

December 4, 2025

Dear Members of the Application Review Subcommittee,

My name is Erin Sullivan, and I am writing on behalf of GliaConnect, a patient advocacy organization serving individuals and families affected by microgliopathies, with a primary focus on ALSP and related leukodystrophies. Our mission is to support patients, elevate community needs, and advance treatments that address the underlying biology of these disorders.

We are encouraged by the work of Savanna Biotherapeutics to develop microglia cell therapies for ALSP, and we appreciate their consistent engagement with our patient community. Their scientific program addresses a critical gap in current treatment options, and their collaboration with families has strengthened trust and transparency—both essential in disease research.

Across our community, we have seen the significant limitations of bone marrow transplant (BMT) for ALSP. Several patients who underwent BMT have continued to progress with little clinical benefit, and we have witnessed multiple tragic outcomes - including death, accelerated decline, negative reactions, and substantial weakening from pre-conditioning, the BMT transplant, and/or infections that develop from the procedure. Further, there are published reports of patient death in presymptomatic patients undergoing BMT. These experiences underscore the urgent unmet need for safer, more effective therapeutic approaches for ALSP.

We are also encouraged by the growing body of data emerging from neurologists and specialty clinics, which shows a rapid increase in the number of ALSP patients being identified and genetically confirmed. This trend reflects both heightened clinical awareness and the expanding availability of diagnostic tools. Importantly, it also underscores the significant potential impact of Savanna's approach: as more patients are recognized earlier in the disease course, a targeted microglia therapy could benefit a rapidly increasing ALSP community. The expanding patient landscape strengthens the rationale for Savanna's program and highlights the urgency of advancing it toward the clinic.

We thank CIRM for its support of Savanna's efforts and for recognizing the importance of advancing innovative therapies for microgliopathies. Your partnership accelerates not only scientific progress but also hope for families facing a devastating and rapidly progressive disease.

I'll close by highlighting the exciting opportunity presented by Savanna's ALSP program: a gateway to treating the many microglia-related disorders affecting GliaConnect patient communities, such as BANDDOS, Nasu-Hakola Disease, Alzheimer's, and many other microgliopathies that may one day be addressed through a microglia-centered approach.

Thank you for your continued commitment to addressing the needs of the ALSP community.

Sincerely,

A handwritten signature in black ink that reads "Erin Sullivan".

Erin Sullivan
Executive Director