Right now.

Every Moment Counts.

Don't Stop Now.

Jennifer Mielnicki Lewis
Director, Grants and Operations

Doug Kearney
Associate Director, Grants Management

Implementing a strategic plan to accelerate outcomes:
An organization case-study

2019 SRAI Annual Meeting
October 19-23

Every Moment Counts. Don't Stop Now.
On The Agenda

1. CIRM’s Strategic Planning Process

2. Grants Management’s Contribution
   • Milestone-based funding
   • Eliminating non-value added policies
   • Developing reports/tools

3. Lessons Learned
CIRM’s Mission

**Accelerate** stem cell treatments to patients with unmet medical needs.
California’s Stem Cell Agency

2004
CIRM created by Patient Advocates and California Stakeholders- Proposition 71

$3B
Committed to CIRM Mission

1000
Cutting Edge Research & Transformative Programs funded

56 Clinical Trials
First in human, cell & gene medicine, some ready for final marketing approval

>1200 Patients
Patients enrolled in Alpha Clinics Clinical Trials
The Strategic Plan Process

Three simple questions that yielded three valuable outcomes

Where are we now?

Where do we want to go?

How do we get there?

Situational Awareness

Measurable Goals

Organizational Clarity
Mission Confirmation

When asked, Board members and Stakeholders agree on CIRM’s Mission

*Accelerate stem cell treatments to patients with unmet medical needs.*

Agreed

100% Board Members

95.4% Stakeholders
Push, Pull, Level

Anything in the way of our mission is fair game
A Vision for Accelerating Success

All parts of CIRM must work in concert to get more treatments to patients faster and with greater success.

Volume • Speed • Quality
Eliminate Grant Whac-A-Mole
Objective Measures of Success

**MISSION**
Accelerate stem cell treatments to patients with unmet medical needs.

**2020 VISION**
- **DISCOVER**: 50 NEW CANDIDATES INTO DEVELOPMENT
- **ADVANCE**: 50% MORE INCREASE PROGRESSION EVENTS
- **ENACT**: 50% LESS ENACT NEW REGULATORY PARADIGM
- **ACCELERATE**: REDUCE TRANSLATION TIME
- **PARTNER**: 50% CLINICAL PROGRAMS WITH COMMERCIAL PARTNERS
- **VALIDATE**: 50+ NEW CLINICAL TRIALS

**CIRM**
CALIFORNIA’S STEM CELL AGENCY

*Every Moment Counts. Don’t Stop Now.*
Four Key System Changes

The improvements having the biggest impact on operational excellence

- Standardize Recurring Program Offerings
- Increase Speed and Cycle Frequency
- Implement Milestone Based Disbursements
- Establish Objective Goals and Metrics
Frequent and Standardized Offerings

**DISCOVERY**
- New Idea
- 2/Year

**TRANSLATION**
- Single Product Candidate
- 3/Year

**CLINICAL**
- Pre-IND Meeting or Equivalent
- Approved Therapy
- 12/Year

Program Offerings Per Year
Benefits of Frequent Repetition

Volume, speed, quality and productivity improved

- **4X** Review Cycles Per Year
- **96%** Applications Reviewed
- **75%** Number of Awards
- **57%** Cost Per Application
- **82%** Time to Approval

Volume • Speed • Quality
How Did Grants Management Participate?

✔ Leadership open to feedback
✔ Honest and vocal about current issues and their potential solutions
✔ Commitment to “Accelerate”
✔ Design and manage IT system changes
What We Did

✓ Milestone-based funding
✓ Eliminating non-value add policies
✓ Develop Reports/Tools
Operational Milestones

Previous Reporting & Payment Schedules

The first disbursement made at award launch and subsequent payments made on a time based schedule.
Operational Milestones

Disbursements upon achievement

Operational milestones are set on objective measures of progress

Milestone #1 Target
Example: 33% Enrolled

Milestone #2 Target
Example: 66% Enrolled

Milestone #3 Target
Example: 100% Enrolled

Every Moment Counts. Don't Stop Now.
Operational Milestones

Disbursements upon achievement

The first disbursement is made at award launch and should be sufficient to achieve the first milestone

Milestone #1 Target
Example: 33% Enrolled

Milestone #2 Target
Example: 66% Enrolled

Milestone #3 Target
Example: 100% Enrolled

Award Launch
First Tranche Delivered

Board Approval
$3M

$1M
Operational Milestones

Disbursements upon achievement

Subsequent disbursements are made upon achieving the milestones

- Award Launch
- Board Approval
- First Tranche Delivered
- Second Tranche Delivered
- Third Tranche Delivered
- Milestone #1 Reached
- Milestone #2 Reached
- Milestone #3 Reached
- Award End
Operational Milestones

Disbursements upon achievement

A delay in achieving a milestone results in disbursements being suspended until the milestone is reached.

