

CURE

Beyond
CIRM 2.0
CALIFORNIA'S STEM CELL AGENCY

now it's personal

The Need for a New Regulatory Paradigm

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HOPE

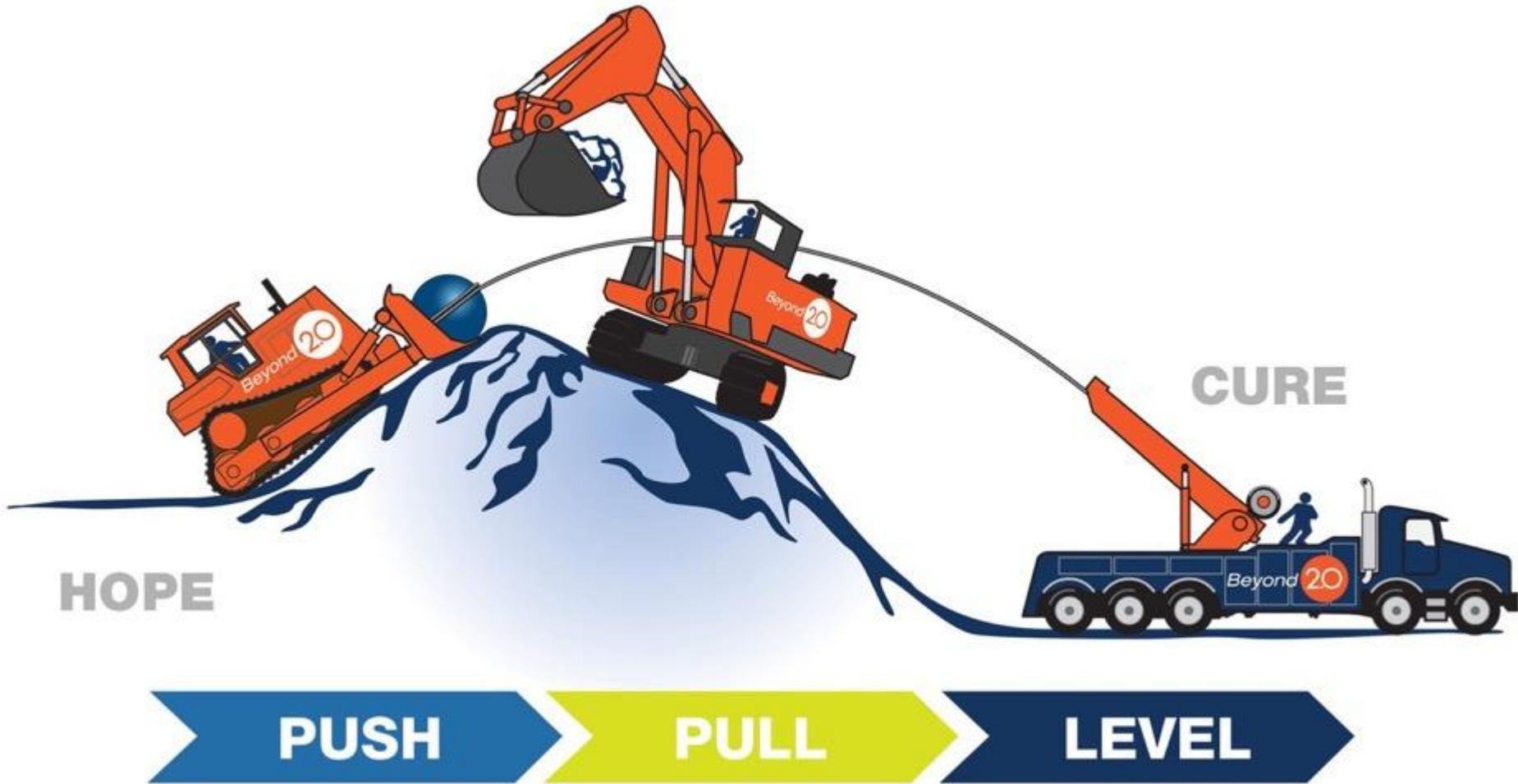
Our Mission

To accelerate stem cell treatments to patients with unmet medical needs

The CIRM Strategic Plan

We are **ALL IN**

Beyond
CIRM_{CALIFORNIA / ITEM CELL AGENCY}**2.0**
now it's personal



The current regulatory pathway

FDA was the most frequently cited impediment to developing stem cell treatments

The percentage of survey respondents that view FDA as an impediment

70

