

Beyond
CIRM2.0
CALIFORNIA'S STEM CELL AGENCY

now it's personal

Strategic Plan Executive Summary

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The Strategic Planning Process

Where are we now?

- Evaluate environment
- Confront brutal facts
- Confirm mission

Where do we want to go?

- Brainstorm options
- Benchmark
- Create a unique vision

How do we get there?

- Determine strategy
- Establish milestones
- Document the plan

Funding Runway

Current levels sufficient to last approximately 5 years

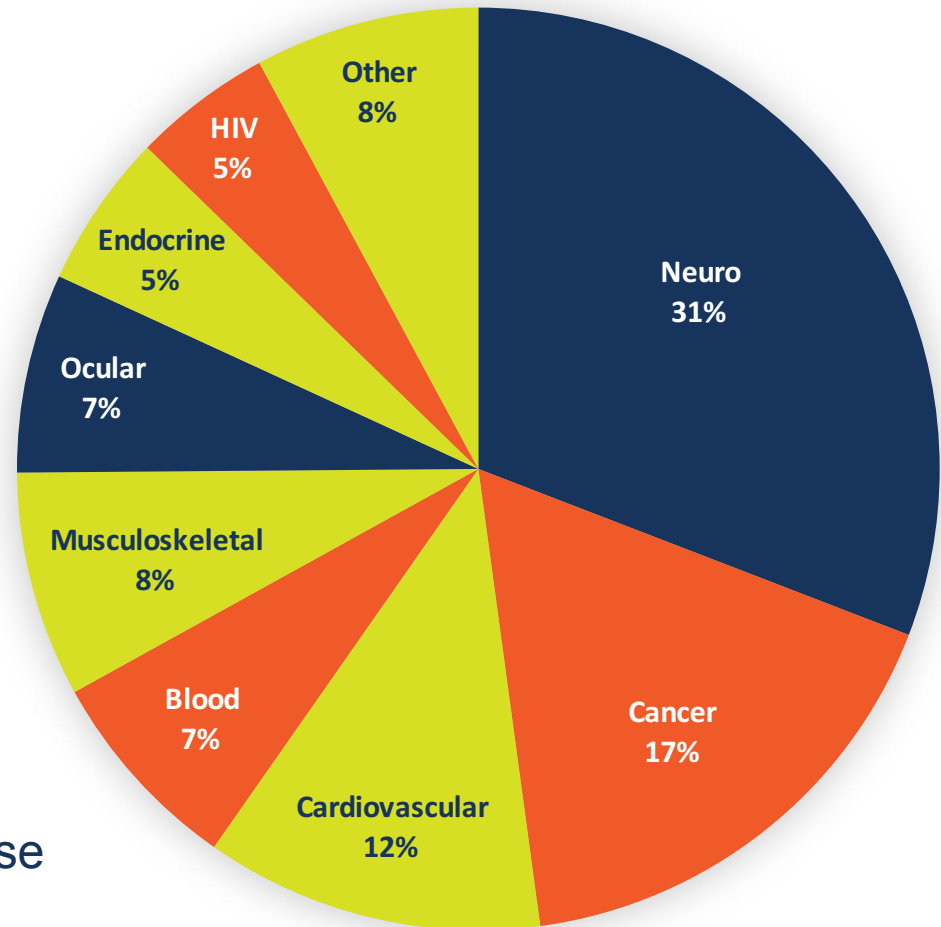


- \$2 billion awarded or spent
- \$775 million uncommitted
- \$190-200 million in new awards per year
- Estimated \$20 million in award reductions annually
- Projected NET commitment rate of \$170 million per year

Where We Are Today

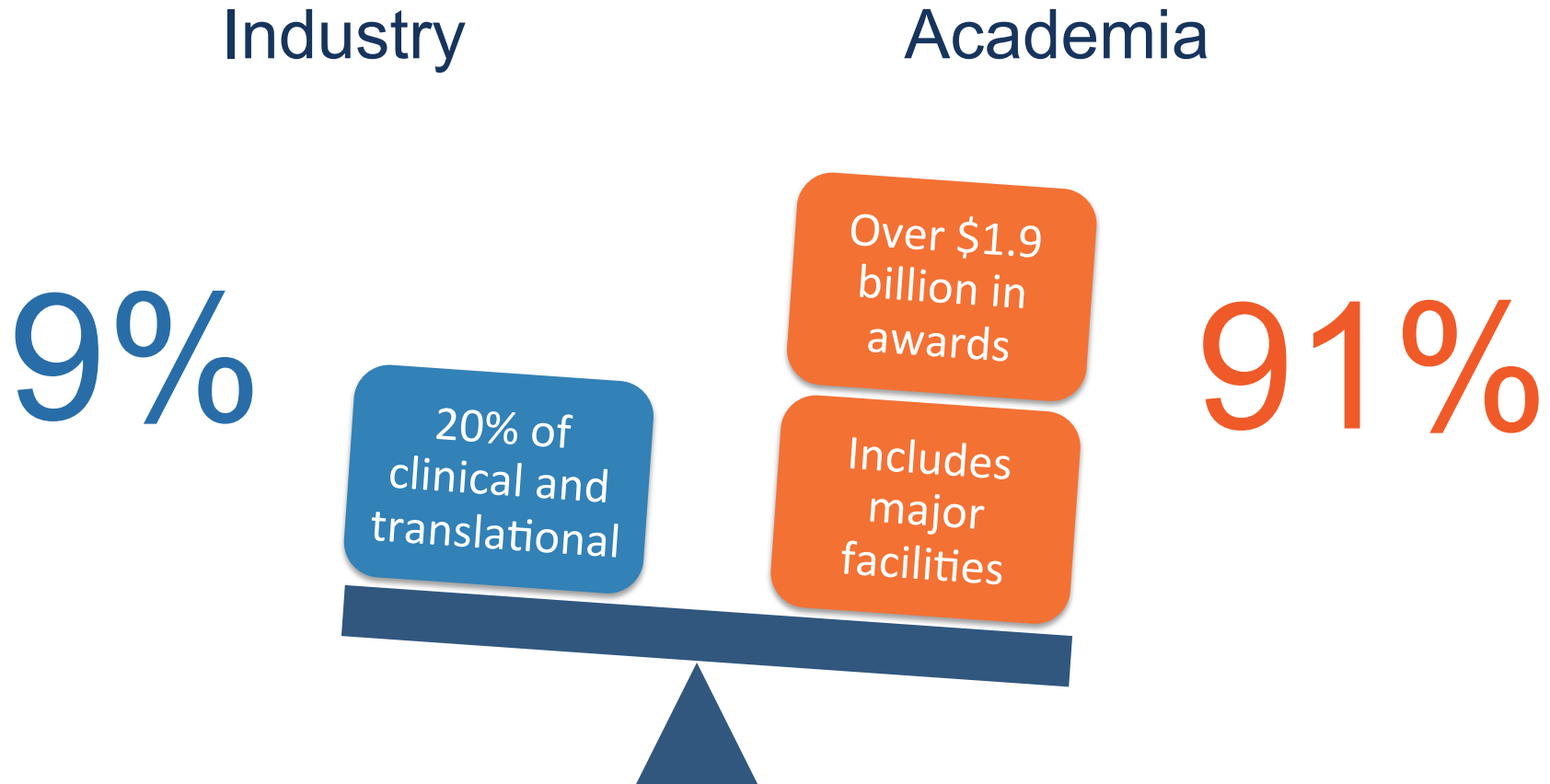
\$1.3 billion invested in disease specific research including 15 clinical trials

