

Beyond
CIRM 2.0
CALIFORNIA'S STEM CELL AGENCY

now it's personal

President's Report

September 21, 2016



On Tap...



Things I will be covering today

- **The CIRM Mission**
- **Review of the Strategic Plan**
- **2.0 Performance**
- **Budget Review**
- **Clinical Program Review**

Our Mission

Accelerate stem cell treatments to patients with unmet medical needs.

CIRM's Strategic Plan



Objective Measures of Success

Beyond
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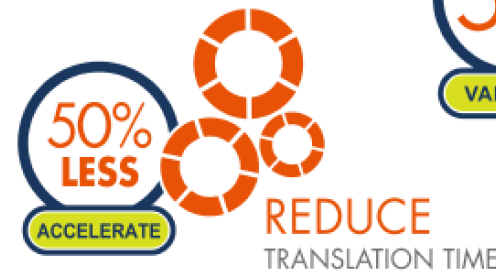
NEW
CANDIDATES
INTO DEVELOPMENT



INCREASE
PROGRESSION
EVENTS

CIRM 2.0
CALIFORNIA'S STEM CELL AGENCY

2020 VISION

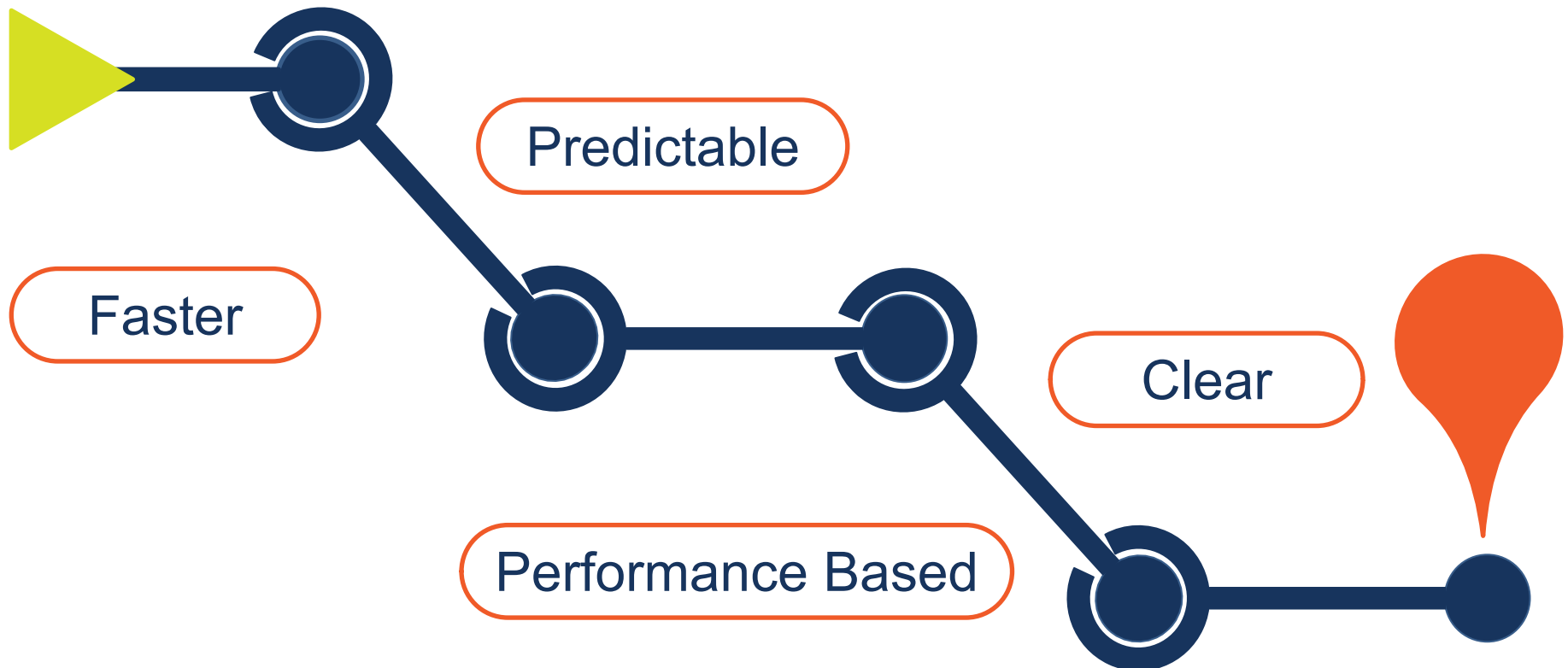


**CLINICAL
PROGRAMS**
WITH COMMERCIAL
PARTNERS



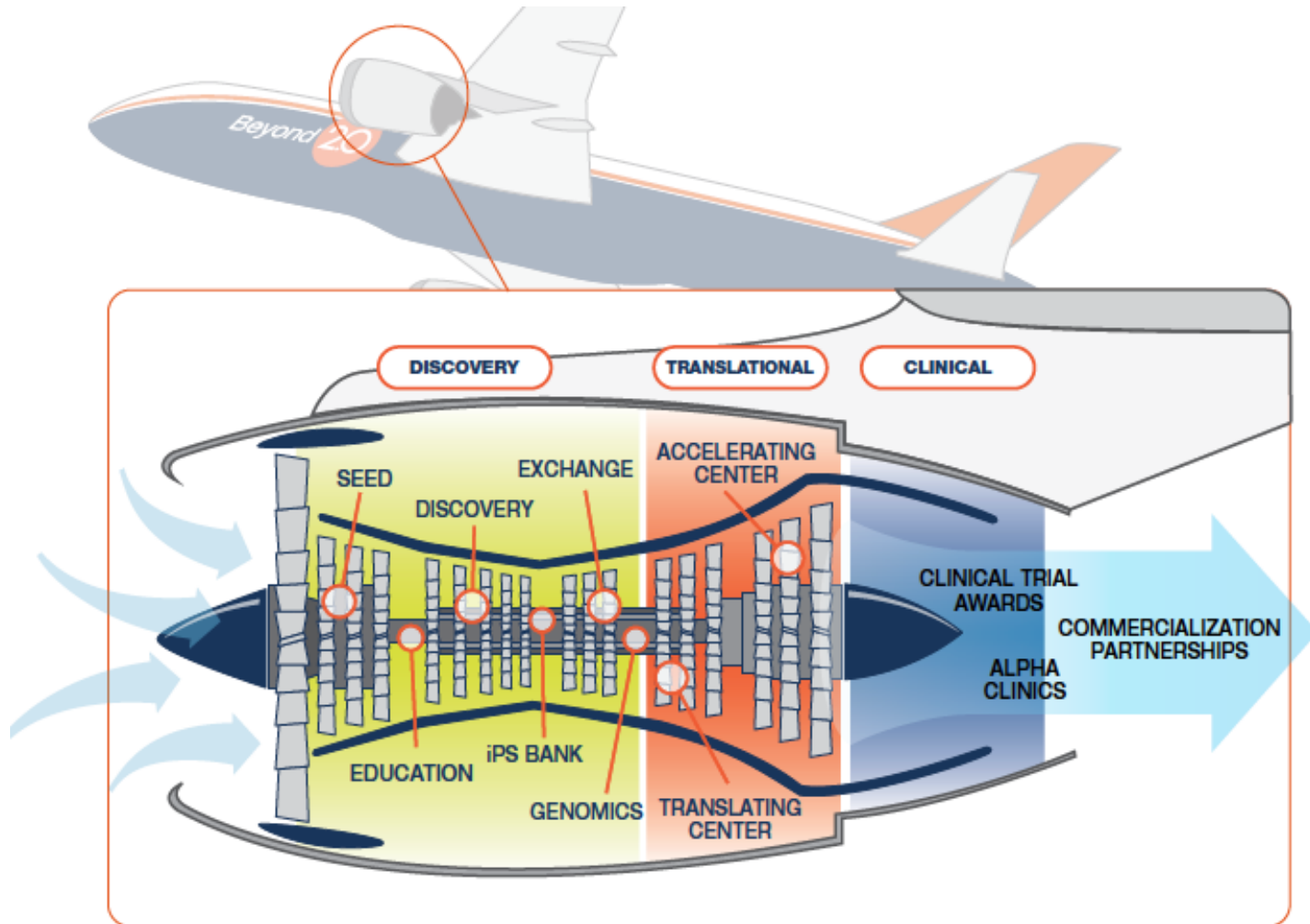
Overarching Themes

Create a process that drives better outcomes



Our Stem Cell Engine

Putting all of the parts together for maximum power



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The Core of the Engine



DISCOVERY



TRANSLATION



CLINICAL

New
Idea



Single Product
Candidate



Pre-IND Meeting
or Equivalent



Approved
Therapy



1-2/Year



3/Year



12/Year

Program Offerings Per Year

The Core of the Engine



DISCOVERY



TRANSLATION



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1-2/Year



3/Year



12/Year

Now All On-Line – Allows Better Budgeting

Funding by Program

Estimates for CY2016 vs original ICOC allocation



Low

\$37M ETF vs
\$53M allocated



High

\$52M ETF vs
\$40M allocated*



Increasing

\$80M ETF vs
\$100M allocated

ETF: Estimated to Finish for CY2016

* Additional funding allocated by ICOC

CIRM 2.0 Performance



Summary of Clinical stage applications to CIRM

- Total Clinical Applications Received: **54**
- Applications Passing Eligibility: **39** (79% - 7 pending)
- Applications with Final GWG Dispositions: **36**
- Applications Under Review: **3**
- Applications Recommended for Funding: **13** (36% of GWG adjudicated, 30% of applications received)

