



TO: Members, Governing Board, CIRM
FROM: C. Scott Tocher, Deputy General Counsel
DATE: September 15, 2016
RE: ATP3 Review Steps

Executive Summary

The Grants Working Group (“GWG”) will meet in the first quarter of 2017 to evaluate applications for the “Accelerating Therapies: Public-Private Partnership” program, known as ATP3. The GWG and Application Review Subcommittee’s review of applications for the ATP3 award and subsequent in-licensed projects will occur in two steps. The first review will identify and select a single awardee, referred to as “Newco.” Then, we will engage in a separate review or reviews to evaluate projects identified by Newco as ones to in-license. The level of GWG review of the proposed in-licensed projects will depend on the status of the projects, as described below.

I. Background: ATP3 Program – Unique Characteristics

In December 2015, the Board approved the concept plan for ATP3. Unlike many of CIRM’s research programs, ATP3 is a hybrid infrastructure/research program designed to push the CIRM research portfolio through preclinical development into clinical trials and ultimately on to commercialization to benefit patients and California’s taxpayers (through the revenue sharing obligations of the licensors of CIRM-funded projects). To do so, the program will fund a public-private partnership to advance high-quality CIRM-funded stem cell technologies through a stem-cell focused entity based in California. As the public partner, CIRM will provide funding to the industry partner for continued development and commercialization of an aggregated group of existing CIRM projects. CIRM, by leveraging its review and administrative infrastructure, will also provide valuable insight and rigor to the process by which the Industry partner chooses its aggregated portfolio. In this way, the focus of the ATP3 program is two-fold: 1) identifying the best management team with the most promising strategy to succeed in commercial stem cell therapy development; and 2) providing the infrastructure to review and administer the in-licensing of CIRM projects by the entity. Because the identification and vetting of ATP3 candidates will entail

different criteria from the scientific consideration of projects to be in-licensed, CIRM proposes a two-step review process for the ATP3 program.

II. The Review Process

Step 1 – Identifying Newco – Making the Award

The GWG will convene to review applications for the ATP3 award and make funding recommendations to the Application Review Subcommittee, which will choose a single awardee. The GWG will evaluate whether a given applicant sets forth the following:

- An exceptional business plan that includes a(n):
 - Aggregation Strategy: a proposal outlining the strategy and scientific rationale for the types of technologies (e.g., the disease indications targeted by the applicant) or technology platforms (e.g., IPS cells, human embryonic stem cells, gene modified pluripotent or progenitor cells, small molecules or a combination of some sort) the applicant intends to in-license;
 - Operational Plan: a description of how the applicant intends to develop and commercialize these technologies; and
 - Value Proposition: unique synergies and benefits the applicant intends to realize through technology aggregation that would result in a world-class cell therapy company.
- A world class management team; and
- A significant up-front capital commitment to establish the business.

The review will focus on whether the applicant is likely to accelerate CIRM-funded technologies toward commercialization, and the plausibility of the scientific approach underlying its business strategy. Issues to consider at stage are whether there is a viable business plan to achieve the objectives of the RFA and whether the strategy for in-licensing CIRM-funded technology creates a compelling value proposition. The successful applicant must demonstrate affirmatively that the business plan is likely to create value for patients, the citizens of the State of California as envisioned by Prop. 71, and shareholders, and will support growth of the business beyond the five-year award period.

The review will also focus on whether the management team has the experience and qualifications to execute on the business plan. Considerations include whether:

- the organizational structure is appropriate to carry out the

- business plan and achieve the objectives of the RFA;
- the team is likely to successfully in-license CIRM-funded technologies; the team be able to raise capital from sources other than CIRM;
- there is a good probability that the Company will be able to in-license the technologies within the proposed timelines;
- there is a good probability that the Company will be able to be successful in accelerating the development of CIRM-funded technologies toward commercialization; and
- the business plan appropriately accounts for project risks and mitigation strategies.

Step 2 – In-Licensed Projects Review

After the Application Review Subcommittee of the Board has made the award to Newco, Newco will be required to expeditiously identify specific CIRM projects to in-license. Development milestones will be agreed to in the Research and Financing Agreement to ensure that Newco adheres to appropriate timelines for in-licensing projects. All proposed projects for in-licensing must undergo a review by the GWG, and the Application Review Subcommittee, as follows:

1. Bucket 1 – Active New Projects: Active CIRM-funded projects that have been approved by the Application Review Subcommittee within the preceding 12 months will not require a new GWG review unless CIRM, in its sole discretion, determines that a review is warranted based on the status of the project, in which case the project will be subject to a good-standing review, described in paragraph 2.
2. Bucket 2 – Active Mature Projects: Active CIRM-funded projects approved by the Board more than 12 months earlier will require a new GWG review, limited to the question of whether the project is in good standing, e.g., has met or is on target to meet milestones, or if the project has not met milestones, has a viable path to accomplish them.
3. Bucket 3 – Non-CIRM or Inactive Projects: These projects will be subject to a full GWG CLIN-style review if Newco wants to use CIRM ATP3 funds – limited to \$75m.

Requested Action: CIRM requests the Board approve this policy for the review of ATP3 applications and subsequent in-licensing of projects.