Dec. 17, 2015 CALIFORNIA'S STEM CELL AGENCY TRANSLATIONAL CLINICAL DISCOVERY INFRASTRUCTURE DEDUCATION Accelerating Therapies PTP3 through public private partnership Maria T. Millan, M.D.

Presentation to CIRM's ICOC

Background: Lack of Industry "Pull"

- CIRM has invested \$2 billion in developing a portfolio of over 300 technologies
- Programs are rapidly moving through preclinical and clinical development
- Industry is beginning to show interest but only 6% of CIRM's academic projects have been licensed by industry (1)
- Out of 3,400 technologies currently marketed by the UC System,
 <2% are stem cell programs (2)



ATP3 Objectives

Accelerating Therapies through Public-Private Partnership

Engage Industry by:

- Creating an opportunity to form an entity that would aggregate CIRM's most promising technologies
 - Offers "multiple shots on goal" and increases the probability of success
 - Makes significant industry investment more attractive
- Leveraging CIRM's administrative and review infrastructure and its external team of world-class subject matter experts to promote access and informed insight into CIRM's portfolio of stem cell projects
- Continued CIRM funding for the development of in-licensed CIRM technologies



Accelerating Therapies through Public-Private Partnership (ATP3)- Concept

- The private partner will in-license, develop and drive toward commercialization an aggregated portfolio of non-partnered CIRM projects
- CIRM will enable the identification and assembly of the most promising technologies and will fund continued development of these projects



Intended Outcomes

- For Researchers continued funding for the advancement of their CIRM project
- For Universities create demand for out-licensing CIRM-funded technologies; greater opportunity for financial return by decreasing risk through aggregation
- For the Citizens of California create an industrial stem cell therapeutic powerhouse that increases the likelihood of the commercialization of stem cell treatments for patients with unmet medical needs



Private Partner (ATP3 Awardee)

- Could be an established company, a spin-off, or a new company with a team formed by Pharma, biotechnology, or by an investor
- Must have an exceptional business plan that describes the synergies, value creation and financial return to stakeholders they expect to create through their technology aggregation strategy
- Must propose a top-tier leadership team with the skill set necessary to successfully execute the business plan
- Will be required to commit significant upfront investment capital necessary to execute on this business plan
- Will have access to CIRM funds to support development costs for inlicensed CIRM programs

Award Information

- CIRM will award up to \$75M of funding over a period of 5 years to continue the development of in-licensed CIRM projects, subject to GWG approval on a per project basis
- The awardee will be required to make an upfront capital investment equal to the total CIRM award
- Subject to pricing, access, and march-in provisions of CIRM's IP regulations
- Required to provide licensors of CIRM projects with a right of first refusal for the return of the CIRM-funded technology if the applicant decides not to develop it



CIRM ICOC Motion

CIRM requests that the Board approve the concept plan for ATP3, with a budget authorization of up to \$75 million to fund an award over 5 years, and delegate authority to the Science and the Intellectual Property and Industry Subcommittees to approve the award terms (e.g. loan) at a joint meeting.

