



April, 15, 2024

Attention: Task Force on Neuroscience and Medicine of the Independent Citizen's Oversight Committee California Institute for Regenerative Medicine

Re: Written Public Comment for 4-17-24 Meeting

Greetings Committee Members

Genetic ALS & FTD are two terrible inherited diseases striking most often in late middle age when most people are still working and saving for the future. The devastation these diseases have on individuals and families cannot be overstated. ALS ends in death for most within three years after diagnosis, with nearly all passing within five years of diagnosis. In FTD, the ability to live and conduct daily life independently is lost within a few years of diagnosis, and death often occurs within 7-10 years of diagnosis.

There is a shared genetic, phenotypic, and biological overlap between these two diseases. As an asymptomatic carrier of the *C9orf72* repeat expansion, the most common genetic cause of both ALS and FTD, I know it cannot currently be predicted which phenotype I will develop. The haunting fate I and so many others at risk of genetic ALS and FTD face is shared by many other Californians at risk for Huntington's Disease, Early Onset Alzheimer's, inherited Parkinson's, and even those with dual *APOE* e4 copies, which all together likely number close to a million Californians at severely elevated risk of these terrible diseases.

Given the above, we call on your committee to adopt two common sense positions:

- 1) Include FTD formally as an included disease in your remit. It could be inferred, given the clear coverage of ALS, but having it made explicit will benefit all.
- 2) Ensure that CIRM is taking advantage of the wealth of genetic knowledge and drive towards genome-editing cures for genetic brain diseases. Of note, there is a shared problem across all brain diseases in the inability to deliver CRISPR components to the brain with current technology (without harming the recipient). Funding breakthroughs in brain delivery for CRISPR will be a sea change in the futures of hundreds of thousands of Californians like myself and so many others.

In appreciation of your hard work on these issues,

Jean Swidler
Executive Director
Genetic ALS & FTD: End the Legacy
EndTheLegacy.org