

# 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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President and Chief Executive Officer



# HOPE

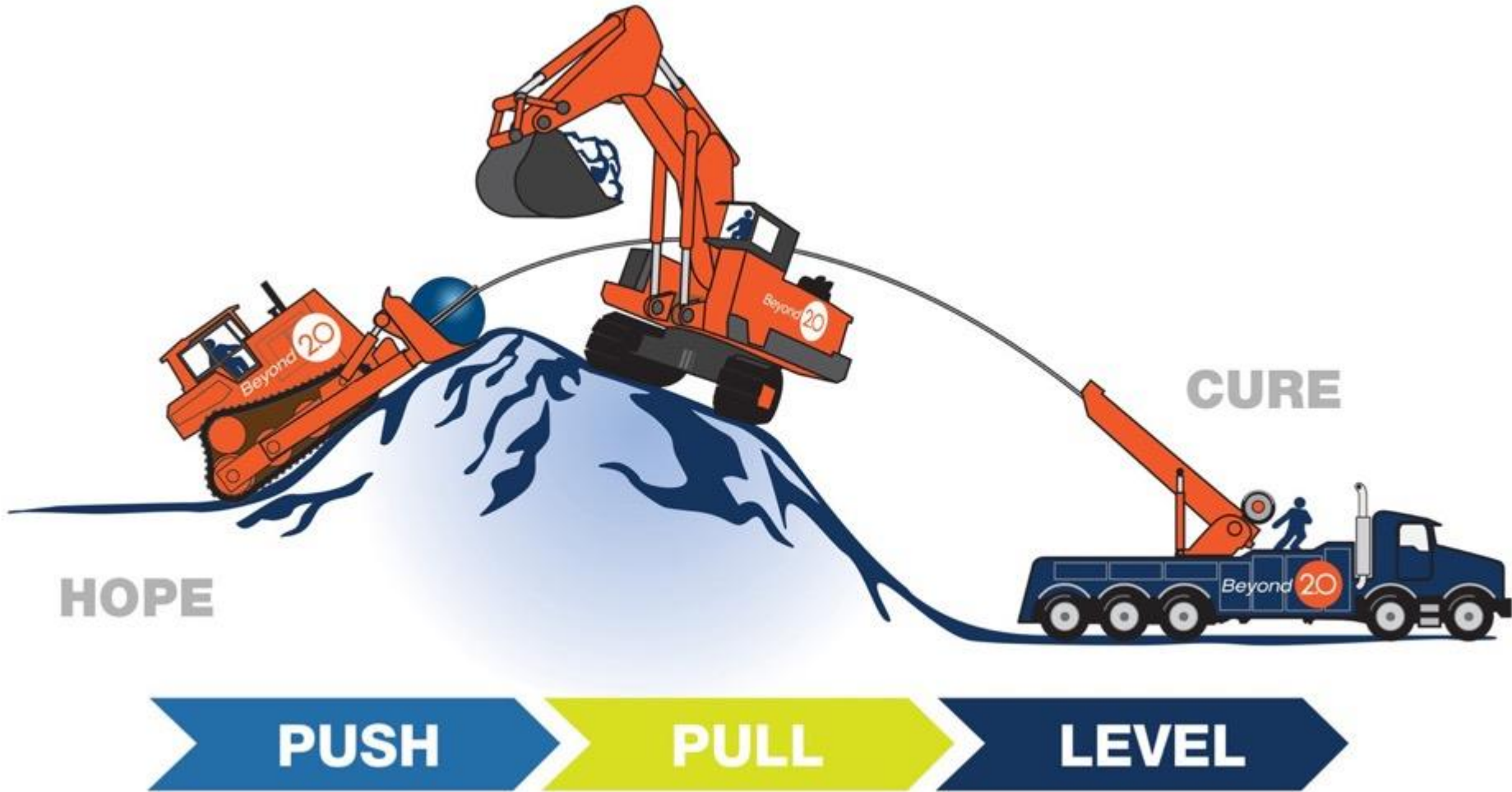
# Our Mission

To accelerate stem cell treatments to patients with unmet medical needs

# The CIRM Strategic Plan

## We are **ALL IN**

Beyond  
**CIRM**<sub>CALIFORNIA / ITEM CELL AGENCY</sub>**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

