

**Summary of Response to ICOC Request for Additional Analysis
Regarding Application RT2-01985**

The reviewers considered the application, the letter of extraordinary petition from the PI, the review summary, and the recent publication in *Nature* by a named collaborator for the proposed research.

The reviewers found the points raised by the PI compelling, and recommend further consideration by the ICOC. The PI and his colleagues propose to study a childhood disorder that can be life threatening. The concerns of the reviewers have been addressed fully and certain misperceptions have been clarified.

The reviewers agreed with the applicant that the investigation addresses a substantial unmet need. Most children who are born with these intestinal malabsorption disorders have few if any therapeutic options with the possible exception of chronic home intravenous nutrition or organ transplantation. In support of the issue of need, the investigators have received IRB approval and the enrollment of subjects has proceeded with progress reported in collecting fibroblasts for the generation of intestinal cells and tissue.

In summary, reviewers observe that a well-respected, world-renowned clinical investigator has assembled a unique team of highly qualified scientists to investigate childhood disorders that can be life threatening and that have a substantive affect of the quality of life. They believe the project merits CIRM funding at the level requested.