- HIV/AIDS (3)
- Solid Tumors (2)
- Heart Failure
- Leukemia
- Sickle Cell Anemia
- Spinal Cord Injury
- Type 1 Diabetes
- Macular Degeneration
- Retinitis Pigmentosa
- Malignant Melanoma
- Chronic Granulomatous Disease
- Glioblastoma



Where We Are Today

The vast majority of funding has gone to academia

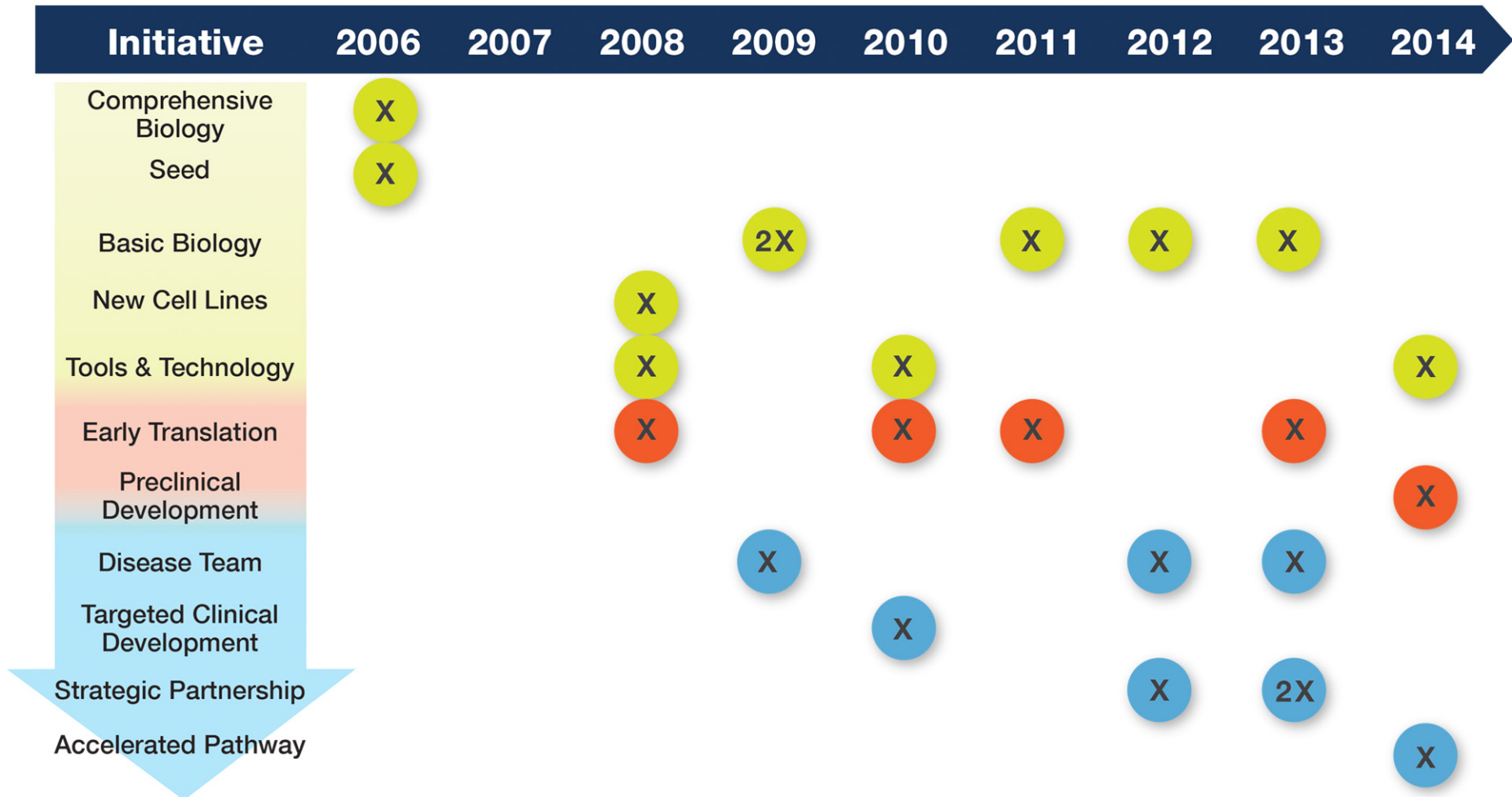


Where We Are Today

\$2 billion allocated to CIRM's five major components



CIRM Existed as an Initiative Based Agency



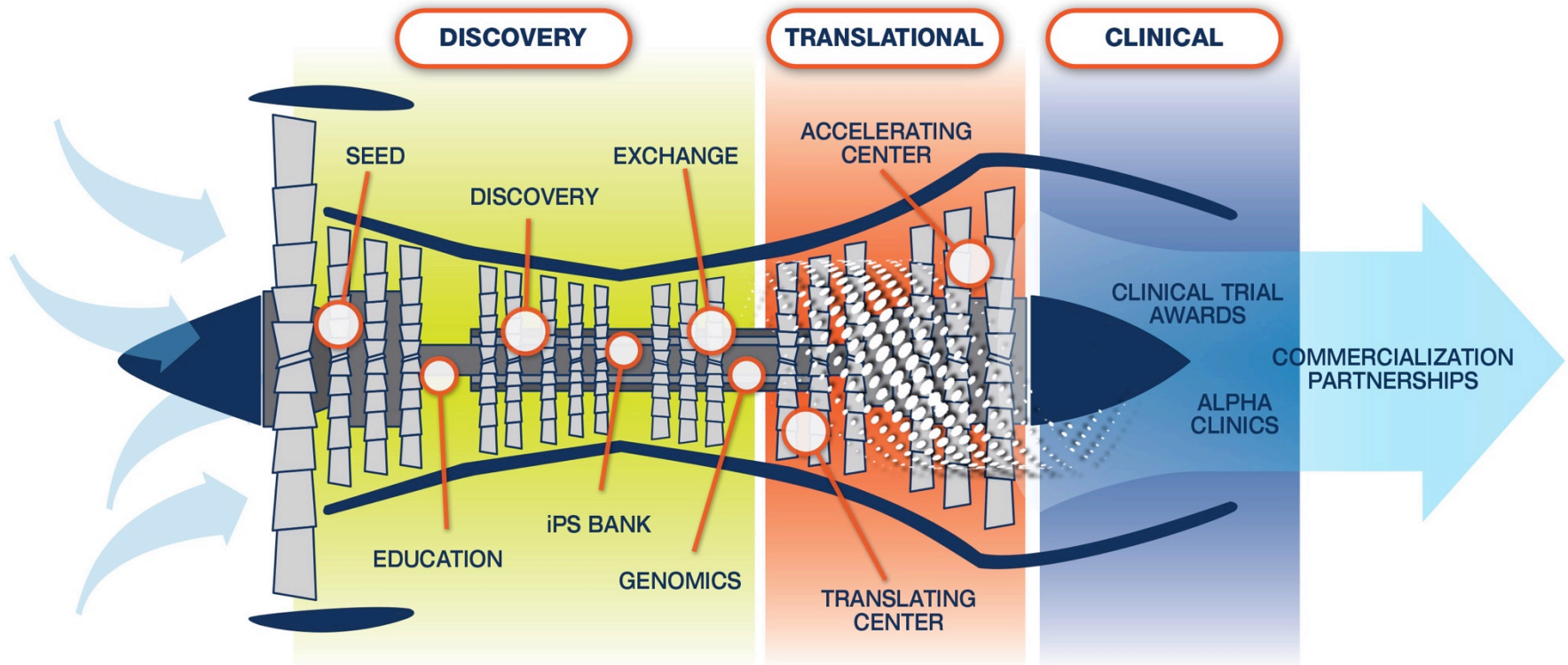
CIRM 2.0 Introduced a Systems Based Approach



PROGRAM	2016	2017	2018	2019	2020
Seed	X	X	X	X	X
Discovery	2X	2X	2X	2X	2X
Specific Question	2X	2X	2X	2X	2X
Translation	2X	2X	2X	2X	2X
IND/IDE Seeking	12X	12X	12X	12X	12X
Clinical Trials	12X	12X	12X	12X	12X
Accelerating Activities	12X	12X	12X	12X	12X

Assembling the Parts

Integrating the pieces will turn CIRM into a stem cell accelerating machine



Stakeholder Meetings



Key insights from an honest and thorough assessment

- Patient advocates want a more active role in achieving the mission
- Investigators want help with IND enabling translational activities and regulatory filings
- Every academic meeting included a request for help linking researchers at different stages
- Many of CIRM's programs are still not widely known to all potential partners

Interesting Survey Findings

Responses from 217 individual stakeholders

- Risk tolerance is high among all stakeholders (64% responded 4+ of 5)
- Number of therapies demonstrating proof of concept in humans is the most important metric of success (70%)
- 62% indicated that CIRM should fund projects that can receive funding from other sources only when it accelerates the project
- FDA is viewed as the biggest impediment to the development of stem cell therapies (70%)

Where We Are Today



Key insights from an honest and thorough assessment

- Historically, CIRM existed as an initiative (vs. system) based agency
- With a few exceptions, most priorities are aligned among stakeholders
- The translation stage represents a significant opportunity for CIRM to impact speed and efficiency
- Stem cells therapies continue to be disadvantaged (regulatory and commercial)
- The regulatory environment is seen as a major impediment to stem cell therapy

The Plan

MISSION

VISION

STRATEGIC THEMES

RESULTS

RISKS



Let's Go!

Mission Confirmation

First, have a great mission

To accelerate stem cell treatments to patients with unmet medical needs.