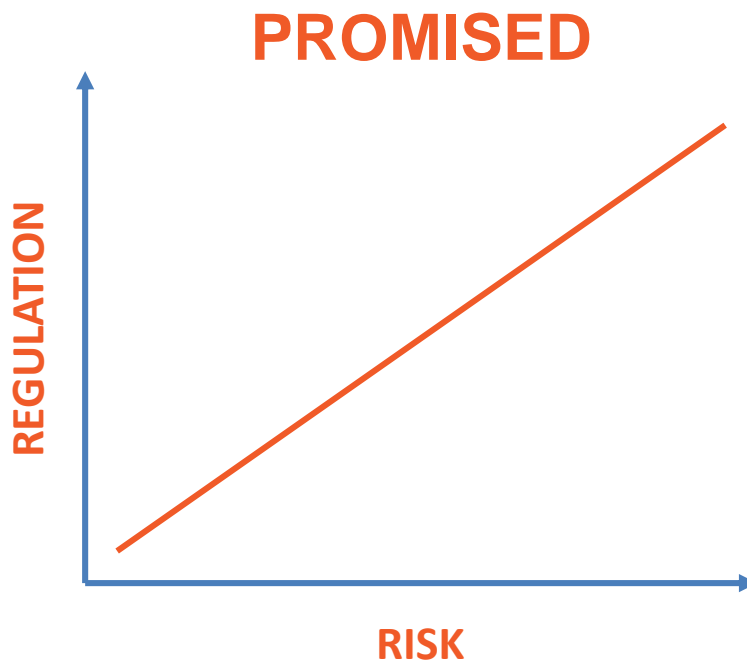
# Promised vs. Delivered

## 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.”

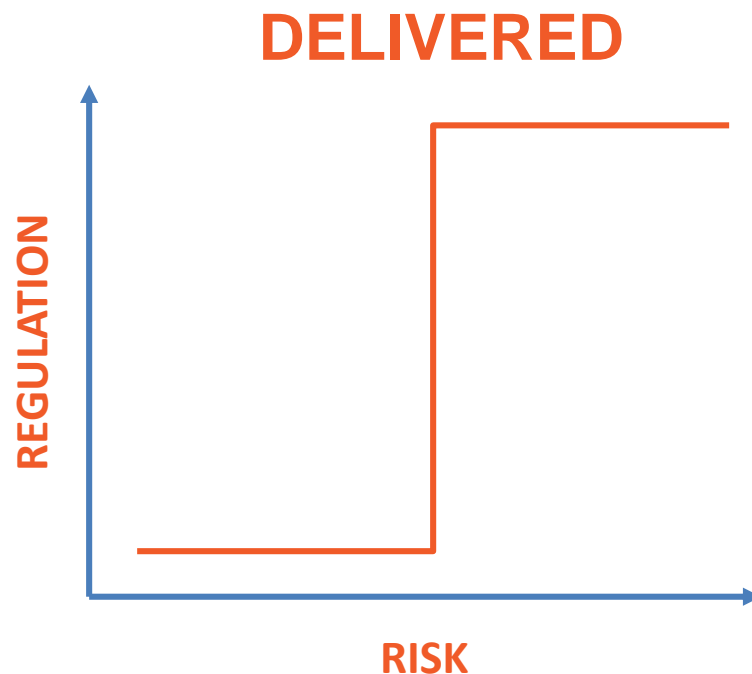
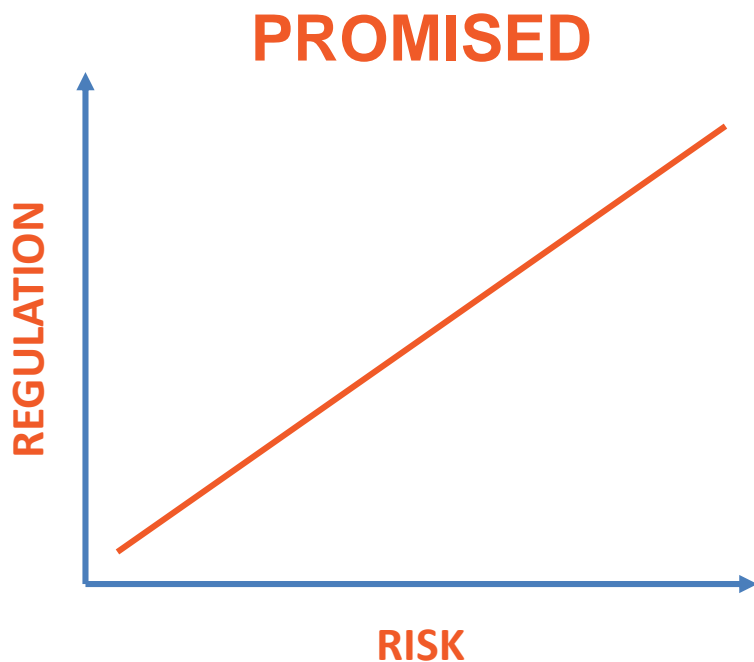
# Promised vs. Delivered

