From: Mark Weiss <mark_weiss@mindspring.com>
Date: Saturday, May 25, 2024 at 11:40 PM
To: Claudette Mandac <cmandac@cirm.ca.gov>, Scott Tocher <stocher@cirm.ca.gov>
Subject: [EXT] Letter of Support for CIRM Grant #TRAN1-16026

a. May 25, 2024

Dear members of the Applications Review Subcommittee'

We are the parents of Max Weiss, who has Gaucher's disease. We write today to passionately urge you to fully support Dr. Gomez-Ospina's groundbreaking project to investigate how gene therapy might cure Gaucher Disease in Max and all other people in the world. She is on the last lap of a cure! With your support, she has already cured Gaucher in mice, and with your financial help, will demonstrate that her methodology does the same in humans! Don't stop supporting her now!

We have lived with some level of dread about Gaucher since before our son Max was born. He was prenatally diagnosed, and some medical advice was to end the pregnancy. Given our desire for a child, our advanced ages, and that the pregnancy was a miracle of IVF very unlikely to be repeated, we embraced the pregnancy. Max is the cherished blessing we yearned for, and he has brought immeasurable joy into our lives.

Even after we understood that ERT therapy would give Max a semi-normal life, we were afraid that his body might not be able to tolerate the ERT enzyme, or worse, that he will not be able to obtain the enzyme after he can't be on our health insurance. In fact, it was only after we were told that Israel provides the enzyme free to all, did we make the choice of life. Now, we sadly imagine a future without this backup. We still worry that Max will always be able to obtain health insurance that covers the huge annual enzyme cost, and we know what happens with untreated disease. (Last year our high-quality medical insurance did refuse to pay for the infusions in a medical setting. Although we successfully appealed, we approach each insurance renewal date with fear.) Dr. Gomez-Ospina's breakthrough genome-editing therapy is the hope we cling to.

Max's Gaucher disease has, and continues to have, a negative impact on our family. Infusions are a lifetime commitment. Max's reproductive choices and where he lives will be constrained. We worry that Max will continue to tolerate the enzyme replacement. We limit our vacation durations. We feel limited in where we can live and have adequate access to advanced medical facilities. We worry that his port, already replaced once because it malfunctioned, will cease to be an

option. We worry that a new infectious disease, more dangerous and contagious than COVID, will interfere with his infusions. Our lives are constrained by his infusion schedule. We dislike providing Big Pharma with exorbitant payments forever.

Dr. Gomez-Ospina has personally discussed her project with us on any number of occasions, and we have contributed Max's cord blood to support her research efforts. We hope that this donation will aid her in studying cell behavior and ensuring their safety in the pursuit of a cure.

We share this personal journey with you not only to convey the challenges we've faced with this diagnosis but also to underscore the lifelong burdens of infusion-based treatment. It is with a fervent hope that children like Max will have improved treatment options in the future that we urge the California Institute for Regenerative Medicine to continue its steadfast support for Dr. Gomez-Ospina's mission to find a cure.

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