96% of stakeholders agree with the mission

MISSION

VISION

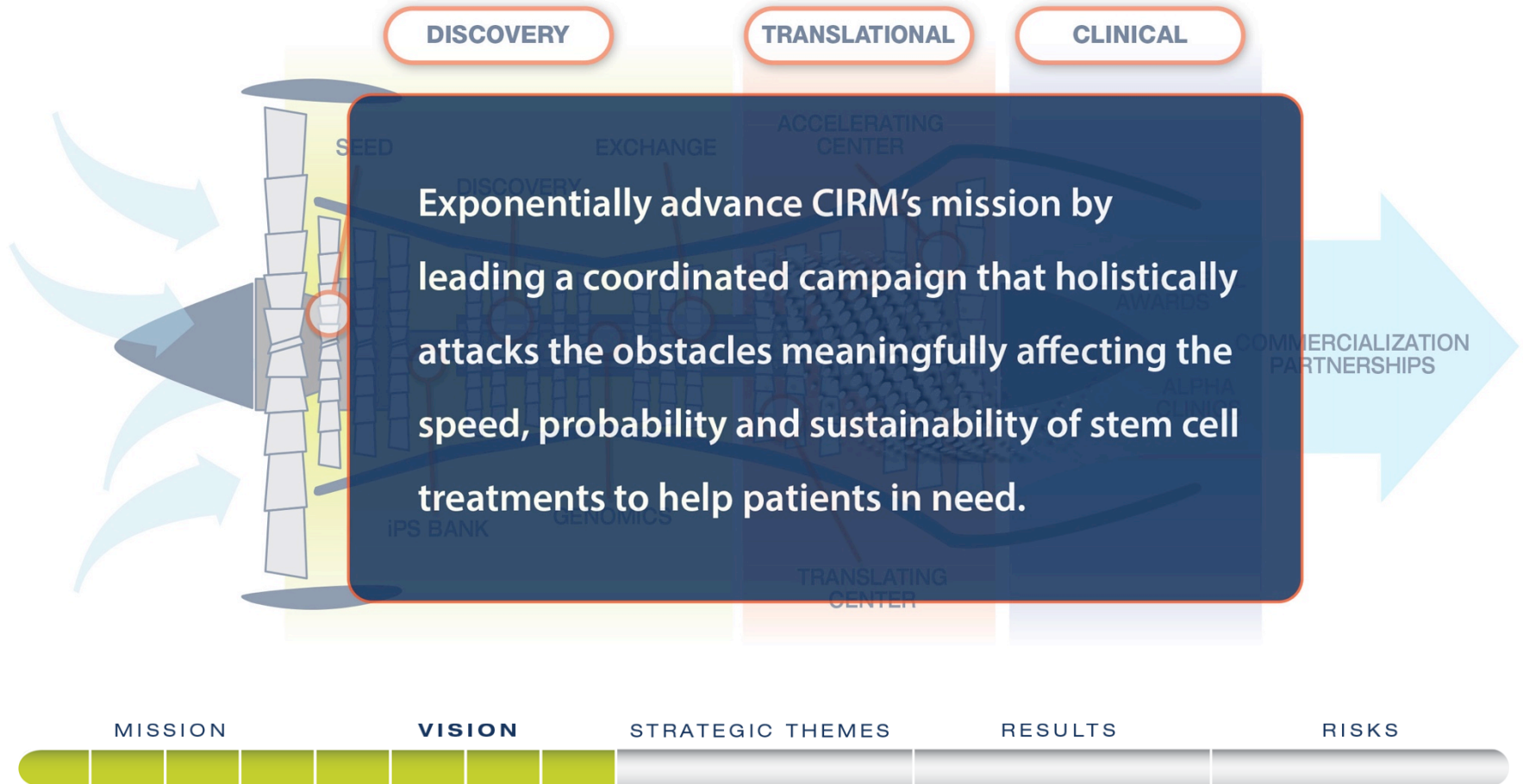
STRATEGIC THEMES

RESULTS

RISKS

Where We Are Going

A bold vision to maximize CIRM's impact





MISSION

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STRATEGIC THEMES

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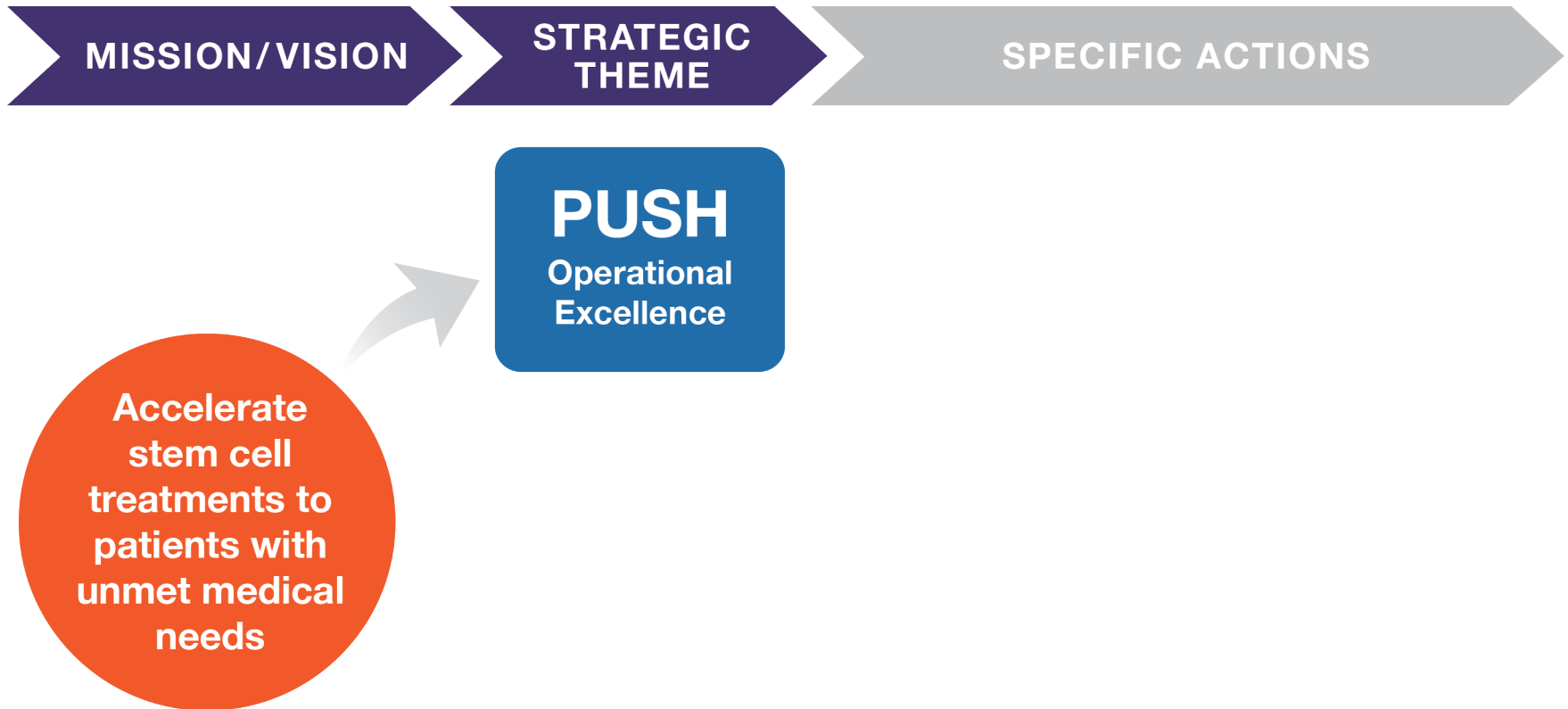
RISKS