A progressive approach was promised,  
where regulation scaled with risk



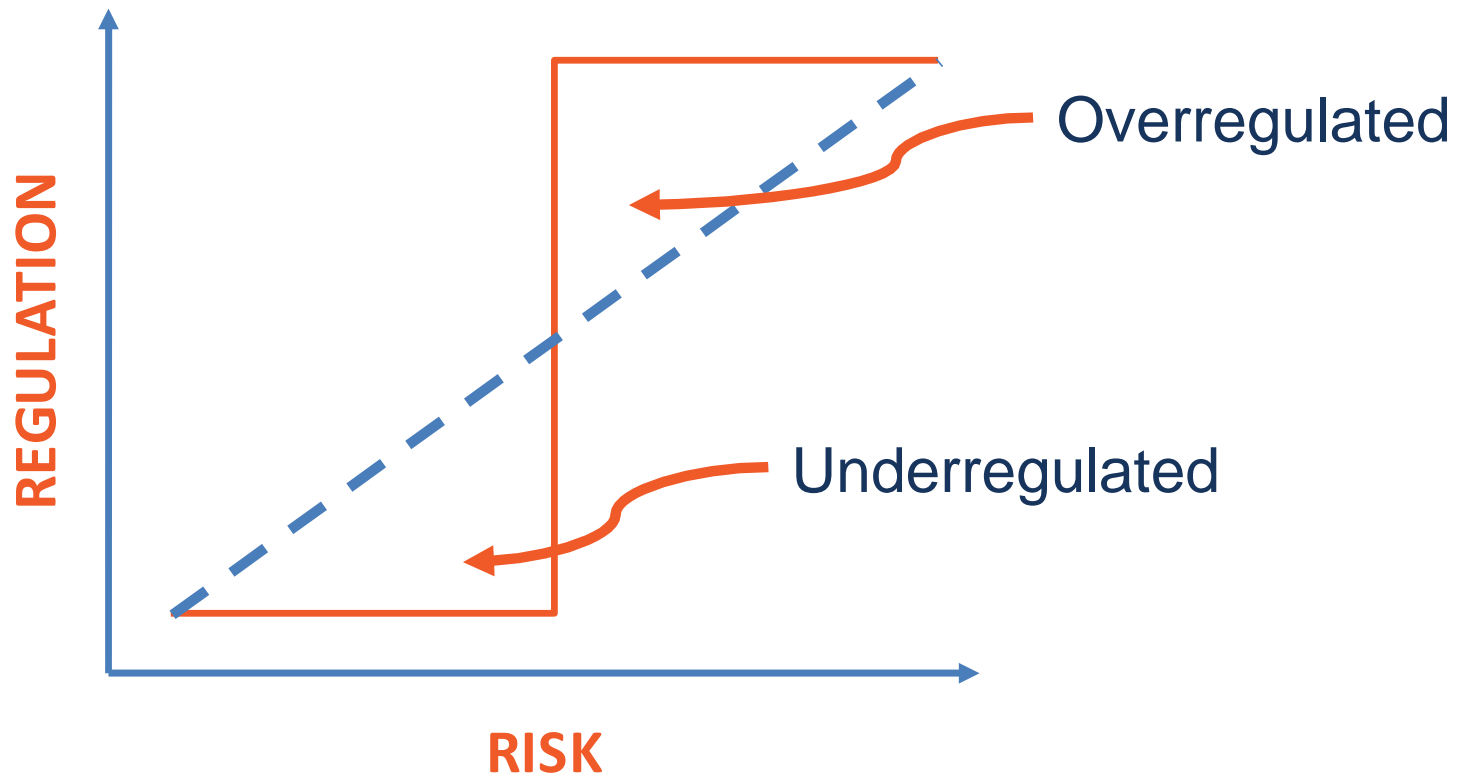
# Promised vs. **Delivered**

