

Beyond
CIRM 2.0
CALIFORNIA'S STEM CELL AGENCY

now it's personal

President's Report
February 23, 2017



On Tap...



Things I will be covering today

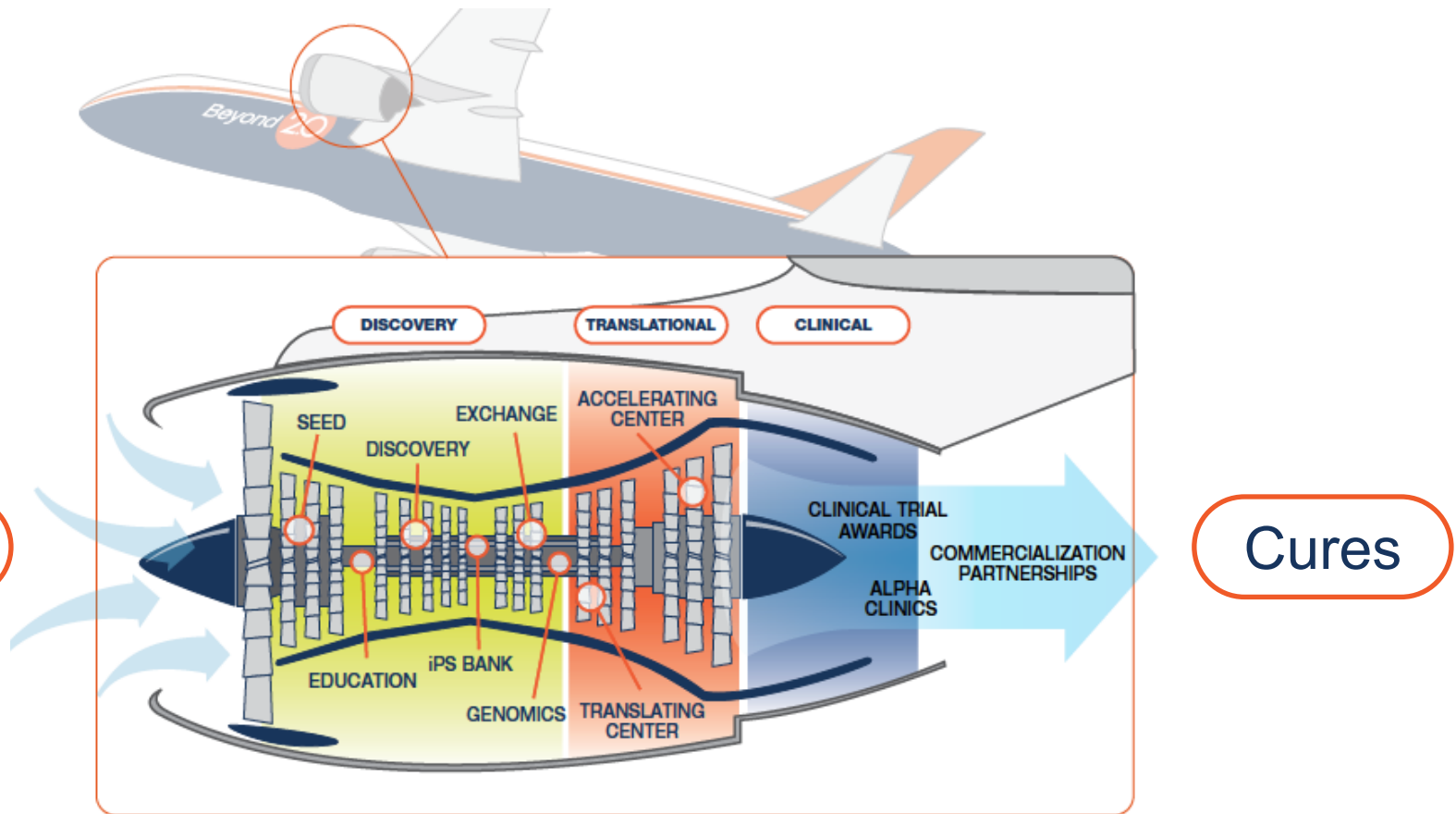
- **The CIRM Mission**
- **Review of the Strategic and Annual Plan**
- **2.0 Performance Review**
- **Budget Review**
- **Accelerating Center and Translating Center Update**

