

Dr Laura Brady
Fighting Blindness
3rd Floor, 7 Ely Place
Dublin 2
Ireland

October 2018

Re: Letter of Support for CIRM DISC2-10973 Application “Small Molecule Proteostasis Regulators to Treat Photoreceptor Diseases” (PI: Jonathan Lin)

Dear Members of the California Institute for Regenerative Medicine (CIRM) Governing Board and Independent Citizens’ Oversight Committee:

Fighting Blindness Ireland write to strongly support Dr. Jonathan Lin’s CIRM DISC2-10973 Application entitled “**Small Molecule Proteostasis Regulators to Treat Photoreceptor Disease.**”

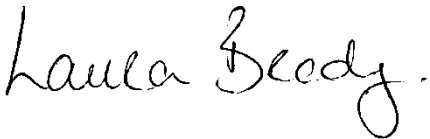
Fighting Blindness Ireland is a patient-led organisation funding research both nationally and internationally towards the development of treatments and cures for genetic forms of sight loss. We represent those living in Ireland with inherited retinal degenerations (IRDs), of which an estimated 5,000 people are affected. At present, there is no approved treatment available for life-altering, often debilitating and progressive diseases such as achromatopsia and cone-rod dystrophies. As such, translational research that addresses the underlying genetic causes of IRDs and which has the potential to lead to new therapeutic modalities remains at the centre of our research strategy. In parallel with our mission to CURE, we also provide counselling services for families affected by sight loss, and are extremely active in the area of advocacy and patient empowerment.

The impact of losing one’s vision should not be underestimated. There is a social, emotional and psychological impact not just on the individual affected by an IRD but also on their family and friends. Additionally, IRDs bring with them a significant cost burden on health services in each country as well as the wider impact they have on the economy. Taking all this into consideration and recognising the absence of therapeutic interventions, this present application addresses an important unmet need within the IRD community.

Dr. Lin's CIRM proposal offers hope to these patients by testing new treatments to prevent vision loss using stem cell models derived from our patients with retinal disease and thereby Fighting Blindness enthusiastically support this research study.

In conclusion, we believe this application and direction of study holds the potential to benefit many stakeholders including the academic, clinical and patient community wherever the geographical location.

Yours sincerely,



Laura Brady, PhD

Research Manager, Fighting Blindness