

Dear ICOC Board Members,

On behalf of patients in California and elsewhere, we thank you for your services in the effort to establish cell and gene therapies (CGT) as a safe and promising treatment modality when no other options are available. We also want to thank you for your attention to the concerns that we raised during the Jan 29th ICOC Governing Board meeting regarding the current implementation of the preferences-triaging process for funding applications to CIRM.

We are very encouraged by the willingness expressed by both the Board and the CIRM staff to consider alternative methods of achieving CIRM's mission, as well as further discussions on the possible adjustments to the current review process during the Mar 5th Science Subcommittee meeting. We are writing to voice support for the ongoing effort to ensure the highest level of scientific rigor in CIRM's review process.

We acknowledge and appreciate CIRM's need to manage the application volume. Relying on scientific merit as the key determinant for CIRM awards has been the cornerstone that enables CIRM's breakthrough impacts in the field of cell and gene therapy over the past two decades. We respectfully request the Board to consider the following to ensure that any changes in CIRM's review process retain or exceed that level of scientific rigor:

1. We agree that addressing "Access and Affordability" by CIRM applications should be an important review criterion. We also fully agree with the points raised by Drs. Levitt, Yamamoto, and Carson during the CIRM Science Subcommittee meeting on March 5th. Specifically, CIRM should provide clear instructions and/or examples of what they expect academic applicants to do to meet CIRM's expectation for "Access and Affordability". If "affordability" does not mean "cheap," then that should be clearly stated so that it is clear to both reviewers and applicants. In addition, as clinical efficacy makes treatments affordable over time through manufacturing improvements, the assessment of affordability should also consider the benefit of the proposed therapy.
2. We applaud the proposed adjustments to incorporate the CIRM GWG members as part of CIRM's application volume management strategy. We are still concerned about the consequence of having too few applications selected for full review, which could yield similar results noted by board member Dr. Shannon Dahl in the ICOC Jan 29th meeting—i.e., the GWG were given so few grants to review that they did not find enough scientifically meritorious CLIN2 proposals in Cycle 1 to recommend funding. Consequently, one out of four funded CLIN2 proposals had a score of 80—well below the GWG-recommended funding score. The revised plan discussed during the March 5 meeting included a "positive selection" process that

would eliminate approximately 2/3 of all applications and would fund close to 50% of those selected for full panel merit review. We believe this process needs to be thoroughly vetted prior to implementation to (a) minimize premature elimination of scientifically meritorious applications and (b) ensure that all applications selected for full panel merit review are in fact meritorious. For the special case of the CLIN2 program, if the number of applications continues to be under 25, we strongly recommend that CIRM offer full review of all applications without triage. This will apply the gold standard in scientific review for applications that are in the most critical clinical research phase of developing new life-saving treatments for patients.

We believe implementation of the above recommendations will enable CIRM to encourage applications with bold ideas supported by strong science.

Thank you again for your attention.

Sincerely,



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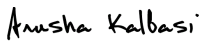
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