

From: Laura Mitic <laura.mitic@bluefieldproject.org>

Sent: Wednesday, April 10, 2024 5:10:03 PM

To: lgoldstein@ucsd.edu <lgoldstein@ucsd.edu>

Subject: Request to include FTD as a neurodegenerative disease eligible for CIRM funding

Dear Dr. Goldstein,

I write to you, chair of CIRM's Neuro Task Force, to advocate for the inclusion of FTD as a target neurodegenerative disease eligible for future CIRM funding. I unfortunately cannot attend the upcoming April 17 task force meeting, so below I share a few thoughts about why FTD is appropriate for inclusion.

While most FTD cases are sporadic, mutations in over 10 genes, most of which have no known relationships with each other, have been identified as FTD-causing. FTD is an umbrella description of several clinical syndromes, which share common clinical symptoms despite different disease-causing mechanisms. Greater molecular understanding of this heterogeneous pathophysiology of FTD is much needed, and CIRM funding would make significant impact in enabling cell-based research.

There are several FTD centers of excellence in California, with experienced investigators who would be interested in applying for funding. Indeed, the Gladstone Institutes and UCSF were an early recipient of CIRM funding (grant # RL1-00650-1), which enabled the creation of a human dermal fibroblast repository (and the subsequent creation of iPSCs) from FTD patients and family member controls. These cells have been shared widely with researchers and have contributed significantly to fundamental discovery research, as well as to therapeutic development. Indeed, for one form of FTD, caused by mutations in progranulin, there are now 6 potentially disease-modifying investigational therapeutics being tested in human clinical trials.

With established natural history studies underway (co-led by California-based investigators), robust and collaborative advocacy partners, and the potential for a clear regulatory path (if drugs for progranulin-FTD are approved), the broader FTD field is well-positioned to take advantage of the transformative funding that CIRM provides.

I encourage the Neuro Task Force to include FTD as a neurodegenerative disease eligible for funding. If I can provide additional information, please do not hesitate to contact me.

Thank you and best wishes,
Laura

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