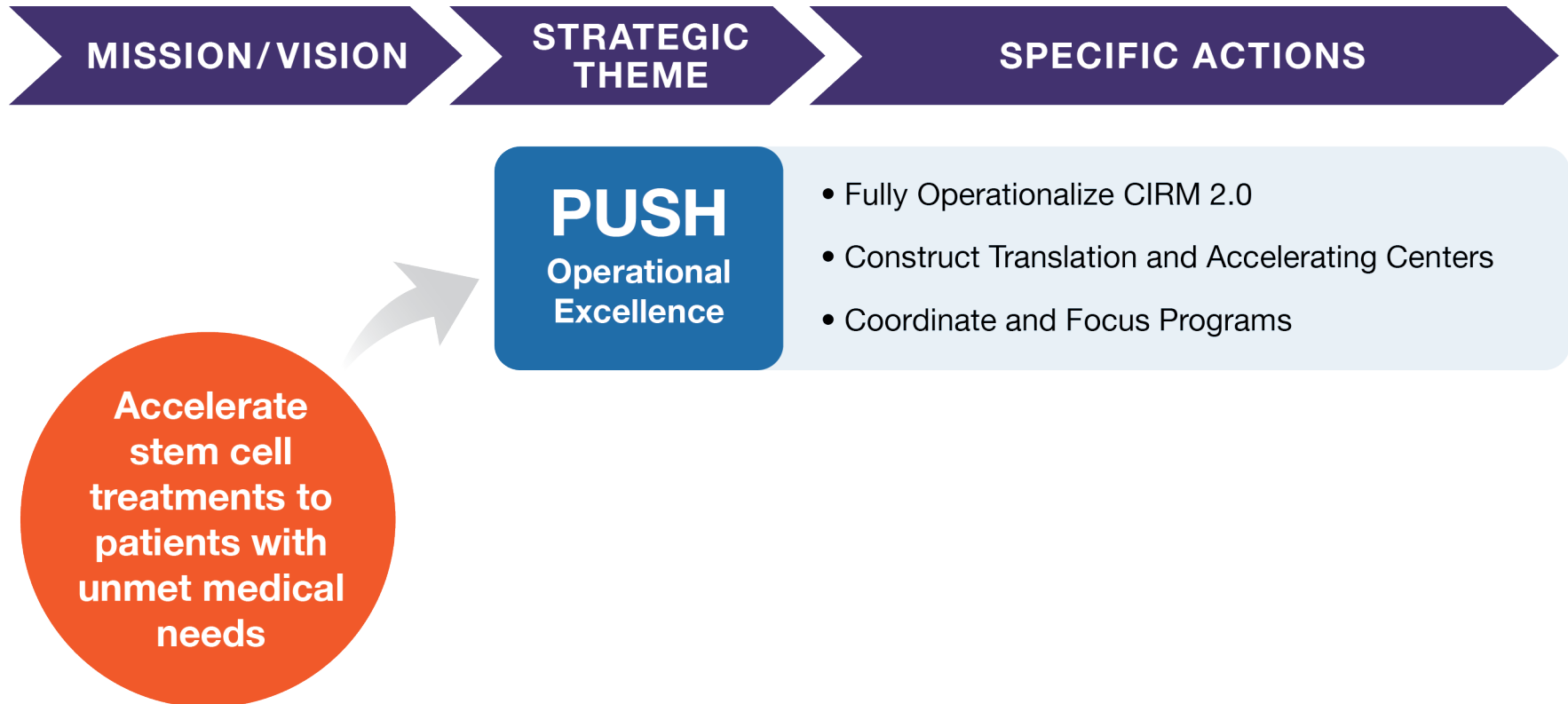




Accelerate
stem cell
treatments to
patients with
unmet medical
needs









HOPE

CURE



MISSION

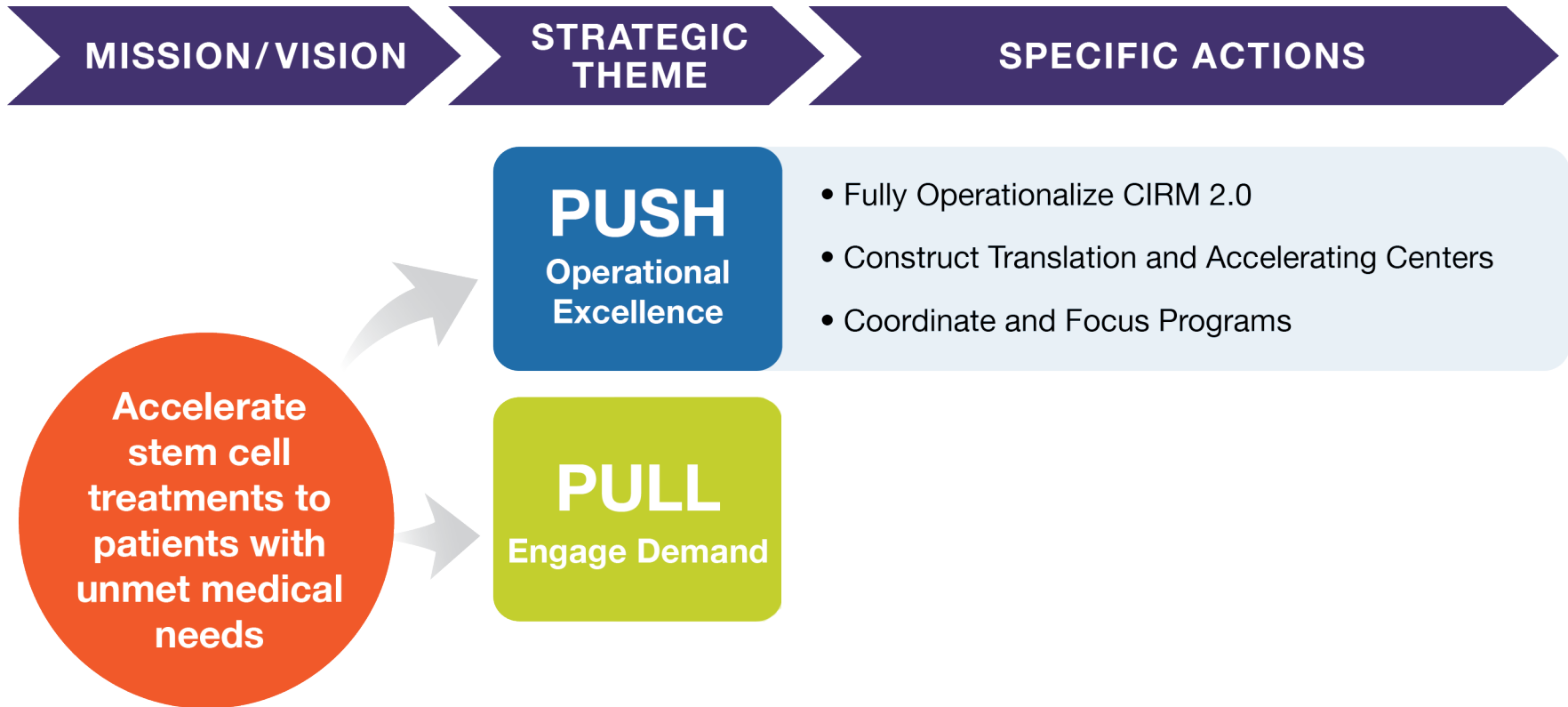
