



BethanysHope
FOUNDATION
Research Leukodystrophy. Live for the Cure.

Friday, October 05, 2018

Dr. Adeline Vanderver, MD
Program Director of the
Leukodystrophy Center of Excellence
Children's Hospital of Philadelphia
3615 Civic Center Blvd.
Philadelphia, PA 19104

Dear Dr. Vanderver, Dr. Eichler and Dr. Fatemi,

On behalf of the Bethanys Hope Foundation, its members and partners, we are extremely pleased to offer a letter of support for GLIA's RDCRN application. It is indeed a pleasure to write supporting the "*Global Leukodystrophy Initiative Clinical Trials Network*". Our Foundation, along with the World Leukodystrophy Alliance, is encouraged by the prospect of significant funding for your research efforts into rare diseases. More often than not the plight of patients and families fighting every day with the devastation of leukodystrophy is set aside for more common and better understood diseases. Your project will undoubtedly help make further progress to achieve the ultimate goal, and end to leukodystrophy in all its forms.

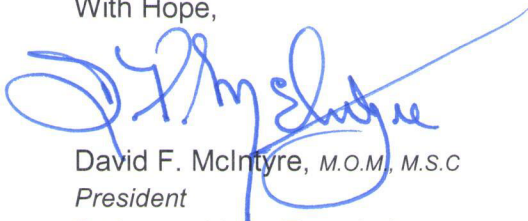
Since our daughter – Bethany – was diagnosed with Metachromatic Leukodystrophy (MLD) in August 1995 the challenges presented have been numerous. Being told our child had an incurable disease that ultimately carried a death sentence is news that no one should ever have to endure. At that time, due to a lack of funding there was very little MLD Research being conducted anywhere around the world with the exception of Dr. John Barranger at the University of Pittsburgh Medical Centre. With the compassion, direction and guidance shown by Dr. Barranger the Bethanys Hope Foundation was established in July 1996 with the idea of developing fundraising mechanisms that could help support his research. However, it was soon realized that a Canadian initiative would be required to ensure a seamless transition of fundraising dollars from our Foundation to a team of researchers. Therefore, under the direction of Dr. Tony Rupar, in conjunction with Western University (London, Canada), the *Bethanys Hope Leukodystrophy Research Laboratory* was established in November 1999. We are proud of the medical breakthroughs that are being made in this world class laboratory, including the prospect of a clinical trial here in London for a MLD treatment in the very near future. Our Foundation has fought tirelessly to ensure that the faces and names of those suffering from the devastation of leukodystrophy are not forgotten, but instead given a voice of hope and dignity.

To date, Bethanys Hope Foundation – with the help of our generous community, dedicated corporate partners and numerous Volunteers – has raised over \$4 Million for MLD Research. We truly believe that collaboration between our research team and leukodystrophy efforts around the world will allow for significant breakthroughs.

The Bethanys Hope Foundation fully supports the mandate of GLIA and looks forward to a successful grant application with the RDCRN. As we indicated many years ago in a similar letter supporting GLIA (September 29, 2013) our Team is willing to serve on the GLIA-CTN advocacy committee, as well as pilot & data committees. We have attended and continue to be committed to attend the GLIA annual meetings. Our Foundation has been actively working with Dr. Genevieve Bernard at McGill University - Children's Hospital (Montreal) and Dr. Rupar from Western University (London) in an international MLD Natural History Study. We are also co-funding *The Canadian Pediatric Society Surveillance Program* (CPSP) to study the incidence of these rare conditions in Canada under the direction of Dr. Sunita Venkateswaran MD, FRCPC, Assistant Professor, Pediatric Neurology, Children's Hospital of Eastern Ontario, University of Ottawa. We believe that a research consortium with the interests of rare diseases at the forefront will help provide a voice to so many who may feel underrepresented. Dr. Rupar and our entire research team are available anytime to collaborate on your initiatives and provide further detail on the world class, cutting edge research that is taking place here in London. We look forward to strong and productive working relationship that will help make inroads in leukodystrophy research.

Should you require anything further in support of your application, please do not hesitate to contact us at your convenience.

With Hope,



David F. McIntyre, M.O.M., M.S.C
President
Bethanys Hope Foundation
London, Ontario, Canada