Our Mission

Accelerate stem cell
treatments to patients with
unmet medical needs

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

Objective Measures of Success

Beyond
CIRM 2.0
CALIFORNIA'S STEM CELL AGENCY
now it's personal



NEW
CANDIDATES
INTO DEVELOPMENT



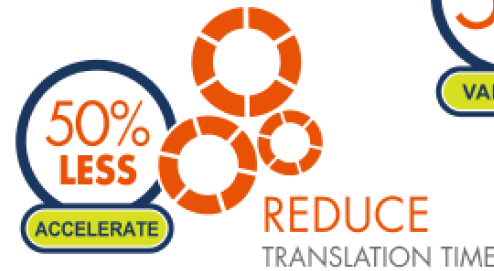
**CLINICAL
PROGRAMS**
WITH COMMERCIAL
PARTNERS



**INCREASE
PROGRESSION
EVENTS**



ENACT
NEW REGULATORY
PARADIGM



REDUCE
TRANSLATION TIME



**NEW
CLINICAL
TRIALS**

2017 ICOC Approved Budget



The **funding allocation** for 2017 (round and number of awards are for guidance purposes only)

Program	Rounds	Number	Allocation
Education	n/a	n/a	\$1M
Discovery	4	40	\$52M
Translation	3	9 -12	\$45M
Clinical	13	Up to 17	Up to \$215M*
Infrastructure	1	2**	\$16M
Totals	20	N/A	\$329

* Includes \$75M reserved for the ATP3 program

** Two new Alpha Clinics

The 2017 Plan

Beyond
CIRM 2.0
CALIFORNIA'S STEM CELL AGENCY
now it's personal

CIRM 2.0

CALIFORNIA'S STEM CELL AGENCY

BIG 6 2017



NEW
CANDIDATES INTO
DEVELOPMENT



PROGRESSION
EVENTS

REFINE
FIRST STEM CELL
ACCELERATED APPROVAL
APPLICATION



FDA

TRAN MILESTONES
HIT ON TIME



REACHING
AWARD MILESTONES

> HALF OF
THE MILESTONES
HIT EARLY



PARTNERED
CLINICAL PROJECTS



NEW
CLINICAL
TRIALS

On Tap...



Things I will be covering today

- **The CIRM Mission**
- **Review of the Strategic and Annual Plan**
- **2.0 Performance Review**
- **Budget Review**
- **Accelerating Center and Translating Center Update**

The Core of the Engine



DISCOVERY



TRANSLATION



CLINICAL

New
Idea



Single Product
Candidate



Pre-IND Meeting
or Equivalent



Approved
Therapy



1-2/Year

DISC1/2



3/Year

TRAN



12/Year

CLIN1/2

Program Offerings Per Year

CIRM 2.0 Performance



Summary of Inception (DISC1) stage applications to CIRM

- Total Inception Applications Received: **173**
- Applications Passing Eligibility: **172 or 99%**
- Applications Positively Selected by GWG: **60 of 101** (71 pending)
- Applications Recommended for Funding: **18** (**30%** of GWG adjudicated, **18%** of applications received)

Note

- This data is from one completed round and one round that is still in process.

CIRM 2.0 Performance



Summary of Quest (DISC2) stage applications to CIRM

- Total Quest Applications Received: **209**
- Applications Passing Eligibility: **141 or 97%** (63 pending)
- Applications Positively Selected by GWG: **78 of 141 (55%)**
- Applications Recommended for Funding: **19 (24% of GWG adjudicated, 13% of applications received)**

Note

- This data is from two completed rounds and one round that is still in process.

CIRM 2.0 Performance



Summary of Translational stage applications to CIRM

- Total Translational Applications Received: **85**
- Applications Passing Eligibility: **61 or 72%**
- Applications with Final GWG Dispositions: **61**
- Applications Recommended for Funding: **14** (**23%** of GWG adjudicated, **16%** of applications received)

Note

- This data is from three completed rounds (2 with final ICOC funding decisions).
- Positive selection was not employed during any round.

