



September 21, 2018

Dear Adeline,

As long standing collaborators on Aicardi-Goutieres Syndrome (AGS) research for more than nine years, we at the Aicardi-Goutieres Syndrome of Americas Association (AGSAA) enthusiastically look forward to supporting your application to the NIH's U54 Rare Diseases Clinical Research Consortia (RDCRC) for the Rare Diseases Clinical Research Network (RDCRN) funding opportunity.

As you know, AGSAA has evolved to become an ambitious collective of dedicated parent advocates working alongside specialist care teams and researchers to eradicate Aicardi-Goutieres Syndrome and improve the lives of its impacted families. In order to change the world for our children and those yet to be diagnosed, we seek to mobilize human and financial resources to achieve results. Everything we do reflects urgency to find solutions.

In our ongoing partnership with you, the Vanderver Lab, and the Leukodystrophy Center of Excellence (LCE) at CHOP, we benefit from a network of tireless experts paving the way to a brighter future for leukodystrophy identification and treatment. Some examples of our joint collaboration efforts and financial contributions to date, many of which are ongoing, include:

- Expansion of a promising compassionate care clinical research trial (JAGA) - \$250,000
- Active support of AGS research program, including recent facilitation of a scientific research grant - \$10,000
- Co-planning role and partial sponsorship of the 2019 AGS Family & Scientific conference - \$15,000; additionally spearheading peer to peer fundraising efforts for supplemental family travel grant fund
- Financial support of The Calliope Joy Foundation's 'TJ's Travel Grants' program to aid more families facing an AGS or similar diagnosis in traveling to CHOP for highly specialized care and treatment options at the Leukodystrophy Center of Excellence - \$10,000
- Fundraising support for both development and pilot stages of a newborn screening platform seeking to identify early, and subsequently treat AGS in infants
- Support in recruitment and retention to studies along with our designation and articulation of CHOP's LCE clinic as our recommended center of care for AGS families

- Foundational management of patient advocacy efforts and moderation of parental support group
- Development of an advocacy campaign to enhance early recognition of AGS in the first months of life via targeted communication to ER, pediatric, and NICU doctors and staff (2019)
- Development of AGS standards of care and accompanying resources for parent and caregiver education (2019)

As parents of AGS children, both living and deceased, we deeply acknowledge the need for a greater understanding of disease cohorts to predict outcomes of earlier diagnosis. The equation is simple: earlier diagnosis + continued development of viable treatment options = hope. To that end we are passionate about making the Newborn Screening a reality for our community, so that new families affected by AGS will not have to suffer the same outdated and unfortunate ramifications of identifying a rare disease. In the case of AGS this can include incessant misdiagnoses and ineffectual - at times even detrimental - treatments and interventions, rampant misinformation at most levels of care, and emotionally draining diagnostic odysseys. We aspire to help families at all levels of their journey and are preparing accordingly to mirror your work with thoughtfully developed supportive resources.

We also welcome the opportunity and honor to participate in the Advocacy Committee for GLIA. This role will allow us to assist in identifying patient and patient family priorities for AGS and in further championing advocacy and treatment efforts across all leukodystrophies.

We are truly thrilled at the opportunity to offer assistance in this important endeavor. Thank you so much for including our Association in what will undoubtedly be an integral step forward in further validating research and viable treatment options for this often debilitating disease.

Sincerely,  
Devon Cordova, VP  
pp: Board of Directors, AGSAA

A handwritten signature in cursive script, appearing to read "Devon Cordova".