- Board Approval
- Award Launch
- First Tranche Delivered
- Milestone #1 Delayed
  Second Tranche Withheld Until Milestone is Met
- Milestone #2 Target
- Milestone #3 Award End

Any shortfall must be covered by the awardees contingency funding.

$1M
Operational Milestones

Disbursements upon achievement

If a milestone is reached early, any savings is carried forward

Milestone #1 Reached Early
Award Launch
First Tranche Delivered
Savings Carried Forward

Milestone #2 Target
Second Tranche Delivered

Milestone #3 Award End

Any excess at award end can be used to further the project or similar research.
Operational Milestones

Funding Partners

Any co-funding requirement that is continuous throughout the award must be met at each milestone

Board Approval

Award Launch

Milestone #1 Reached

Milestone #2 Reached

Milestone #3
Award End

Every Moment Counts. Don't Stop Now.
Operational Milestones

The Results

- Milestone #1 Reached
  - First Tranche Delivered
  - Second Tranche Delivered
  - Third Tranche Delivered
- Milestone #2 Reached
  - Award End
- Milestone #3 Reached
  - Award Launch

Milestones Hit On-Time

UP 4X
19% vs 79%
Eliminating Non-Value Add Policies

MISSION ALIGNMENT

ACCELERATE THE PROCESS

ELIMINATED

WHAT’S THE PURPOSE?

BUSINESS VALUE

PROCESS TIME
Prior Approval Requests

Eliminating non-value add policies

✓ Processed 320 requests per year
✓ 85% for rebudgeting, carryforward and extensions
✓ 98.9% approved
✓ 66% were “retroactive” requests (not seeking prior approval)

Decision: remove these types of prior approval requirements
Protocol Approvals

Eliminating non-value add policies

- Federal Requirement
- Administrative heavy process
- Compliance vs. Risk

Decision: accelerate the process through self-certification
Reports & Tools

Building a measurement system

✓ Streamlined Reporting
✓ Award Burn Rate vs. Milestones
✓ Progress Report Checklist
✓ Turnaround
# Reports & Dashboards

## Streamlined Progress Reporting

### Progress on Individual Aims/Milestones

<table>
<thead>
<tr>
<th>Aim/Milestone 1</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Description of Aim/Milestone</strong></td>
</tr>
<tr>
<td>M1: Enroll and transplant first patient in first cohort of 3 patients</td>
</tr>
<tr>
<td><strong>Completion Dates</strong></td>
</tr>
<tr>
<td>Estimated: Oct 31, 2018</td>
</tr>
<tr>
<td>Actual: Oct 30, 2017</td>
</tr>
<tr>
<td><strong>Previous Aim/Milestone Progress</strong></td>
</tr>
</tbody>
</table>

During this reporting period we have made progress toward enrolling our first patient into cohort 1. We received 2 patient referrals during this reporting period. After reviewing the eligibility criteria, one patient was not eligible, and the other patient is currently in the screening process.

In addition to pursuing patient recruitment, we have implemented a media and communications campaign to educate providers and patients, locally, regionally, and nationally about the clinical trial. The campaign consists of media interviews, newsletter publications, distribution of provider and patient brochures to healthcare facilities and at healthcare events, establishing a social media presence on Facebook, Twitter, and Youtube, and creating patient-centered videos.

Moreover, we are in the planning stages of a full-scale education and outreach campaign. Under the direction of our Outreach Coordinator, we are planning a long-term strategy to identify affected populations, by zip code, so we can provide direct education and outreach to the affected communities. In collaboration with community leaders and ambassadors, the education and outreach will be in the form of focus groups, small in-person interviews, and small community events.
### Reports & Dashboards

#### Streamlined Progress Reporting

**Aim/Milestone Progress in This Reporting Period**

**Aim/Milestone Status:** [ ] Completed  
**Actual date Aim/Milestone was achieved:** 10/30/2017

**Progress on Aim/Milestone**

Describe progress for this Aim/Milestone during this reporting period, including major findings and/or negative results. This narrative should include supportive figures and should be written in sufficient detail to document the efforts and outcomes of those activities.

If the Aim/Milestone was completed during this reporting period, describe in the context of the success criteria.

During this reporting period we successfully enrolled and transplanted our first patient in the first cohort. There are no major findings or negative results to report; however, the patient will be continually monitored for safety.

In addition to enrolling and transplanting the first patient, we are working in collaboration with LightBox Collaboration, a consulting firm, who will be helping with the planning, design, and implementation of our media and communications campaign. Moreover, we are in the process of sending abstracts and brochures to upcoming regional and national healthcare events, so that we can educate providers and patients about the clinical trial.

