CALIFORNIA'S STEM CELL AGENCY

MEMORANDUM

TO: CIRM SCIENCE SUBCOMMITTEE OF THE ICOC
FROM: CIRM LEADERSHIP
SUBJECT: Consideration of Funding Policy Regarding "n of 1" Proposals
DATE: JANUARY 25, 2024

At the November 28, 2023, meeting of the Governing Board's Application Review Subcommittee (ARS), Board Members considered Grants Working Group recommendations for a CLIN2 grant application involving an "n of 1" clinical trial. An "n of 1" clinical trial is an FDA-regulated trial that tests an individualized therapy for a single patient, for severely debilitating or life-threatening (SDLT) genetic disease. In their programmatic review, the ARS considered whether "n of 1" clinical trials should fall within the scope of CIRM's funding mandate given that the immediate impact of such a study is limited to one patient and the potential for further development of the given drug product for a broader set of patients is not anticipated.[1] The ARS also raised the concern that CIRM could be faced with supporting a large number of individualized trials with no clear benefit to the broader California population

The ARS requested that the Science Subcommittee carefully consider this issue, including the potential benefit to California of supporting these trials, and develop a programmatic recommendation for consideration by the full board on the eligibility of "n of 1" clinical trial projects for CIRM funding. The ARS deferred a final funding decision on the CLIN2 application pending the subcommittee and full board's decision**. At their January 17, 2024, meeting, the ARS *recommended to pause accepting n of 1 grant applications until a CIRM rare diseases strategy is drafted, reviewed, and recommended by the Science Subcommittee to the ICOC in the coming months.***

[1] Per FDA guidance, in these situations, drug development targeted to a larger number of patients with the same disease is not anticipated because of the specificity of the mechanism of action of the ASO combined with the rarity of the treatment-amenable patient population.

The policy discussion of the programmatic eligibility of “n of 1” clinical trials at the January 25 ICOC meeting will not entail an evaluation of the merits of the particular application that gave rise to this item. Based on the guidance of this committee and the final decision of the full board regarding “n of 1” clinical trials, the ARS will resume consideration of the specific CLIN2 grant application consistent with the board’s determination.