Divine is more of a cooperative computer game, appropriate for the entire family, as even children as young as five years of age are able to obtain overall health advantages. I hesitate to refer to this software as just another computer game, as there are no winners or losers and absolutely no competition or violence. The graphics are good; the game is enticing and the creative images are soothing, as is the contemplative music. It is also a creative and user-friendly product.

To help you navigate your quests, interactions with various guides, teachers and allies—whose knowledge of this imaginary world is exemplary—prove advantageous, as they provide timely clues and advice, rendering the challenges and tasks easier to grasp and embrace.

With repetition and practice, a world of fantasy will gradually open itself up to you, a special place where you will discover ancient secrets, perform amazing feats, and carry out tasks once thought impossible. Using focused attention and breathing, juggling balls with laughter, building a stairway through meditation, or making a sphere float through space have never been more delightful or good for reducing stress, tension, and anxiety. You can empower yourself today by creating your own peaceful inner and outer environment by taking a journey into the realms of the Wild Divine.

THE STRESSERASER

www.stresseraser.com—$299.99

The StressEraser also uses biofeedback technology by measuring finger pulse. Normally, pulse rises on inhalation and slows during exhalation. Since breathing influences pulse rate, the StressEraser gives visual and auditory feedback, by way of waves on the screen, to help you achieve optimal breathing patterns and obtain the focus necessary to clear your mind.

Once the StressEraser starts to track your pulse, it will guide you to optimal relaxation states by displaying a series of waves on its screen which reflect internal nerve activity. To achieve maximal breathing patterns, the StressEraser acts as your personal guide by prompting timely exhalation with a visual triangle and/or a series of rhythmic beeps. Your goal is to breathe regularly and deeply so as to create wide, smooth waves at regular intervals. Each wave is scored on a scale of 1 to 3 points or squares, with 3 squares reflecting the most therapeutic respiration and mental focus. This device is portable, easy to use, and even keeps records of each practice session.

It is no exaggeration to say that the StressEraser saved my sanity, if not my life last year during a trip to the Dominican Republic. After a week of fun family activities, I fell ill with some sort of intestinal bug and ended up in the hospital with Spanish-speaking physicians who were considering surgery. YIKES!

After 12 hours of debate, the surgery issue was laid to rest, but after 3 days in the hospital without any food or my usual medications, I felt edgy, anxious, and horribly uncomfortable. The discomfort was so disconcerting, I felt as though my only options were to jump out of my skin or off the roof of the hospital. Thankfully, I remembered my trusty StressEraser, whose familiar sounds and waves drew me in and lulled me into a deep meditative state both day and night. I am forever grateful for the opportunity to personally experience the utility and extraordinary benefits of this mini biofeedback tool.

Currently priced at $299, its potential therapeutic outcome greatly outweighs the cost. It was worth every penny to me. ✻

The CMT "Circle of Friends"

The people who have become involved in the CMTA's Circle of Friends program are making an important contribution that will benefit all of us as we work to find a cure for CMT.

If you'd like to start a Circle of Friends, please call us today at 1-800-606-2682, email us at cof@charcot-marie-tooth.org, or visit us on the web at www.charcot-marie-tooth.org/cof. Additional donations have been made to the following Circles of Friends:

David Corley's Quest:
Ms. Carol A. Gillis

Ethan Spade Walk
Employees of Genesis Health Care

Grace Caldarone Courage Crusade:
Mrs. Linda Alix
Mrs. Holly H. Barton
Mr. Jason Caldarone
Mr. and Mrs. Edmund F. Capozzi
Mr. and Mrs. David G. Carter
Ms. Gemma Dean
Mr. and Mrs. David Elfman
Mr. Chet Feldman
Mrs. Rita D. Gatta
Ms. Elena M. Gemma
Mr. and Mrs. Ronald K. Gourd
Mr. and Mrs. James R. Heagney
Mr. and Mrs. Robert P. Hildum
Mrs. Patricia G. Houston
Mr. and Mrs. Wm. M. Johnston, Jr.
Mr. Vince Mauro
Mr. and Mrs. Gerard McLoughlin
McPhail Associates, Inc.
Mrs. Sandra A. O'Brien
Mr. and Mrs. John A. Papitto
Ms. Joan Wertheimer
Mr. and Mrs. Nicholas J. Zaccagnino

ASK THE DOCTOR

Dear Doctor,

Will the controversial medicine Angioprim help with the numbness in my arms and legs? Could it make my CMT worse if I take it? It says it will help with circulation.