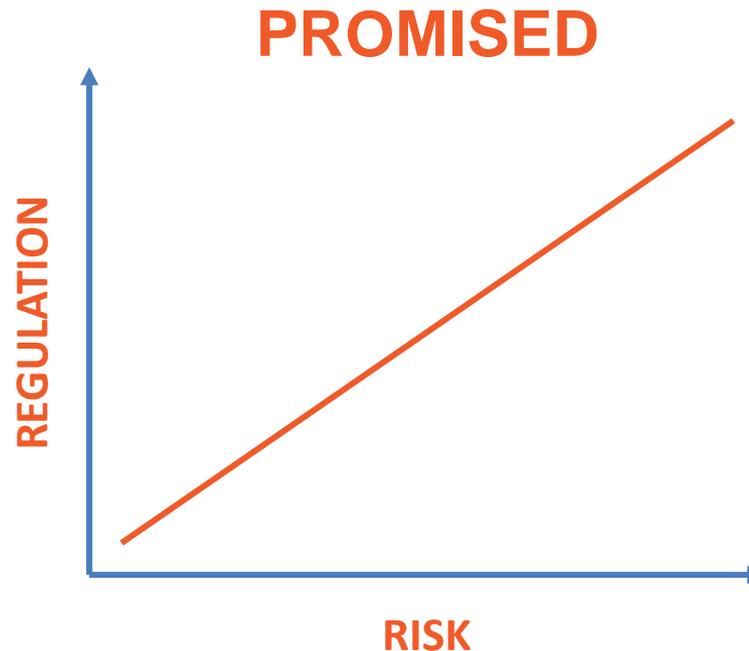
Promised vs. Delivered

FDA originally proposed a “tiered” approach

- “Under this tiered, risk-based approach, we propose to exert only the type of government regulation necessary to protect the public health.”
- “The regulation of different types of human cells... will be commensurate with the public health risks presented...”
- “These planned improvements will increase the safety of human cells... while encouraging the development of new products.”

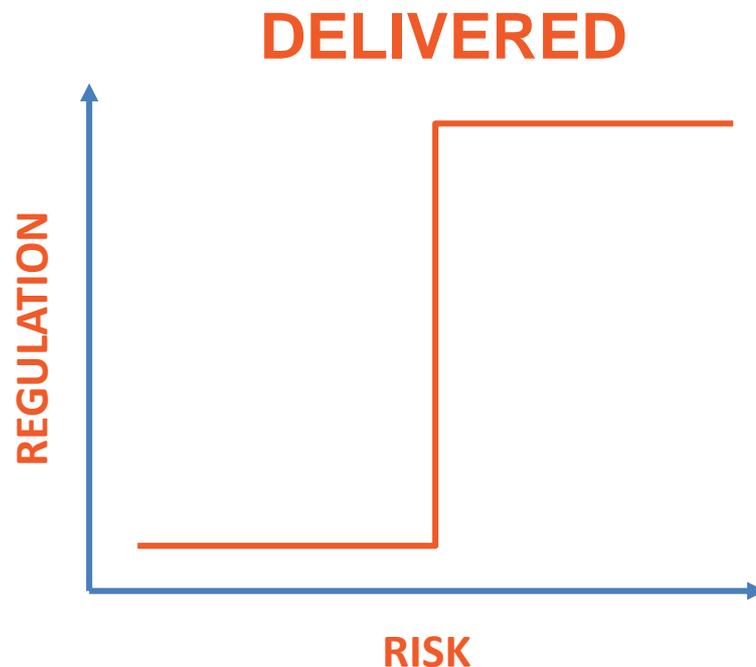
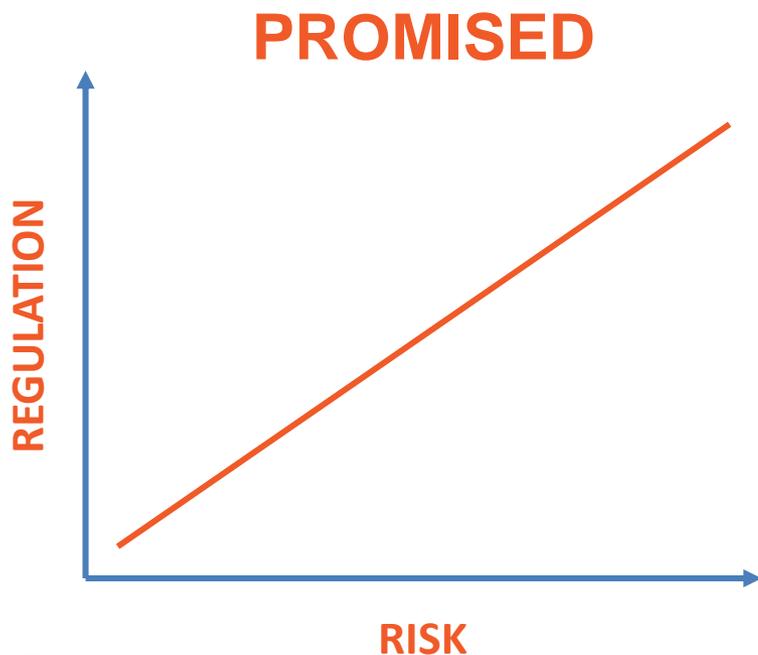
Promised vs. Delivered

