CIRM

CALIFORNIA'S STEM CELL AGENCY

MEMORANDUM

TO: CIRM GOVERNING BOARD
FROM: SENATOR ART TORRES (RET.)
SUBJECT: Memo re. SB 247 (Creation of Rare Disease Advisory Council)
DATE: JUNE 18, 2021

A rare disease is one that affects fewer than 200,000 people in the US. There are currently more than 7,000 diseases that are listed as rare, affecting the lives of almost 30 million Americans. Often the individuals and families affected by a rare disease are spread across the US, making it hard for them to access resources or even find a physician knowledgeable about the condition. Because of that families face many obstacles such as delayed diagnosis, misdiagnosis, lack of treatment options and high out-of-pocket costs where treatments are available.

It is not just hard for families, it is also challenging for state legislators, policymakers and government officials to have a good understanding of these conditions and the communities affected by them, and to help address the needs of these communities. To help change that the National Organization for Rare Diseases (NORD) wants to create a Rare Disease Advisory Council (RDAC) to provide the rare community with a voice in state government.

SB247 would create an RDAC in California. SB247 has already passed the Senate and is now on its way to the Assembly. If approved, it would give rare disease patients a unified voice in California state government. The RDAC will act as the advisory body on rare diseases to the Legislature and state departments that provide services to, or that are charged with the care of, rare disease patients. It will also be tasked with adopting and implementing regulations, researching and determining the most appropriate method to collect data on rare diseases, and identifying best practices for rare disease care.

The RDAC would allow the rare disease community to communicate directly with lawmakers and other stakeholders interested in identifying and solving pressing challenges. It would also help the state by quickly delivering direct feedback, solutions, and resources to government officials with one community voice.

In creating this council, California would join seventeen other states that have already enacted similar legislation in support of their rare disease community. Those states include: Alabama, Connecticut, Illinois, Kentucky, Massachusetts, Minnesota, Missouri, New Hampshire, New York, Nevada, North Carolina, Ohio, Pennsylvania, Tennessee, Utah, Virginia, and West Virginia.

CIRM already funds research involving many rare diseases including severe combined immunodeficiency, retinitis pigmentosa, sickle cell disease and cystinosis. We have seen great progress made in these areas, but there is more work to be done, and the RDAC could be an important voice in ensuring the needs of the rare disease community are served.

Board Request:

Endorse adoption of SB247.