The doctor replies:

This is not a medication regulated by the FDA and does not reveal exactly what is in it. Their website lists a proprietary blend of synthetic amino acids, lysine and cysteine (an antioxidant.) None of these things are known to induce neuropathy, but it is also not known if they accomplish what they advertise to do. I would proceed with caution, especially if the cost is high.

Dear Doctor,

My son who is 8 just saw the neurologist for the first time and has mild sensory deficits in both his feet/legs and hand. Based on my husband's history of CMT and my son's symptoms, they have diagnosed him with CMT. They took a blood sample in order to determine the type of CMT that he has. I forgot to tell his neurologist that he has a pectus. Is there any correlation between CMT and pectus? My son does have breathing issues.

The doctor replies,

I know of no association between pectus (excavatume) and neuropathy of any kind, but I did find one prior publication on this point from a well-respected group. You might want to access: DeRoos S., Ryan M., Ouvier R., Peripheral Neuropathy in cardiofaciocutaneous syndrome. *Pediatric Neurology.* vol. 36, issue 4, pp. 250-252.

Dear Doctor,

I am 65 and have CMT. My gynecologist has recommended Reclast injections for osteopenia, rather than Atenol, after surgery for colon cancer removed most of my colon and part of my rectum. Are there any indications that Reclast injections may increase symptoms of CMT?

The doctor replies:

Zoledronic acid (Reclast) is a newer osteoporosis agent that is also used in other bone disorders such as multiple myeloma and Paget's disease of bone. There is no known risk for neuropathy or CMT, but musculskeletal pain is one side effect in some patients.

Dear Doctor,

My son, who is 20, has CMT. He also has a very rare bone disorder. He had no problems or need for treatment of this disease until recently. He has been put on a 6-month course of interferon alpha daily injections, and a once-monthly intravenous infusion of Zometa. Are either of these drugs problematic for CMT patients?

The doctor replies:

I know of no neuropathy issues with Zometa. There are a few very rare reports of neuropathy being triggered by interferon alpha, but the risk is quite low.

CMTA REMEMBRANCES

Your gift to the CMTA can honor a living person or the memory of a friend or loved one. Acknowledgment cards will be mailed by the CMTA on your behalf. Donations are listed in the newsletter and are a wonderful way to keep someone's memory alive or to commemorate happy occasions like birthdays and anniversaries. They also make thoughtful thank you gifts. You can participate in the memorial and honorary gift program of the CMTA by completing the form below and faxing it with your credit card number and signature or mailing it with your check to: CMTA, 2700 Chestnut Parkway, Chester, PA 19013.

Honorary Gift:
In honor of (person you wish to honor)
____
Send acknowledgment to:
Name:__________________________
Address:________________________
____

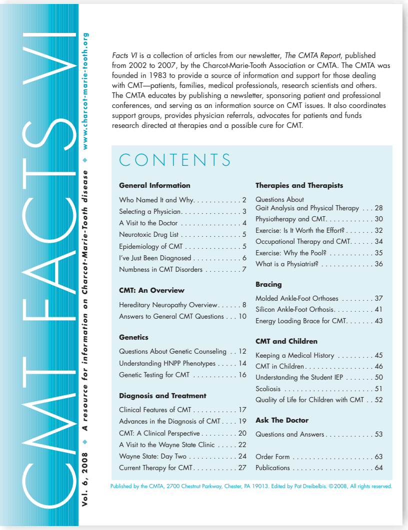
Occasion (if desired):
☐ Birthday ☐ Holiday ☐ Wedding
☐ Thank You ☐ Anniversary ☐ Other