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



# This is A Real Problem

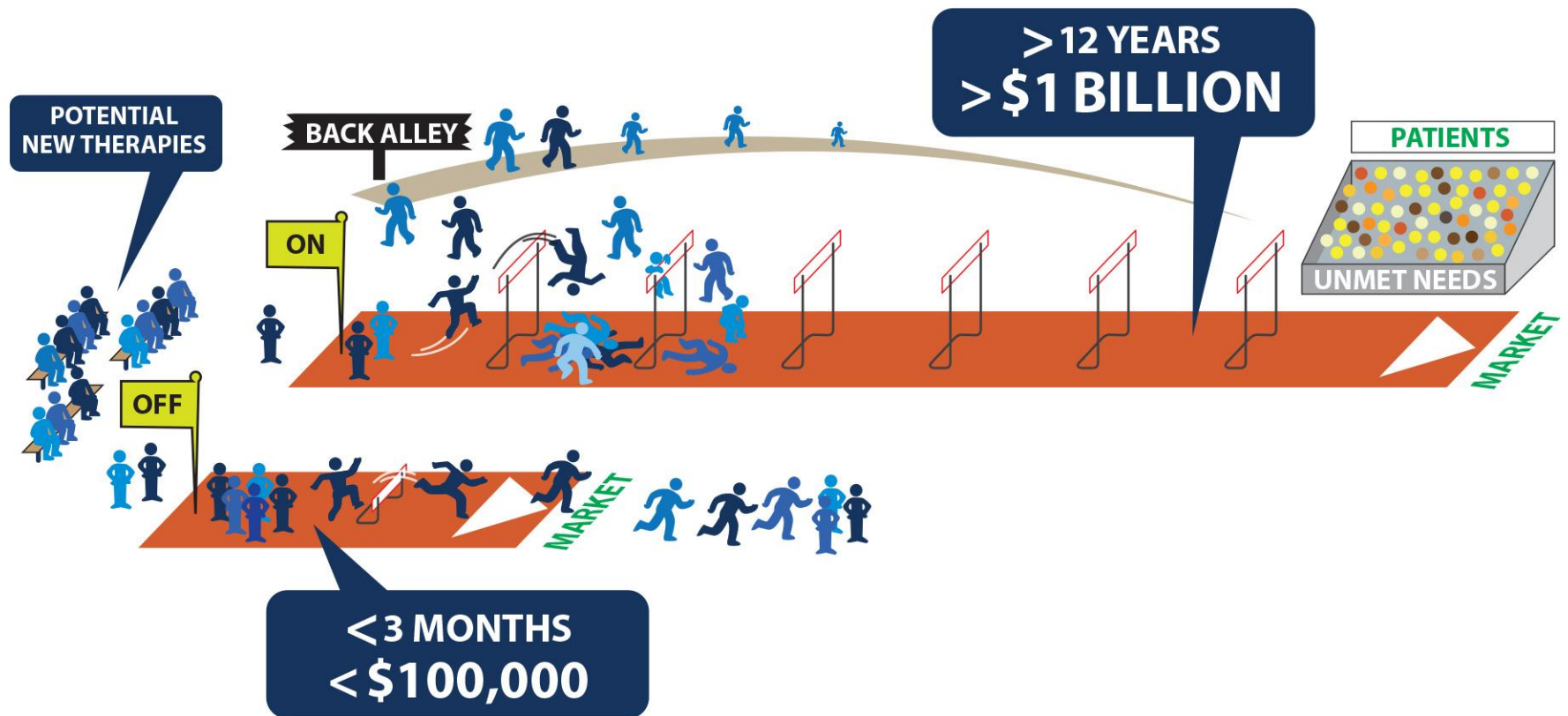
It leads to both  
**Under** and **Over** regulation





