What is Myelin Disease?

A vital fatty membrane called myelin insulates every nerve of the brain and spinal cord. Like the plastic sheath around an electrical wire, myelin assures the proper conduction of nerve impulses from one part of the body to another. When this sheath is damaged, the results can be devastating. Each year, thousands of children and young adults fall prey to inherited metabolic disorders or acquired diseases, like the leukodystrophies and multiple sclerosis, which selectively attack this vital membrane. The symptoms include impaired vision, hearing, balance, speech, mobility and cognition; in many cases resulting in complete paralysis or early death.

Myelin diseases are only partly understood. Most are untreatable and progressive. Some, particularly those that strike children, are rapidly fatal. Every minute counts in the battle to defeat them. But even if effective treatments to halt the loss of myelin could be found tomorrow, they would neither reverse neurological damage nor restore lost function which has already shattered millions of lives.

About The Myelin Project

This is exactly why the parents of one young boy struck by adrenoleukodystrophy (ALD) launched The Myelin Project, putting its crucial agenda on track. Since its inception in 1989, The Myelin Project has mobilized hundreds of volunteers from other afflicted families around the world, and has funded practical myelin research while inducing international scientific collaboration. The Myelin Project currently has branches in The United Kingdom, Germany, and Italy.

Unlike many self-perpetuating medical charities, The Myelin Project is wholly committed to putting itself out of business as quickly as possible — as soon as remyelination treatments become standard in medical practice. We are keeping our organization lean and responsive, staffed almost entirely by volunteers. Our quick yet thorough review process allows us to evaluate and approve funding for cutting-edge research proposals without wasteful duplication and unnecessary administrative overhead. Nearly 88% of the money donated to The Myelin Project goes promptly and directly into applied research.

Our mission is to:
1. Raise awareness of demyelinating diseases
2. Induce scientific collaboration
3. Provide general information for patients and their families
4. Fund myelin research, to…
5. Find a cure for demyelinating diseases

Fundraising for The Myelin Project

The Hammerfest Triathlon is a USAT sanctioned sprint distance triathlon that takes place at the picturesque Owengro Inn Beach and Tennis Club in Branford, CT. It is organized by Jean Kelley, board member of The Myelin Project, and Race Productions. The triathlon boasts commanding views of the Long Island Sound. A cookout and Fun Run follow the race. Race Productions has over 15 years of experience producing multi-sport races, including Hammerfest and Brian’s Beachside Boogie, an early spring duathlon held at Hammonasset State Park in Madison. All proceeds from these events go to The Myelin Project.

Fourteen years ago, the staff of Race Productions came to know and be inspired by a young boy in the Branford community. At that time, 9 year-old Brian Kelley was struggling with the effects of adrenoleukodystrophy, ALD, an x-linked disease of the central nervous system. At the age of 6, Brian was a typical little boy, doing well in first grade and skiing black diamond trails with his family. That March, he was diagnosed with ALD. The treatment at the time was a bone marrow transplant; Brian would get a missing enzyme from his brother in hope of arresting the disease before it would impact him. Unfortunately, by the end of August, the effects of the disease took away Brian’s ability to walk, talk or speak. Now at the age of 23, despite the many disabilities with which Brian copes each day, he continues to inspire us all. In 1996, Race Productions decided The Myelin Project would be the beneficiary of future races in Brian’s honor.

Dr. Jeff Kocsis (Yale University) has shown that mesenchymal stem cells derived from adult human bone marrow when transplanted into experimental demyelinating lesions, produce a significant improvement in functional outcome. They do this by stimulating new myelin formation.

Ian Duncan, Ph.D. at the University of Wisconsin-Madison and his colleagues have worked extensively on the cells that could be used for transplant repair including human embryonic and neural stem cells. Most recently they have provided the first definitive proof that widespread remyelination restores function, confirming that this is an important therapeutic strategy. Ian is the chairman of The Myelin Project’s Scientific Advisory Board.

In Milan, Italy Dr. Luigi Naldini (San Raffaele Telethon Institute for Gene Therapy) and his group have been working on gene therapy for the treatment of two severe leukodystrophies, metachromatic leukodystrophy and Krabbe’s disease. Their work is based on using lentiviral vectors in combination with hematopoietic stem cells and is heading into Phase III clinical trials.
Also in Milan, Dr. Gianvito Martino’s lab (San Raffaele Hospital) has been working in the field of stem cell biology, particularly neural stem cells. They have shown that these cells, when transplanted into models of MS, have marked effects on the immune system and help protect against neurodegeneration. The goal is to take this approach into clinical trials.

Dr. Robin Franklin’s group at the University of Cambridge have been working on the idea that the brain contains a population of endogenous stem cells or progenitors that can be recruited to become myelinating cells in MS, and possibly other myelin disorders. They have identified some key molecules that might be used in future clinical trials, to promote ‘host’ repair.

Dr. Wolfgang Brück (University of Göttingen) has been studying the immunopathology of MS lesions in attempts to unravel the mechanisms behind endogenous remyelination. It appears that in MS lesions, sufficient myelin-forming precursor cells are present but they do not differentiate into mature cells.

In Paris, Dr. Anne Baron Van Evercooren (L’Hopital de la Salpetriere) and her colleagues have been involved in studying remyelination, both by transplanted cells (exogenous) and by host cells (endogenous). They have worked both with myelinating cells from the peripheral nervous system and central nervous system. They have shown activation of neural stem cells from key areas of the CNS in animal models and in the brain of MS patients. They have also evaluated the biological properties of human fetal neural precursors on transplantation into animals.

Thinking about making a tax-deductible charitable contribution to myelin research? Here are a few things to consider...

- Currently, The Myelin Project’s administrative and general fundraising expenses account for less than 12.5% of our total support and revenue ensuring that your donation goes to RESEARCH and EDUCATION, not travel expenses and overhead costs.
- Your donation will go toward financing practically oriented experiments conducted within the framework of a coherent overall plan. All research proposals are reviewed in advance by our panel of leading experts in the field, The Myelin Project’s Scientific Advisory Board.
- We promise you that your donation will be well-spent, funding those proposals that are most likely to yield clinically relevant results. Your donation is 100% tax deductible in the U.S. to the full extent of the law.