Memorial Gift:
In memory of (name of deceased)
____
Send acknowledgment to:
Name:__________________________
Address:________________________
____

Amount Enclosed:__________ ☐ Check Enclosed
☐ VISA ☐ MasterCard ☐ American Express
Card #__________________________
Exp. Date _______________________
Signature _______________________

Gift Given By:
Name:__________________________
Address:________________________
____

CMT FACTS VI


CMT FACTS VI

Vol. 6, 2008 ◆ A resource for information on Charcot-Marie-Tooth disease ◆ www.charcot-marie-tooth.org

Facts VI is a collection of articles from our newsletter, *The CMTA Report*, published from 2002 to 2007, by the Charcot-Marie-Tooth Association or CMTA. The CMTA was founded in 1983 to provide a source of information and support for those dealing with CMT—patients, families, medical professionals, research scientists and others. The CMTA educates by publishing a newsletter, sponsoring patient and professional conferences, and serving as an information source on CMT issues. It also coordinates support groups, provides physician referrals, advocates for patients and funds research directed at therapies and a possible cure for CMT.

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is now available...

The sixth and newest edition of the CMT Facts Series has just been published and is available for purchase. It is 64 pages in length and is divided into sections on general information, genetics, diagnosis and treatment, therapies and therapists, bracing, CMT and children, and "Ask the Doctor."

The compilation of articles goes back as far as 2002 and captures the most significant articles from The CMTA Report from then to the present. Some of the more interesting articles involve numbness in CMT, HNPP phenotypes, current therapies for CMT by Dr. Michael Shy, exercise options, various types of bracing, IEPs for children, and the interesting and diverse questions posed to members of our Medical Advisory Board and answered by them in a ten-page section of the publication.

The cost for members is $12 and for non-members, $15.

(Please see next page for the entire CMT Facts series and other publications.)

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THE CMT FACTS SERIES: An Indispensable Collection of Articles about CMT

CMT Facts 1

- Facts about professionals who treat patients
- Genetics
- CMT and physical therapy
- CMT foot: surgical options
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- Hope and fear
- For parents
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- Orthotics survey
- Pulmonary function
- Managing pain
- Orthopedics and children
- Shriners Hospitals
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- AFOs and foot issues
- Emotional issues
- Pain and CMT
- Pregnancy
- Social Security
- Vitamins and herbs
- Physical therapy
- Occupational therapy
- Genetic testing
- Medical terminology
- Special section on HNPP

The Patients' Guide to Charcot-Marie Tooth Disorders

2008/178 pages—Now available in print and CD formats

The Patients' Guide is an excellent source of information and resources for patients, family members, and physicians. Topics include CMT in children, genetics and genetic counseling, orthopedic considerations, exercise, and current and prospective treatments for CMT.

Bonus features included on CD: *Charcot-Marie-Tooth Disease: An Overview* and *Charcot-Marie-Tooth Disease: A Guide for Physicians.*

PRINT FORMAT:
Nonmember Price: $15.00
Member Price: $10.00

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Nonmember Price: $10.00
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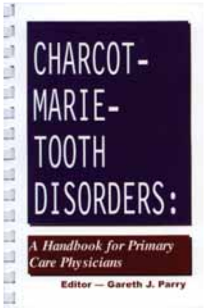
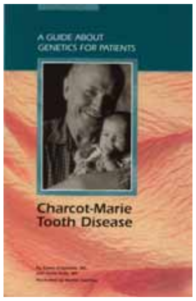


Handbook for Primary Care Physicians

1995/130 pages

Edited by Dr. Gareth J. Parry, Professor of Neurology at the University of Minnesota, the *Handbook for Primary Care Physicians* is an excellent source of information about the causes, symptoms, and treatment/management of CMT. Patients will also want to read it.