# 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**

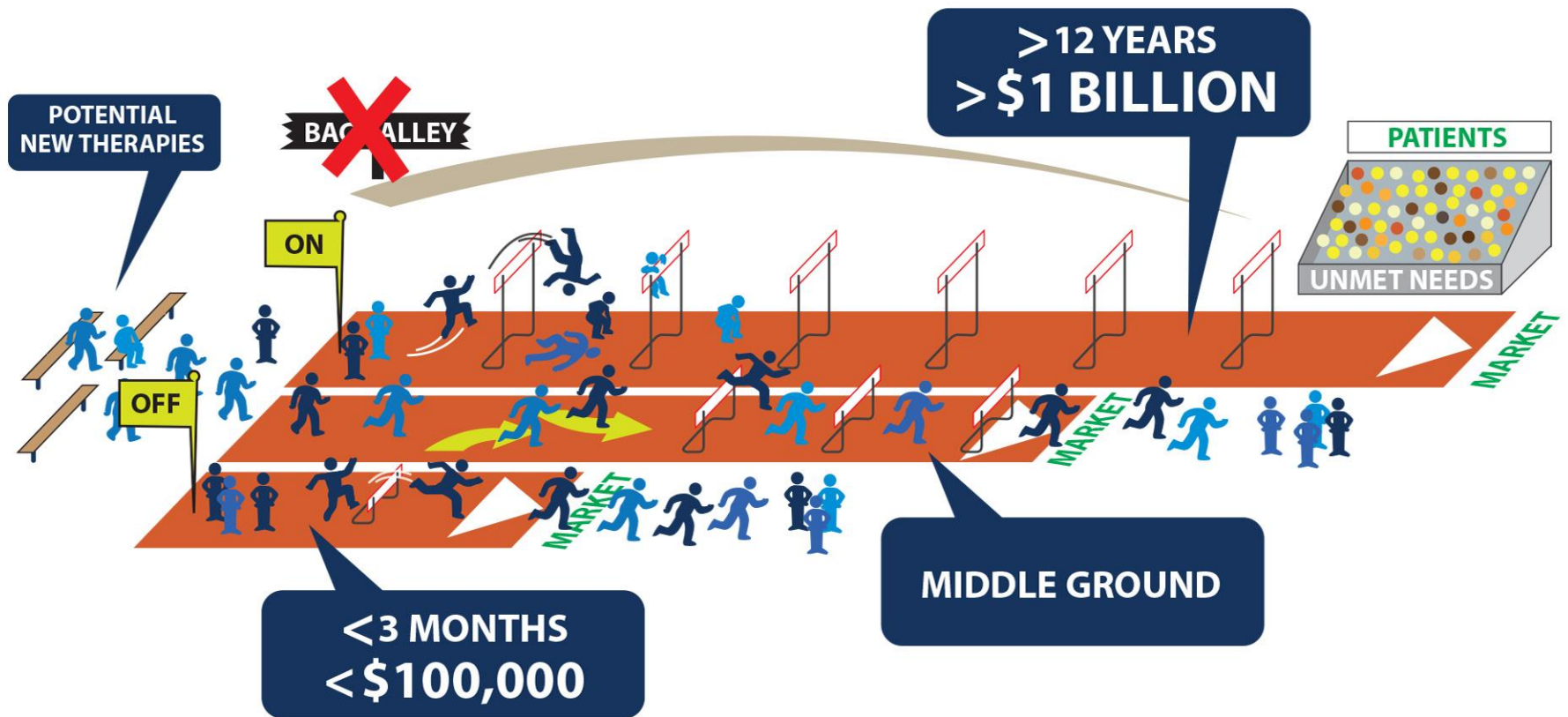
By having a system that approves nothing after 15 years, we are neither protecting nor helping those in need

# 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**