Takeaway: We will need approximately 150 more clinical trial applications to reach our goal

CIRM 2.0 Performance



Review and Approval has changed significantly

- Total number of reviews for 2016: **20**
 - Historical average of **5.8**
 - No personnel increase
- ICOC Approvals: **Now Monthly**
- Time from clinical application to approval: **<85 days**
- Time from clinical approval to contract: **<45 days**

CIRM 2.0 CORE

Progress Update



LEGAL

- Employee COI Policy
- Internal Governance Policy
- Board Bylaws
- Email Retention
- Contracting Agreement
- Contracting Policy

HUMAN RESOURCES

- On Boarding
- Compensation Policy
- Employee Handbook
- Telecommute Guidelines & Policy
- Professional Devel. Guidelines & Process

GRANTS MANAGMENT

- DTE GAP
- 2.0 Conversion Kit
- Operational Milestones
- Update GM SOP
- Big 6 Metrics Reporting

IT

- Desktop Support System
- Tracking Mechanism for Ongoing IT Projects

FINANCE

- Travel Policy & Procedures
- Business Expense Policy
- Forecasting
- Budgeting Process Improvements

APPLICATION AND REVIEW

- PA/RFA Development & Publication
- Application Development & Revisions
- Honoraria Payment/Reimbursement Process
- On Boarding New GWG Members

BOARD

- Update Board Training Binder Documents
- Board Member Terms

Accelerating Center: Driving stem cell clinical development from IND to market



Immediate benefits of engagement

- ✓ Prepare and optimize grant applications and budgets
- ✓ Prepare and file INDs
- ✓ Plan, design, and execute high quality clinical trials
- ✓ Discount pricing to CIRM grantees

Contract executed in just 65 days from ICOC decision

Open for business

Grand Opening Ceremony October 4th!

On Tap...

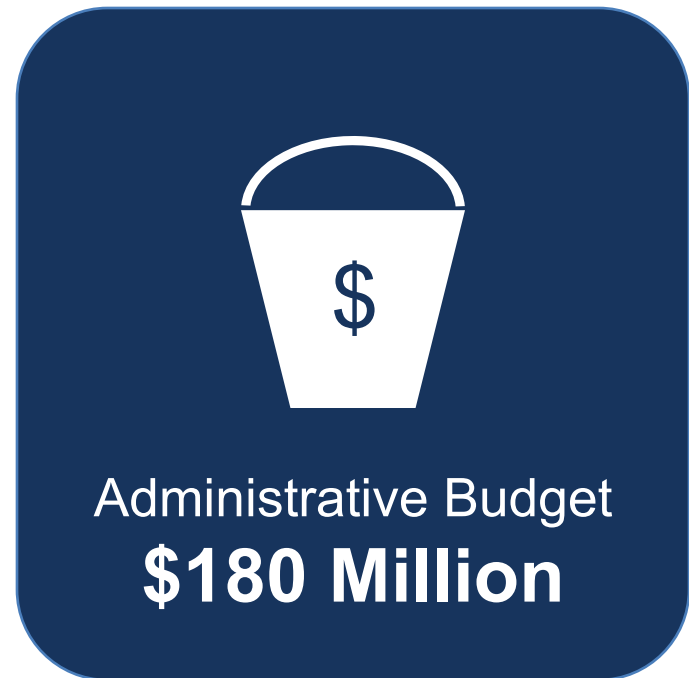


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The Buckets

Two separate funding sources



The Little Bucket

Administrative funding



Administrative Budget
\$180 Million

Funding available to mid 2020

- \$119 million spent
- \$61 million remaining
- Current spend rate of \$16 million per year
- Fiscal responsibility is very important