Nonmember Price: $20.00
Member Price: $15.00

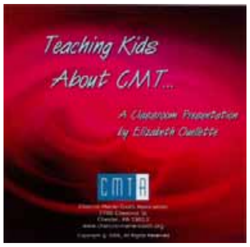


A Guide about Genetics for Patients

2000/21 pages

Illustrated with easy-to-understand diagrams, this booklet outlines the basics of genetic inheritance and CMT.

Nonmember Price: $5.00
Member Price: $4.00



Teaching Kids about CMT... A Classroom Presentation

2006/DVD 1 hr.

This hour-long DVD of an actual classroom presentation demonstrates a number of games and other exercises to teach classmates of children with CMT about the disorder.

Nonmember Price: $10.00
Member Price: $7.50

LETTERS

Dear CMTA,

I am concerned over the typical response in your newsletters to the effect of prescription drugs upon those of us with CMT. I notice, from time to time, the following type of question and answer.

Q: Are statins safe for CMT patients to take?

A: There are no data to show that statins should not be taken by CMT patients.

I believe that when both negatives are removed that the resulting statement is, clearly, not true. I believe that the absence of proof is NOT proof of absence. It makes your typical answer, at a minimum, misleading. When I asked my neurologist the same question I received a different answer. He indicated that there have been NO safety studies for statins in a population of only CMT patients. Given the low incidence of CMT in the population, it is not likely that any adverse effects would be detected in studies of the general population without specific subset analysis.

As it is possible that an effect of a drug on CMT patients, or patients in general, may not be reversible, even if the drug in question is stopped, a better wording is necessary for answers to questions about the possible effects of drugs where no study on the CMT population has been performed.

—M G. PhD

(Editor's Note: The doctor who answers most of our drug questions replied: "As far as the statin question goes, the area is still controversial, but the man is correct in that there are no studies with statins and CMT. That is why we advise weighing the known benefit against the small, but uncertain, degree of risk, before using the drugs.")

Dear CMTA,

It was September 5, on Labor Day weekend. I was a good roller skater, but I was having so much pain in my leg. I came home and was crying because I was in so much pain. My Mom finally decided we should go to the hospital. The first time we went, they told me it was a pulled muscle and they sent me home with pain mediation. The next day, the pain was worse, so we went back. After an x-ray, the doctor took my Mom to another room. She came back and told me that they had found a tumor in my pelvis. The tumor was cancer.

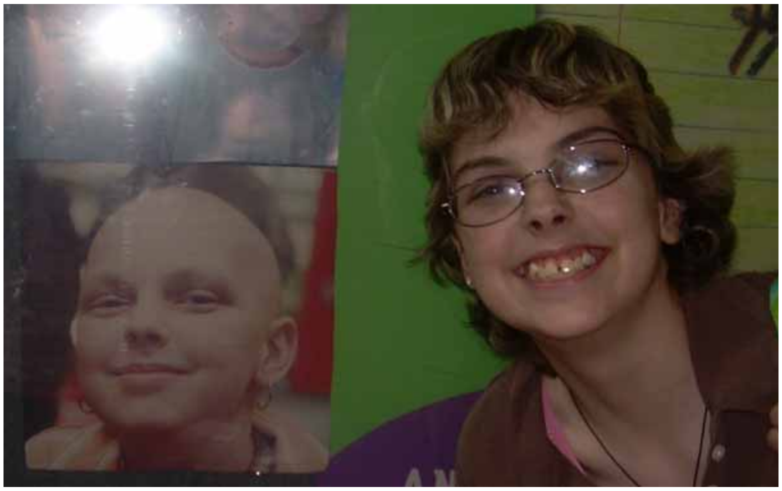
I thought I could have chemotherapy and everything would be fine. I was wrong. I had a chemo drug called Vincristine and that started "it" all. I had inherited CMT from my mother's side of the family. Two weeks went by and I had to go back in for more chemo. When I tried to get out of the car, my Mom had to drag me because my leg was not working right, but it got better once I started walking, so I thought it was no big deal.

