



www.thecalliopejoyfoundation.org

Dear Dr. Vanderver;

On behalf of the Calliope Joy Foundation, we are pleased to write a letter of support for the Leukodystrophy Center of Excellence at the Children's Hospital of Philadelphia's application to the NIH's Rare Diseases Clinical Network. We understand this award would facilitate collaboration, provide funding, and raise the profile of the exciting work in this field. Since our daughter Calliope's diagnosis with leukodystrophy back in 2012, it has been exciting and meaningful for us to witness breakthroughs in clinical care and research that are transforming what it means to have leukodystrophy for our community of patients and families.

To assist with your application to the NIH, we are happy to review the current and future philanthropic and advocacy commitments we will make to the program at CHOP and your collaborators at partner institutions.

Since 2013, the Calliope Joy Foundation has provided \$235,000 in direct funding to projects for research and clinical care for the Leukodystrophy Center.

Our foundation also provides care packages to newly diagnosed patients, travel grants for patients seeking financial assistance to travel to CHOP in order to participate in clinical trials. The travel grant program also assists families receiving treatment in the US and abroad. Since 2013, our care package and travel grant program have provided approximately \$35,000 in-kind support and direct cash funding to families in the US.

Since 2013, The Calliope Joy Foundation has provided \$20,000 to support talented college undergraduates from Wellesley College who are interested in careers pediatric neurology with a focus on leukodystrophy. We would expand this program to include partner hospitals and research centers through the NIH grant.

Since 2016, the Calliope Joy Foundation has spent \$20,000 on educational videos about newborn screening, genetic counseling, gene therapy, and palliative care as part of our an

on-line forum called the Leukodystrophy Family Forum (www.leukodystrophyforum.com). An important part of our advocacy outreach has also included the publication of two children's books. The first, *Loie's Disease*, explains leukodystrophy in terms children can understand. The second book, *"Fixing Genes and Treating Diseases"* explains the science of gene therapy with illustrations and language young people can understand. We expect to continue and expand such advocacy and hope to hire a group of family ambassadors to do more direct outreach to patient families and host a monthly on-line support group with trained moderators.

We have offered financial support to help cover the expenses related to family conferences for H-ABC leukodystrophy and AGS-leukodystrophy, and support the annual GLIA meetings. We expect to grow our role in the support of such family conferences as more natural history studies, compassionate use studies, and clinical trials begin.

Our foundation has worked closely on newborn screening efforts across the country and has sought to support the work of our partner patient advocacy groups as they work with RUSP and state officials on how best to implement newborn screening and facilitate follow up care.

In 2020, our foundation is excited to partner with GLIA to co-host an umbrella research and family conference that brings together stakeholders from industry, clinicians and researchers, and patient advocacy groups with an expanded and special focus on how leukodystrophy patients can participate in clinical trials.

Finally, we would be happy to serve on the advocacy committees for the GLIA-CTN, and participate in the executive, data, pilot and career development committees as part of the administrative activities of the consortium

In conclusion, please know the Calliope Joy Foundation is dedicated to supporting this exciting collaboration and we are eager to build on our partnerships to improve care, fund research, and better support families living with leukodystrophy.

Sincerely,

Patrick J. Carr and Maria Kefalas
Co-founders, the Calliope Joy Foundation