

SYNGAP RESEARCH FUND

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Application Review Subcommittee
California Institute for Regenerative Medicine
601 Gateway Blvd #400
South San Francisco, CA 94080

August 24, 2024
Mill Valley, CA

RE: DISC4-16360 Proposal; PI: Dr. Giorgia Quadrato

Dear CIRM Application Review Subcommittee,

I am writing to you today as both a patient advocate and a father of a child with SynGAP1-related intellectual disability. As the CEO of the SynGAP Research Fund, I have dedicated my life to advancing research and improving outcomes for individuals affected by genetic disorders that cause intellectual disability. I urge you to fund the application to discover electrophysiological biomarkers for stratifying intellectual disability, as this research is crucial for the California population and beyond.

My personal journey with my child has shown me the immense challenges faced by families dealing with intellectual disabilities. SynGAP1, which accounts for up to 2-5% of all ID cases, is just one of many genetic causes of intellectual disability, each presenting its own unique set of challenges. However, what unites all these conditions is the urgent need for better diagnostic tools, more accurate prognoses, and targeted interventions.

Electrophysiological biomarkers hold tremendous promise in this regard. Here's why funding this research is so important:

- **Precision Medicine:** These biomarkers could allow us to move beyond broad diagnoses and toward a more nuanced understanding of each individual's condition. This precision is crucial for developing targeted therapies.
- **Early Intervention:** With better biomarkers, we could identify issues earlier in a child's development, allowing for more timely interventions that could significantly improve long-term outcomes.
- **Treatment Efficacy:** As we develop new treatments, these biomarkers could serve as objective measures of efficacy, accelerating the drug development process.

- Resource Allocation: For families and healthcare providers, understanding the specific subtype and severity of intellectual disability can help in allocating resources and support more effectively.
- Research Acceleration: These biomarkers could become powerful tools in research, helping us understand the underlying mechanisms of intellectual disability and potentially leading to breakthrough treatments.

With her proposal, Dr. Quadrato has attracted world-leading scientists from top universities in the Los Angeles area new to the field of mental illness. The benefits of this research would extend to countless families with rare genetic forms of ID.

As someone, who has navigated the challenging landscape of rare genetic disorders, I can attest to the hope that advancements like this bring to families. Every step forward in our understanding of intellectual disability is a step toward better lives for our children.

I implore CIRM to recognize the transformative potential of this research. Your support would not only benefit the immediate population of California but could also pave the way for advancements that help children and families worldwide.

Thank you for your consideration of this critical research opportunity.

Sincerely,

Mike

J. Michael Graglia

Tony's Father, Patient Advocate, & CEO of SynGAP Research Fund

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