A progressive approach was promised,
where regulation scaled with risk



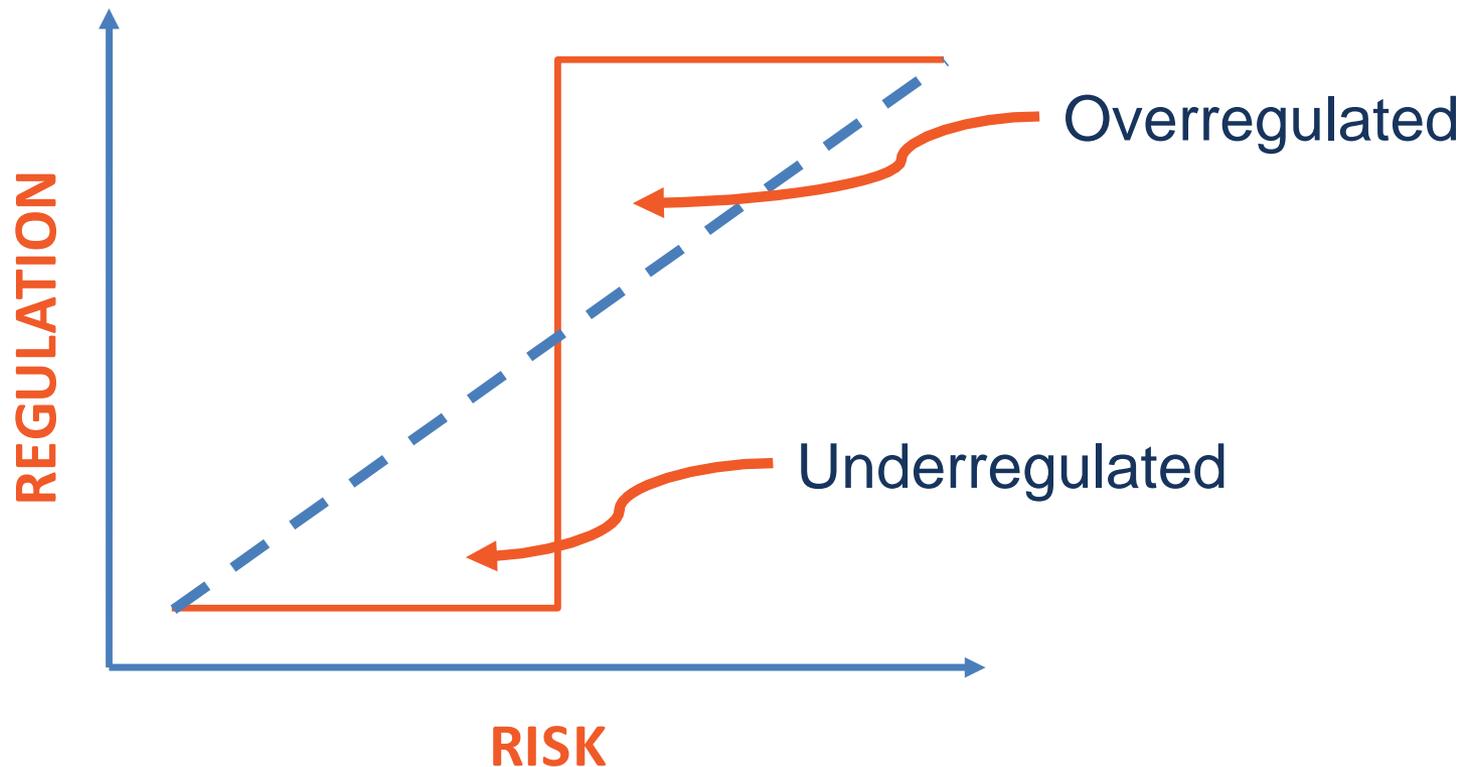
Promised vs. **Delivered**

Instead, we received binary regulation that is either OFF or ON



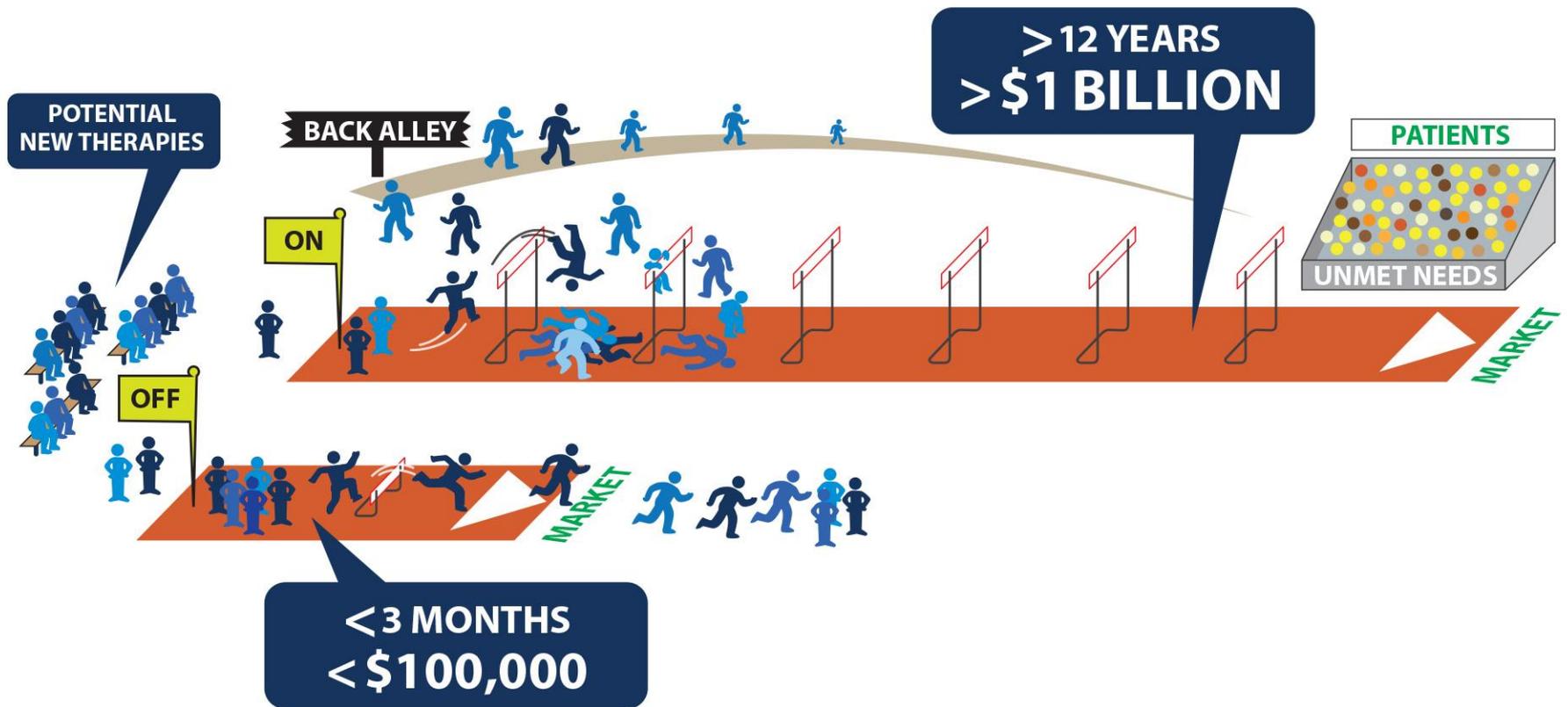
This is A Real Problem

It leads to both
Under and **Over** regulation



The Effects of the System

The current approach drives sub-optimal behavior



This Causes Inconsistent Application

FDA attempts to compensate with selected enforcement

- The cells from corneas have been successfully transplanted for more than 50 years
- Last year alone, 47,000 Americans had their eye sight restored through corneal cell transplantation
- If FDA enforced its current regulations, surgeons would not be allowed to perform this procedure
- **Uncertainty has a chilling effect on new product development**

As a Result Performance Suffers

The number of stem cell products approved by FDA after 15 years under the current paradigm

0

The Three Reasons We Need A New Approach



The current system:

- **Is not the tiered approach promised and needed**

It is inconceivable that the entire spectrum of cell therapies can be appropriately regulated with an “**ON-OFF**” switch