Lastly, we are in the process of recruiting an Education and Outreach Coordinator who will play an integral role in executing our education and outreach strategy. We are in communication with leadership at the California Genetic Disease Screening Program to obtain data that will identify affected populations by zip code. Once we obtain the data, and clean the data, we will be able to implement our strategy of providing direct education and outreach to affected communities.

**Issues**

*Describe any issues that impede research progress and plans for resolution.*

Discuss any changes to this Aim/Milestone and provide justification (note that a PAR may be required for changes).

There are no issues that will impede research progress.

**Plans**

*Describe plans for the next reporting period.*

For the next reporting period, we will continue screening potential participants with the goal of enrolling and transplanting our second patient. In addition, we will continue to work on our media and communications campaign, and our education and outreach strategy.
Reports & Dashboards

Enhanced financial reporting

- Quantitative milestone measure
- Current funding is sufficient until?
- Track financial issues
- Discuss contingency plan with grantees
# Award Burn Rate vs Milestones

## Milestone Progress vs. Financial Burn

### Color Coding of Next Milestone
- Early, On Time, or BEHIND less than 30 days.
- BEHIND 30 to less than 120 days.
- BEHIND 120 days or more.

For projects with Operational Milestone reports days overdue is measured against the milestone's target date. For projects without Operational Milestone reports days overdue is measured against the due date of the first scheduled progress report after the milestone's target date that includes the milestone-progress section. If there is no such report, days overdue is measured against the milestone's target date.

### Color Coding of Burn
- Projected funds exhaustion at or after estimated completion of next milestone.
- Projected funds exhaustion BEFORE estimated completion of next milestone.

## CLIN Type Awards

<table>
<thead>
<tr>
<th>Project</th>
<th>Coding</th>
<th>Financial Progress</th>
<th>Next Milestone vs Burn Rate</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Blood and Cancer Therapeutic Area</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Disease Area Details: Hematologic</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Therapeutic Approach: Biologic</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>$6.9M</td>
<td>$3.1M</td>
<td>$2.4M</td>
<td>$2.4M</td>
</tr>
<tr>
<td>Funds Exhaustion: 4/23/2018 from rpt Feb 2018</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>M1: Pivit Non-Human Primata Tox Study...</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>M1: Next Payment: $3.8M</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Disease Area Details: Hematologic</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Therapeutic Approach: Cell Therapy: Allogeneic</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Stage: Clinical Trial, Phase 2</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>$6.9M</td>
<td>$2.4M</td>
<td>$7.8M</td>
<td>$2.8M</td>
</tr>
<tr>
<td>Funds Exhaustion: 3/31/2018 from rpt</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>M1: Manufacture and release 86% of...</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>M1: Next Payment: $1.8M</td>
<td></td>
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<td></td>
</tr>
</tbody>
</table>
## Progress Report Checklist Turnaround

<table>
<thead>
<tr>
<th>Responsible Officer</th>
<th>All Rpt so far this Year</th>
<th>Progress Rpt so far this Q</th>
<th>Financial Rpt so far this Q</th>
<th>All Rpt so far this Q</th>
<th>All Rpt 2017 Q4</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>% done within 30 days</td>
<td>avg turnaround days</td>
<td>n</td>
<td>% done within 30 days</td>
<td>avg turnaround days</td>
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<tr>
<td>Melnicki, Jennifer</td>
<td>100</td>
<td>8.0</td>
<td>16</td>
<td>100</td>
<td>9.9</td>
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<td>Thompson, Gabriel</td>
<td>75</td>
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<td>12</td>
<td>100</td>
<td>23.0</td>
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<tr>
<td>Wells, Ryan</td>
<td>100</td>
<td>3.8</td>
<td>15</td>
<td>100</td>
<td>6.5</td>
</tr>
<tr>
<td>All GMOs</td>
<td>95</td>
<td>11.1</td>
<td>64</td>
<td>100</td>
<td>11.7</td>
</tr>
<tr>
<td>All SOs and GMOs</td>
<td>95</td>
<td>11.7</td>
<td>125</td>
<td>96</td>
<td>12.1</td>
</tr>
</tbody>
</table>
Lessons Learned

✓ Adhere to Mission
✓ Adhere to Process
✓ If Process is Flawed, Fix it
✓ Ask Why, and “That’s the way we’ve always done it” is Not an Answer
✓ Rely on Objective Measures of Success
CURED

Evangelina Padilla-Vaccaro

Diagnosed at birth with Severe Combined Immunodeficiency

Brenden Whittaker

Diagnosed with Chronic Granulomatous Disease

Every Moment Counts