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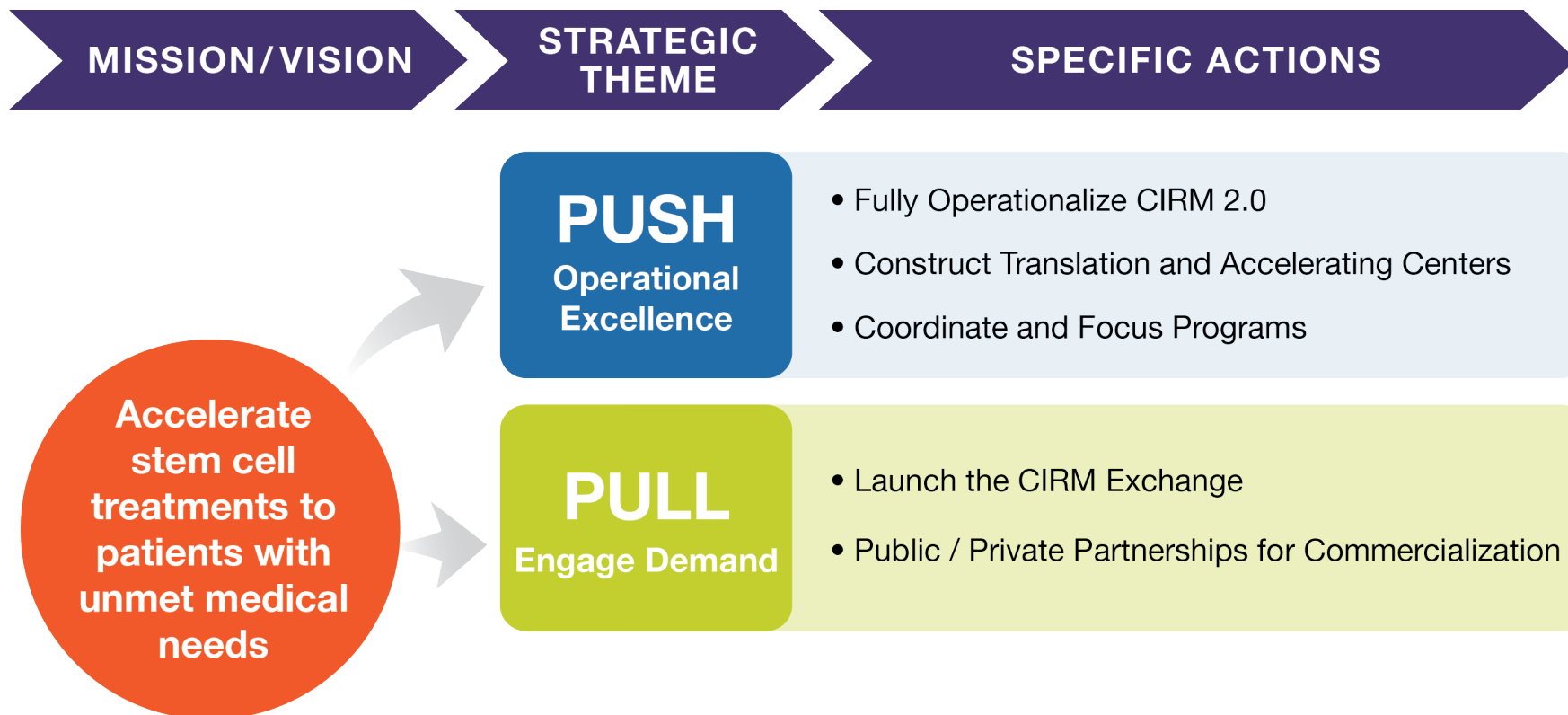
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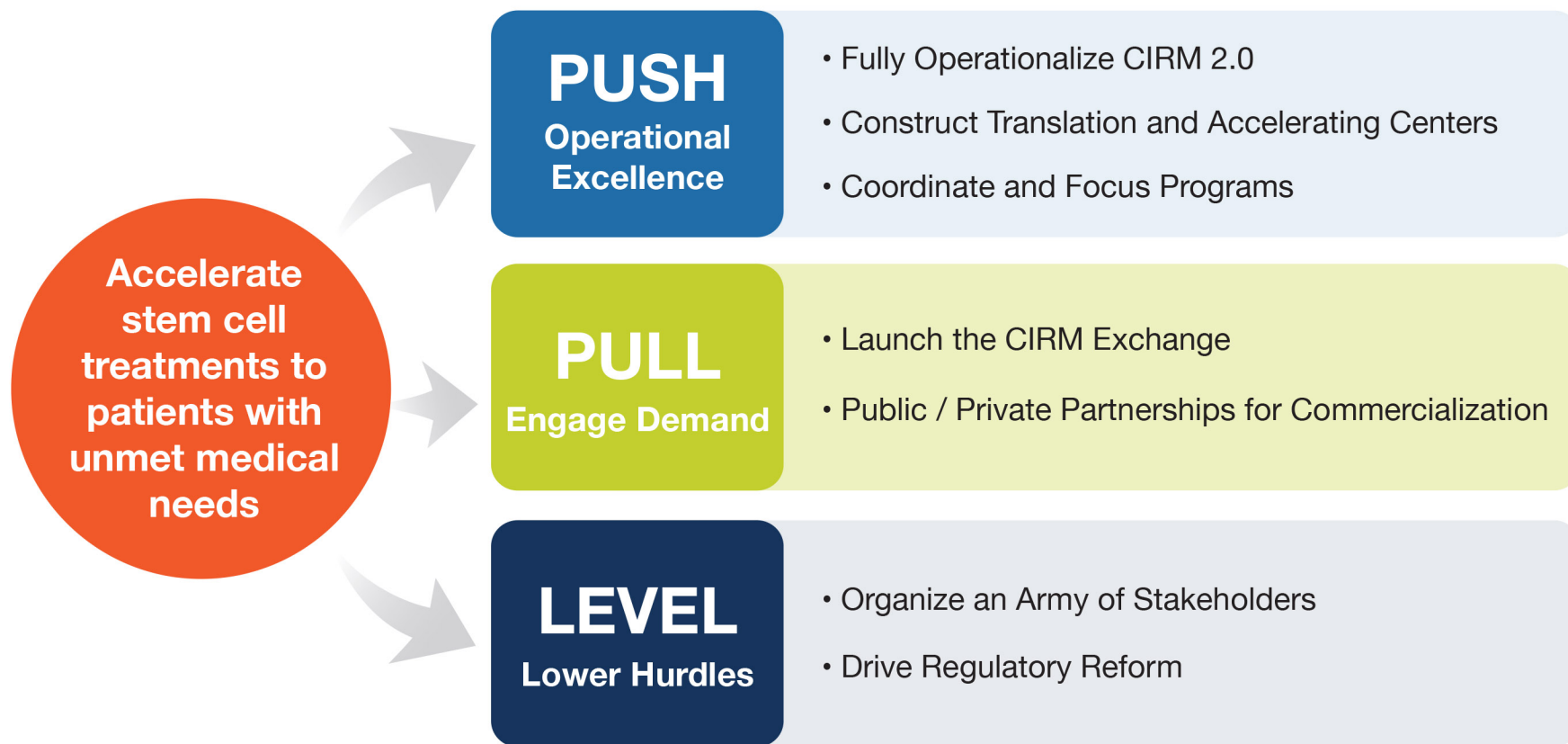