- **Is inconsistently applied to compensate for itself**

The regulation is arbitrarily followed by the agency that created it – driving uncertainty for those developing new products

- **Has delivered poor results**

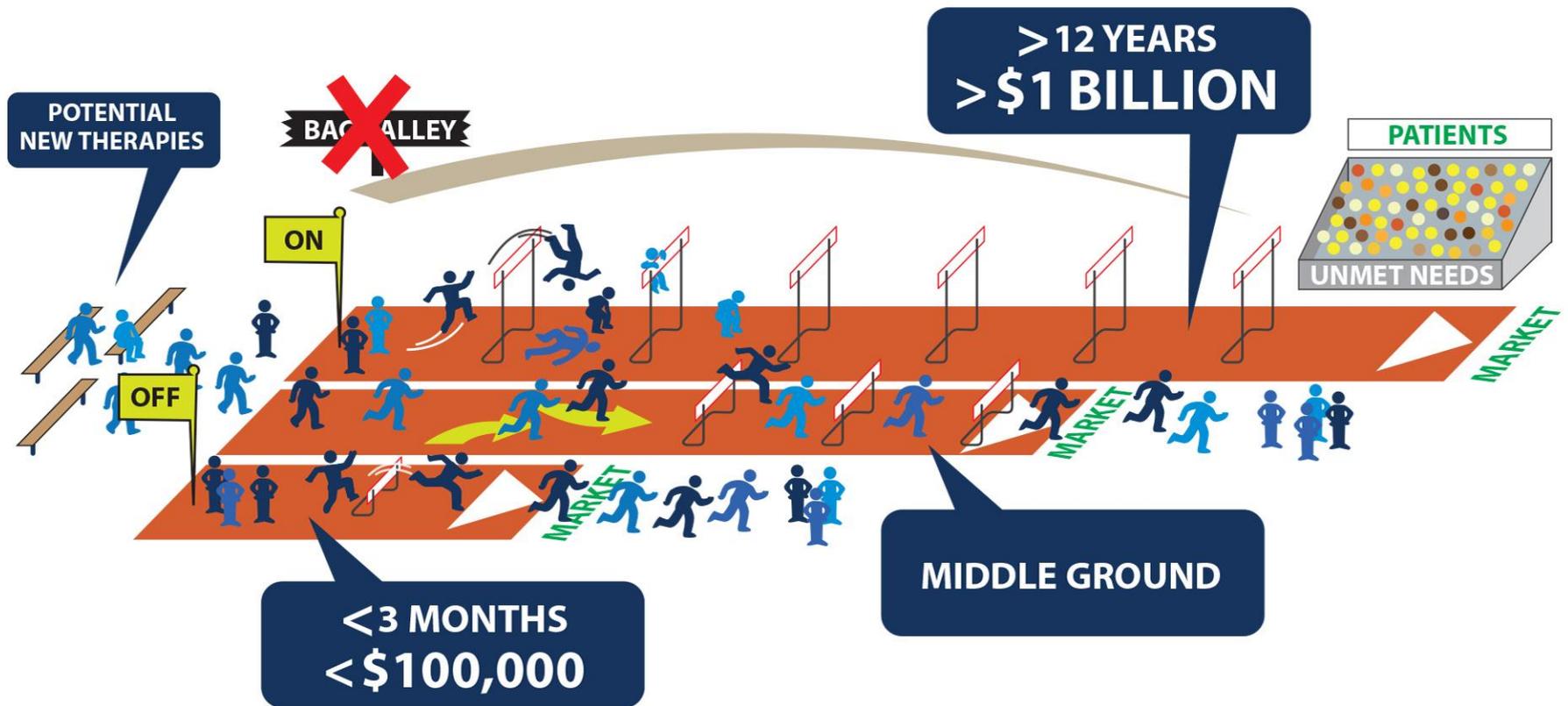
By having a system that approves nothing after 15 years, we are neither protecting nor helping those in need

For Clarity

- CIRM does **not** oppose regulation and does **not** support the abolition of regulation
- CIRM is **not** anti-FDA
- We **do** believe that the regulatory burden placed upon the development of cell therapies must:
 - ✓ be scaled to more accurately reflect the risks,
 - ✓ be balanced against the very real consequences of doing nothing, and
 - ✓ be fairly and consistently applied.
- CIRM is not wed to any single approach. We only want to see the problem fixed... now.

The System Can Be Fixed

And used to drive better outcomes for patients



We all need to

IMAGINE BETTER

and

ACT