CIRM 2.0 Performance



Summary of Clinical stage applications to CIRM

- Total Clinical Applications Received: **74**
- Applications Passing Eligibility: **50 or 72%** (5 pending)
- Applications with Final GWG Dispositions: **48**
- Applications Under Review: **5**
- Applications Recommended for Funding: **22** (**44%** of GWG adjudicated, **30%** of applications received)

Key Takeaways

- Eligibility passing rates are down
- The GWG/ARS is dealing with a very high volume/workload
- GWG approval rates are up
- Significant additional volume needed to reach our goals

On Tap...



Things I will be covering today

- **The CIRM Mission**
- **Review of the Strategic and Annual Plan**
- **2.0 Performance Review**
- **Budget Review**
- **Accelerating Center and Translating Center Update**

The Buckets

Two separate funding sources



The Little Bucket

Administrative funding 2016 full year activity

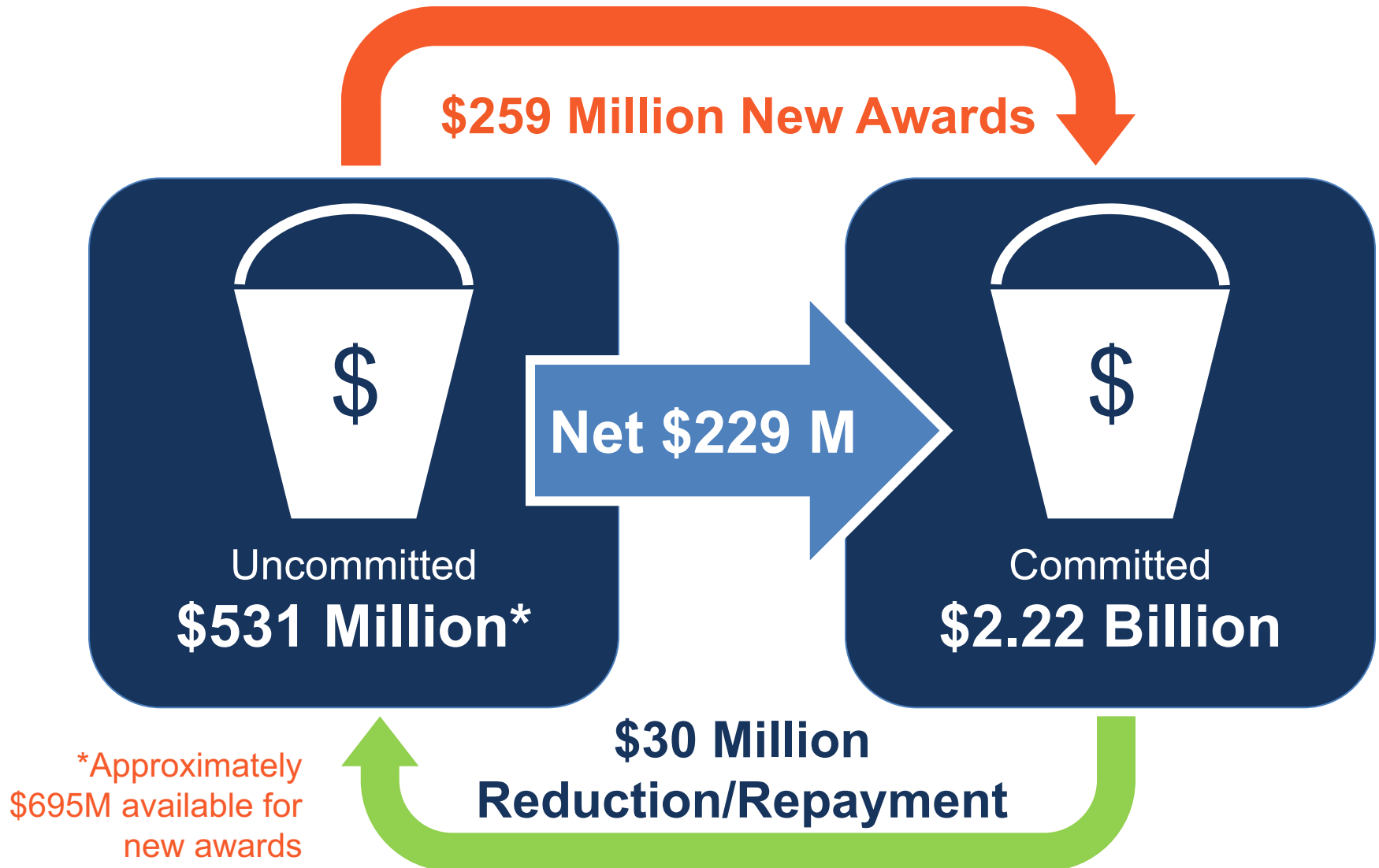


Administrative Budget
\$180 Million

Funding available through 2020

- \$123 million spent
- \$57 million remaining
- Current spend rate of \$15 million per year
- Fiscal responsibility is very important

Calendar Year 2016 Actuals



On Tap...



Things I will be covering today

- **The CIRM Mission**
- **Review of the Strategic and Annual Plan**
- **2.0 Performance Review**
- **Budget Review**
- **Accelerating Center and Translating Center Update**

The “Pitching Machine”

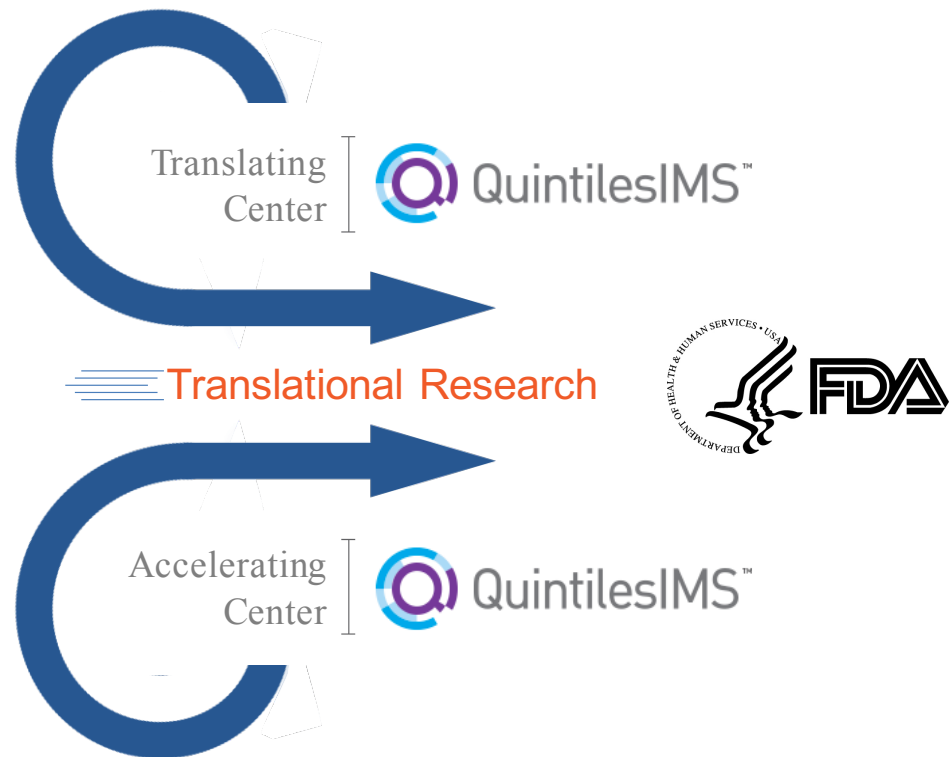
Designed to help efficiently move great ideas into clinical trials

Goal

Discovery to Clinic in <4 years.

Status Report

- Both the AC & TC are up and running (now the Stem Cell Center)
- Already assisting 5 programs, including several 1.0 programs
- Playing a strong role with “hunting” activities



Thank You!

