ACHROMA CORP

Funding a Cure for Achromatopsia

10/02/18

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:

I write to strongly support Dr. Jonathan Lin's CIRM DISC2-10973 Application "*Small Molecule Proteostasis Regulators to Treat Photoreceptor Disease*." Achroma Corp. is a federally recognized 501 (c)(3) non-profit dedicated to funding and expediting a cure for those affected with achromatopsia. We represent patients with achromatopsia throughout the country and state of Pennsylvania. We strive to raise funds to cover the considerable expenses of research, development, and patient studies, which will result in clinical trials to treat achromatopsia. Achromatopsia causes the complete absence of cone photoreceptor function, (which are the cells in our retina we rely on to read, walk safely, drive, see faces and color, and to visually navigate through life). As a result, achromatopsia patients are 'day blind' (cannot see outside in daylight without dark tinted glasses), have extreme sensitivity to light, poor acuity, nystagmus (rapid uncontrolled eye movements), and color blindness. In simple terms, normal eye cones help the eyes adjust to bright lights, see detail at distances, and differentiate colors. We strongly urge CIRM to support research into achromatopsia because currently, **there is no cure**.

Please allow me to explain a bit further how achromatopsia affects a child. My son, Raymond (now age 12) was diagnosed with achromatopsia when he was an infant. The most significant problem that Raymond deals with each day is extreme light sensitivity and day blindness. The best way to describe this is in terms of what we all can experience when leaving a dark movie theater and walking into bright sunlight. That sudden "white out" glare you may experience only for a few seconds is what Raymond can experience all day in bright lighting without his tinted glasses. However, for most of us, cone cells quickly take over and our eyes can adjust to the bright light. Raymond, however, has no cone function, and therefore his eyes cannot adjust to bright lighting, leaving him trapped in that "white out" state. If his eyes become too overwhelmed with the light, he becomes literally blinded by the light (will not be able to see much of anything). Fluorescent lighting as well as UV light negatively affect his vision. Controlling the lighting (and therefore the amount of light that enters the retina) by using specific custom-tinted glasses, contacts, or both, is essential to maximizing his visual functioning.

Raymond needs to be close to things in order to see them clearly as he has very poor DISTANCE vision. He cannot see things high on the wall or far away (such as a smart board, chalk board, TV) without the use magnification aides; he needs to get close to everything he does. Depending on the size of the room/setting, he cannot recognize classmates or differentiate adults without hearing their voices across the room. Raymond is TOTALLY colorblind and sees things only in shades of gray- meaning lights and darks.

Dr. Lin's CIRM proposal offers hope to patients like my son, by testing new treatments to prevent vision loss using stem cell models derived from our patients with retinal disease. As both the President of Achroma Corp. and the mother of someone with achromatopsia, I enthusiastically support Dr. Lin's research to help people with inheritable retinal diseases throughout the world.

Yours Sincerely,

Bridget Vissari

Bridget Vissari
President, Achroma Corp.