The Big Bucket

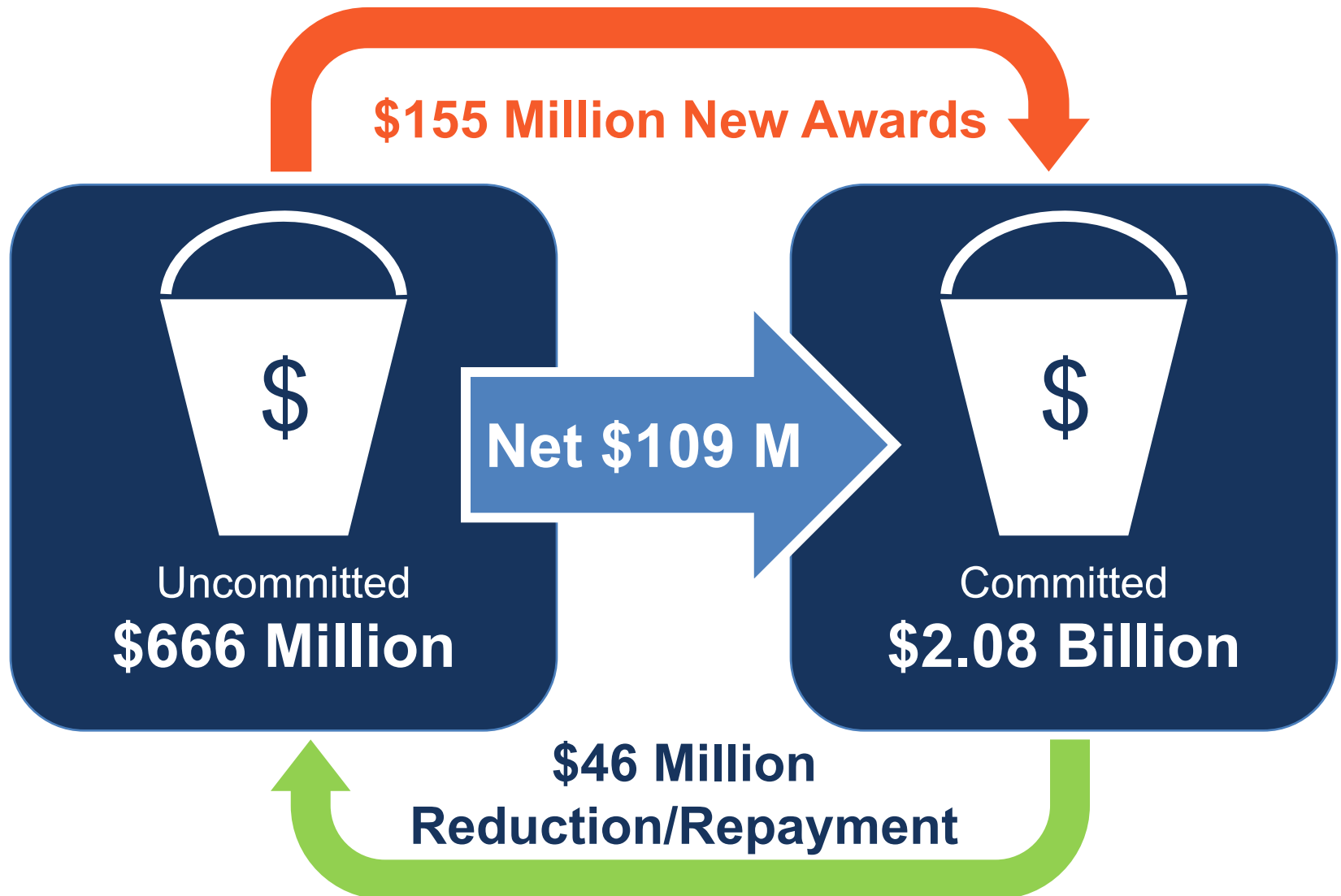
Award funding



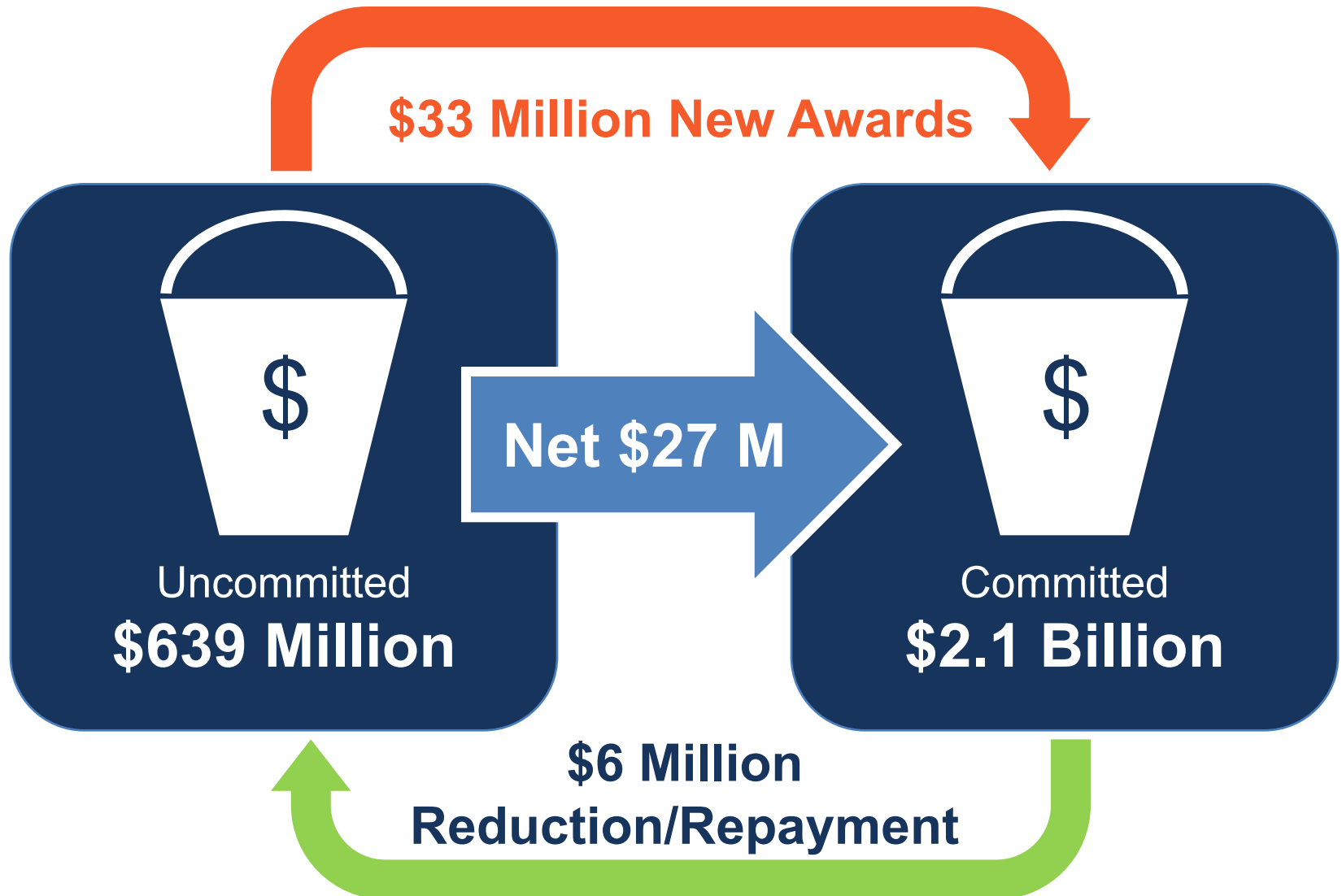
Estimated Depletion in FY 2020

- \$2.11 billion awarded or spent
- \$639 million uncommitted
- \$900 million currently under active award management
- Models assume a return rate of 3-5% of the “active” balance
 - Approximately \$40M annually

Award Activity Full Year 2016



Award Activity 1Q17(est.)



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The Clinical Programs



CLINICAL

Pre-IND
Meeting



Approved
Therapy

Availability
12/Year



All Meritorious Applications

CLINICAL 1 IND ENABLING

For the development of candidates from the Pre-IND meeting stage to filing a successful IND

Amount: \$5M
Target 18 months

Prerequisite: Pre-IND
Meeting
Next: CLINICAL 2



CLINICAL 2 CLINICAL TRIAL

For the conduct of any phase of a clinical trial

Amount: Up to \$20M
Duration: 60 months maximum

Prerequisite: Active IND
Next: CLINICAL 2
As needed



CLINICAL 3 ACCELERATING ACTIVITY

Supplemental funding for opportunistic activities that will accelerate the overall development of an active Clinical 1 or Clinical 2 candidate

Amount: As needed
Duration: As needed

Prerequisite: CLINICAL 1 or
CLINICAL 2
Next: None

Scope of Clinical Grants

Clinical stage grants cover Pre-IND through all stages of clinical trials



Stem cell or progenitor cell therapeutic candidates



Hematopoietic stem cells being developed in a novel way to address a rare condition or unmet medical need



Small molecules or biologics that target stem cells and are not likely to be funded from other sources

Applicants for Clinical stage programs can be either in or out of state

Co-Funding Requirements

Matching funds vary by phase of trial and type of institution

Applicant Type	Pre-Clinical	Phase 1	Phase 2	Phase 3	CLIN3
Non-Profit	None	None	40%	50%	Same as parent award
For-Profit*	20%	30%	40%	50%	Same as parent award

*Solvency must be demonstrated

Thank You!

