



TO: Chairman Thomas and Members of the ICOC

FROM: Patricia Olson, Ph.D., Vice President – Discovery & Translation

DATE: December 6, 2017

RE: Amendments to DISC and TRAN Concept Plans

### **Background**

The Board approved concept plans for CIRM's Discovery ("DISC") and Translation ("TRAN") programs on July 23, 2015, and additional amendments on September 24, 2015. In December of 2016, the Board amended the DISC and TRAN concepts plans were amended to limit eligibility to California organizations. For the reasons below, the CIRM team proposes reopening the TRAN program to non-California organizations. Additionally, the team recommends reducing the award cap for DISC2- Quest<sup>1</sup> programs.

### **Proposed Amendments**

#### **1. TRAN Awards – Eligibility of Non-California Organizations**

Under the current concept plan for the TRAN program, non-California organizations are not permitted to apply for funding. A non-California organization is defined as an organization that pays and employs 50% or less of its employees in California. Prior to 2017, non-California organizations were permitted to apply. This initial policy was based on CIRM's clinical research ("CLIN") program, which was designed to ensure that CIRM had the ability to attract the best research clinical programs to California. Based on experience during the 2016 research year, the first year of the 2.0 TRAN program, the demand and quality of projects seeking funding exceeded CIRM's budget. At the time, given the quality and volume of pre-clinical work being conducted at California institutions, permitting non-California organizations to apply for funding for work conducted in California was not necessary to achieve CIRM's aims. As a result, the TRAN concept plan was amended for 2017 to limit eligibility to California organizations.

While 2016 saw a high demand for quality projects within California, 2017 saw a 33% drop in applications and 50% fewer applications funded compared to 2016. While CIRM budgeted for approximately 9-10 TRAN awards in 2017, only 6 projects qualified for funding. To increase the pool of quality of awards and to tap external demand that will enable CIRM to reach its strategic goals, the team proposes the TRAN concept plan be reopened to non-California organizations. It is important to note that while non-California organizations could apply for a TRAN award, only the cost of research activities

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<sup>1</sup> The goal of a Quest Award is to produce, within 2 years, a project deliverable that is a novel candidate therapeutic, device, diagnostic test or tool that can immediately progress to translation to enable broad use.

conducted wholly in California and the share of costs for research activities conducted outside of California that are directly required to support research conducted in California would be paid to such an organization.

## 2. DISC2 Quest Awards – Award Budget Cap Revision

To accomplish CIRM's goal of accelerating and advancing the discovery of novel stem cell treatments, the agency established three types of Discovery Stage Programs: 1) DISC 1 – Inception Awards; 2) DISC 2 – Quest Awards; and 3) DISC 3 – Challenge Awards.

The objective of the DISC-2 funding opportunity is candidate discovery, specifically to promote the discovery of promising new stem cell-based technologies that could be translated to enable broad use and ultimately, improve patient care. Projects funded through the Quest Awards should propose technology that is uniquely enabled by human stem/progenitor cells or directly reprogrammed cells, or uniquely enabling for the advancement of stem cell-based therapies. Currently, a successful Quest applicant is eligible for direct project costs of up to \$700,000 to achieve a candidate that is a diagnostic, device or a tool; \$1.4 million is available if the candidate is a therapeutic. The rationale for the distinction between the types of awards is that it is generally more expensive to achieve a therapeutic candidate than it is to achieve a device, diagnostic or tool prototype candidate.

To efficiently manage the resources allocated for the Discovery programs while ensuring that the development pipeline remains vital, the team proposes a reduction of the above caps as follows: \$900,000 for direct costs for therapeutic candidate discovery and \$500,000 for direct costs for medical device, diagnostic and tool candidate discovery.

**Requested Action: CIRM requests the Board approve the proposed amendments to the TRAN and DISC concept plans.**

Attachments