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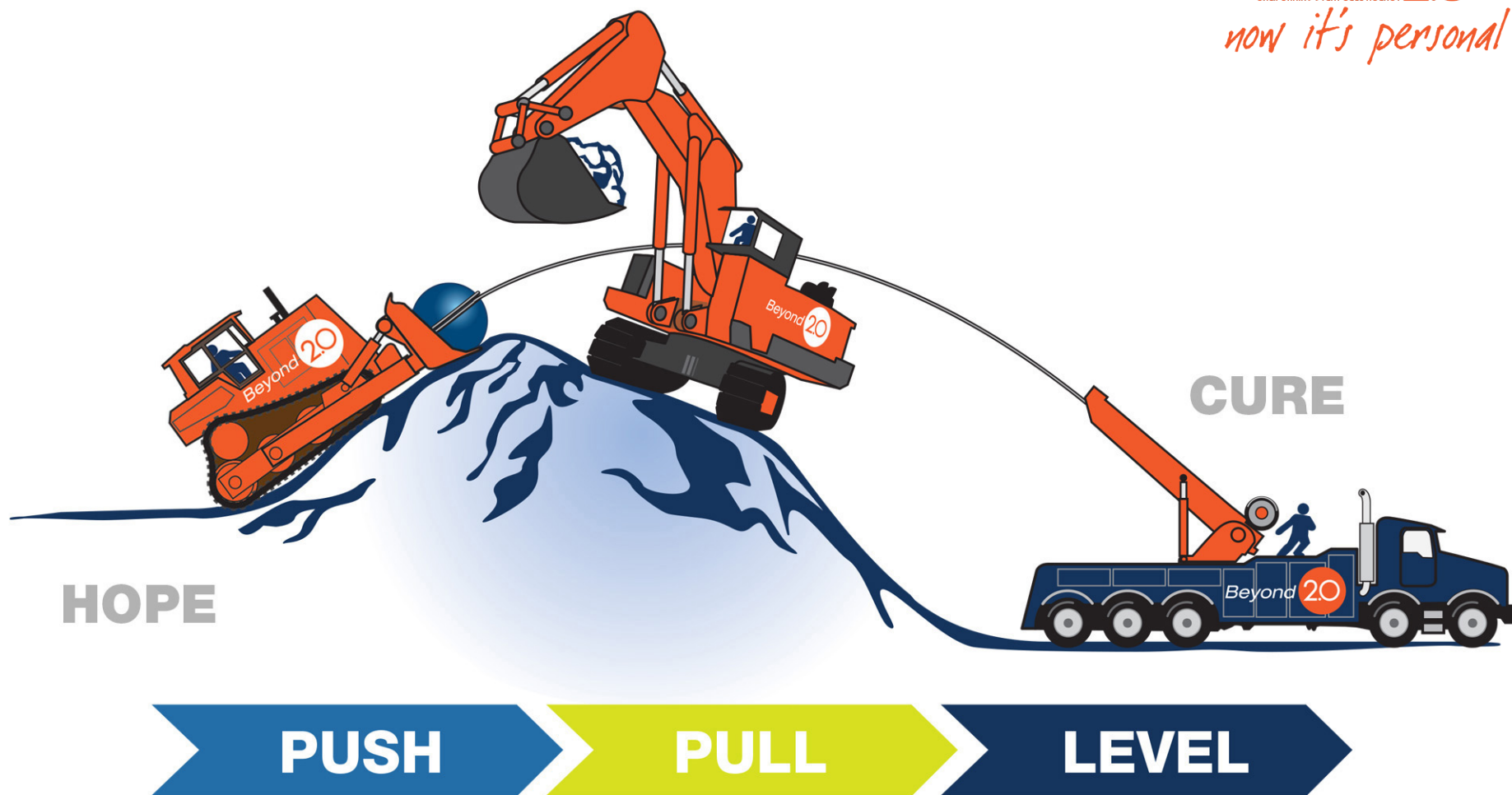
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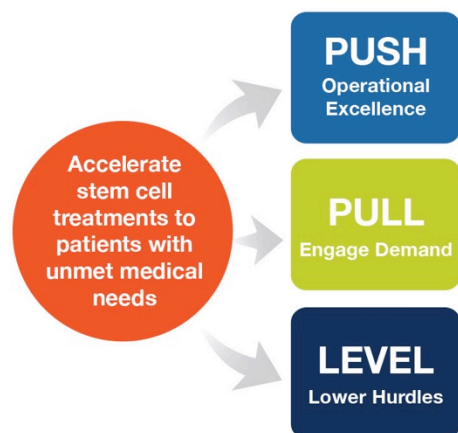
RESULTS