While I was visiting the fish tanks in the hospital with my uncle, my leg collapsed under me. My uncle caught me before I hit the floor. Apparently, the chemo brought out my CMT and made it worse.

My hands and feet were paralyzed. I could not do anything. It was so hard at age 13 to count on everyone to do everything for me. I am doing better now, but I never know if I will fully be back to normal. I try to take life one day at a time.

(As a note, if anyone with CMT has the opportunity to try serial casting, you should know that it made my hands much better and has improved things for me so much.)

—Christina



Christina during chemo (left), and after.

WRITE TO US!

**Pat Dreibelbis,
Editor,
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**Dana Schwertfeger,
Director of Member Services
dana@charcot-marie-tooth.org**

CMT PATIENT MEDICATION ALERT:

Definite high risk (including asymptomatic CMT):
Vinca alkaloids (Vincristine)

Moderate to significant risk:
Amiodarone (Cordarone)
Bortezomib (Velcade)
Cisplatin and Oxaliplatin
Colchicine (extended use)
Dapsone
Didanosine (ddI, Videx)
Dichloroacetate
Disulfiram (Antabuse)
Gold salts
Lefluonamide (Arava)
Metronidazole/Misonidazole (extended use)
Nitrofurantoin (Macrodantin, Furadantin, Macrobid)
Nitrous oxide (inhalation abuse or vitamin B12 deficiency)
Perhexiline (not used in US)
Pyridoxine (mega dose of Vitamin B6)
Stavudine (d4T, Zerit)
Suramin
Taxols (paclitaxel, docetaxel)
Thalidomide
Zalcitabine (ddC, Hivid)

Uncertain or minor risk:
5-Fluouracil
Adriamycin
Almitrine (not in US)
Chloroquine
Cytarabine (high dose)
Ethambutol
Etoposide (VP-16)
Gemcitabine
Griseofulvin
Hexamethylmelamine
Hydralazine
Ifosfamide
Infliximab
Isoniazid (INH)
Lansoprazole (Prevacid)
Mefloquine
Omeprazole (Prilosec)
Penicillamine
Phenytoin (Dilantin)
Podophyllin resin
Sertraline (Zoloft)
Statins
Tacrolimus (FK506, Prograf)
Zimeldine (not in US)
a-Interferon

Negligible or doubtful risk:
Allopurinol
Amitriptyline
Chloramphenicol
Chlorprothixene
Cimetidine
Clioquinol
Clofibrate
Cyclosporin A
Enalapril
Fluoroquinolones
Glutethimide
Lithium
Phenelzine
Propafenone
Sulfonamides
Sulfasalazine

What is CMT?

- is the most common inherited neuropathy, affecting approximately 150,000 Americans.
- may become worse if certain neurotoxic drugs are taken.
- can vary greatly in severity, even within the same family.
- can, in rare instances, cause severe disability.
- is also known as peroneal muscular atrophy and hereditary motor sensory neuropathy.
- is slowly progressive, causing deterioration of peripheral nerves that control sensory information and muscle function of the foot/lower leg and hand/forearm.
- causes degeneration of peroneal muscles (located on the front of the leg below the knee).
- does not affect life expectancy.
- is sometimes surgically treated.
- causes foot-drop walking gait, foot bone abnormalities, high arches and hammer toes, problems with balance, problems with hand function, occasional lower leg and forearm muscle cramping, loss of some normal reflexes, and scoliosis (curvature of the spine).
- has no effective treatment, although physical therapy, occupational therapy, and moderate physical activity are beneficial.
- is usually inherited in an autosomal dominant pattern, which means if one parent has CMT, there is a 50% chance of passing it on to each child.
- Types 1A, 1B, 1C, 1D (EGR2), 1E, 1F, 1X, 2A, 2E, 2I, 2J, 2K, 4A, 4E, 4F, HNPP, CHN, and DSN can now be diagnosed by a blood test.
- is the focus of significant genetic research, bringing us closer to solving the CMT enigma.

THE CMTA *Report*

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