RISKS





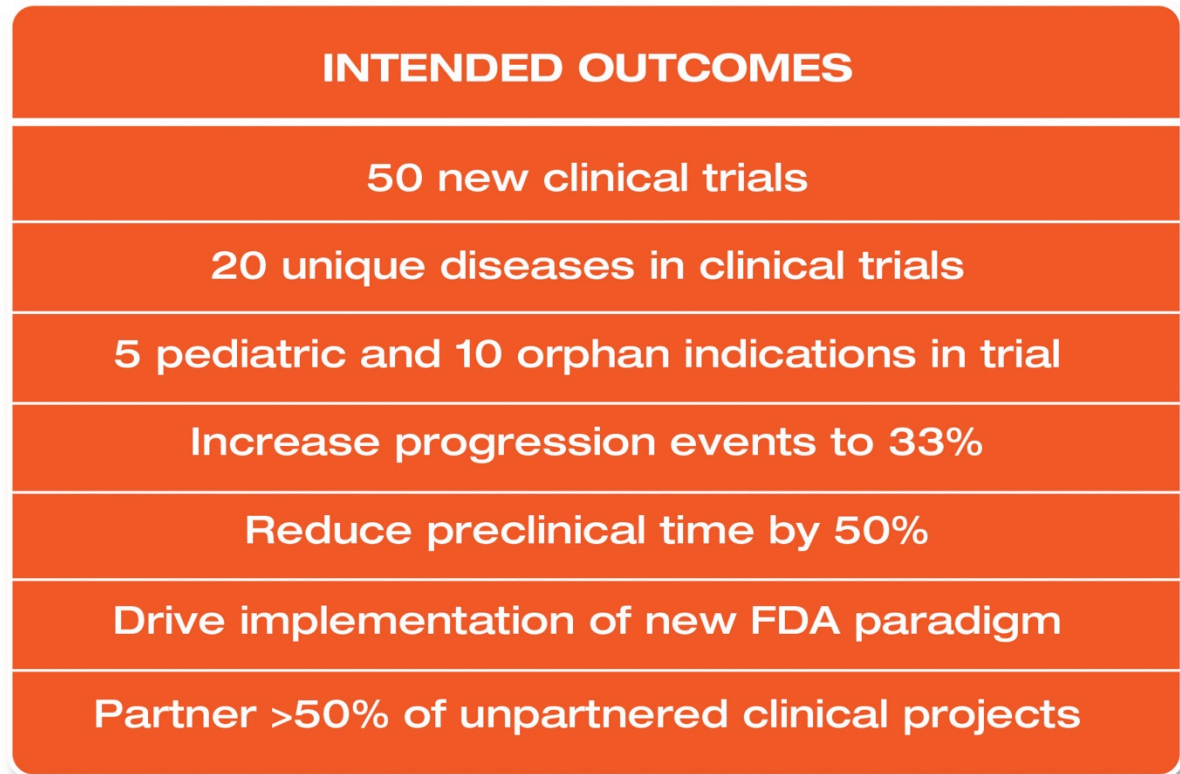
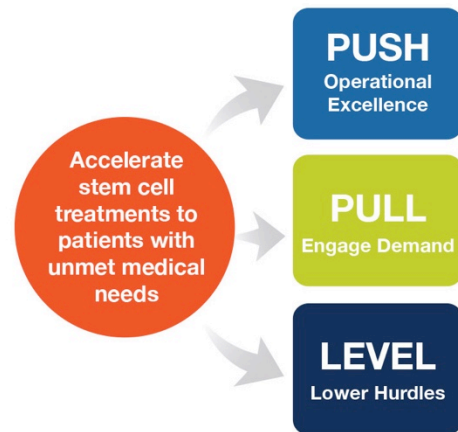
Financial Outlook



Program	2016	2017	2018	2019	2020	Total
Education	10	10	10	10	10	50
Discovery	15	50	50	35	30	180
Translation	25	40	45	35	30	175
Clinical	100	85	75	75	65	400
Infrastructure	15	15	20	20	15	85
Totals	165	200	200	175	150	890



Financial Outlook

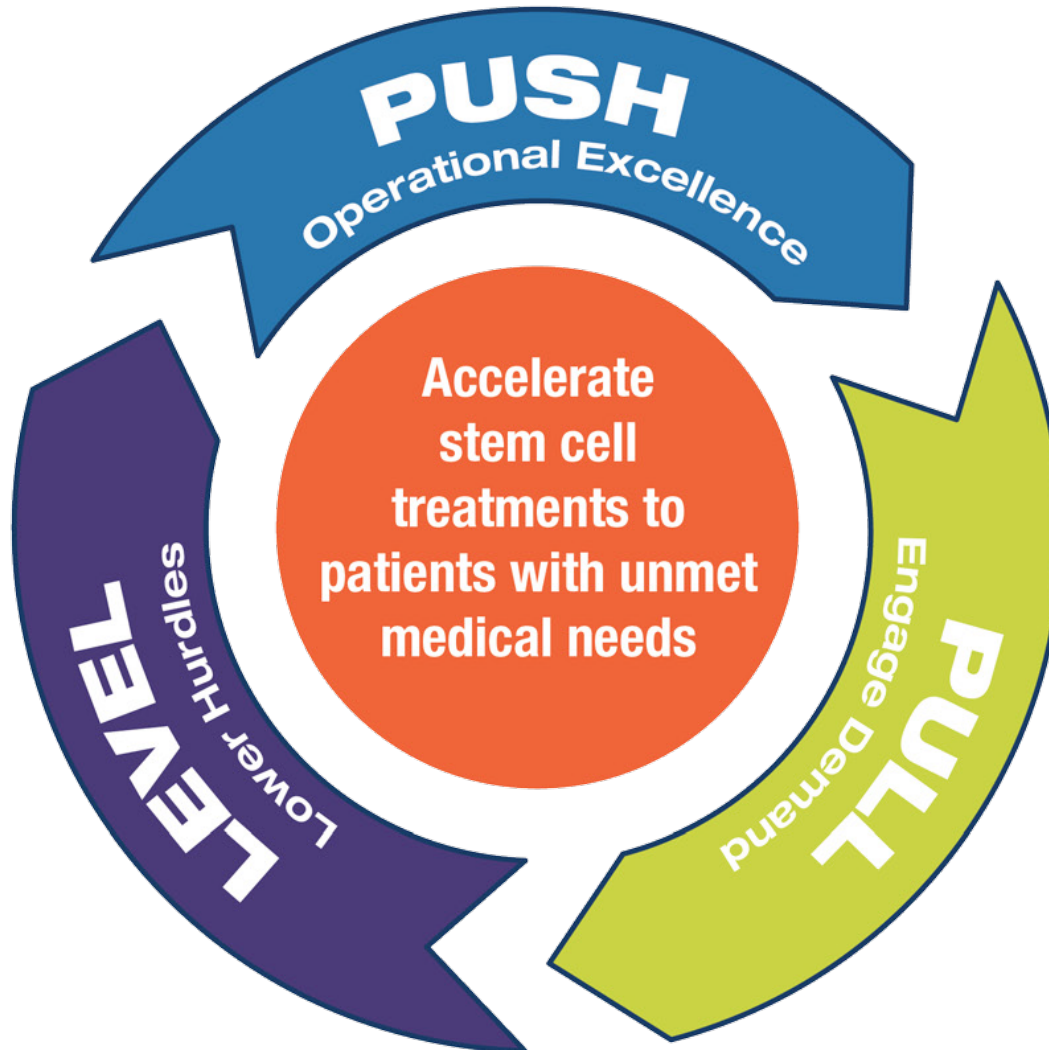


Risks

Key risks include but are not limited to the following

- There may be an insufficient number of meritorious therapeutic candidates to reach our goals
- There may be insufficient interest from qualified applicants to participate in key competitions
- The current limited funding of the agency could affect the ability to retain or attract personnel
- Investors may be uninterested in stem cell therapies
- The FDA may be unwilling to improve the regulatory environment

Moving Beyond CIRM 2.0 with an Integrated Approach



Questions or comments?

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