Elevator Pitch & FAQ

This document is not meant to be prescriptive. It offers sample language to help you navigate conversations about CIRM and regenerative medicine with less scientifically knowledgeable audiences. A separate document contains proof points in the form of specific numbers we can use to tell CIRM's story. The messaging guide offers additional language for talking about aspects of CIRM with various audiences, and for use in emails or other written materials. You can mix and match language from these documents, and edit to suit your needs.

Short descriptions of CIRM

Unknown audience in CA

Short: CIRM is a state agency that funds biomedical research with the goal of generating new therapies.

Longer: CIRM is a state agency created by the people of California to advance scientific research with the goal of generating new therapies. You might remember that in 2004 there was a proposition to fund stem cell research in the state. That's how we were created. In 2020 another proposition extended our funding to include gene therapy, and to include a focus on neurological diseases. We fund science, train students to work in regenerative medicine, and help Californians all over the state access our clinical trials.

Notes: For an unknown audience the emphasis should be that we are a California agency focused on cures. Follow up questions and conversation will allow you to get into the fact that we specifically fund cell and gene therapies, support clinical trials, and other aspects of our work. You can also start this with "I work for a state agency that…" or "We are a state agency that…" depending on the context.

Maybe science audience

CIRM is a state agency created by the people of California to advance stem cell and gene therapy research with the goal of generating new therapies. etc…

Notes: This slight alteration assumes the audience knows something about biomedicine, but you aren't sure how much. The focus is the same, but we're including "cell and gene therapy," which might be relevant for this audience.

Certainly a science audience

CIRM is a California agency that funds cell and gene therapies from discovery research through clinical trials with the goal of generating new therapies. Etc…

Notes: This version assumes the audience knows biomedicine and understands research funding and something about therapy development. You take it from here.

Follow up questions

What's regenerative medicine?

Regenerative medicine aims to fix the root cause of a disease. It can involve either cell or gene therapy, or a combination of the two, with the ultimate goal of fixing defective genes or cells that are the underlying cause of a disease.

What's cell and gene therapy?

Cell therapy means transferring a specific type of cell into a person to treat or prevent a disease. These cells can come from the patient (autologous) or from another source (allogeneic).

Gene therapy involves adding a working copy of a gene or fixing a faulty gene in a person's cells so that the body can make the correct protein in the correct amounts.

Gene-modified cell therapy is a combination of both cell therapy and gene therapy approaches. It involves editing a cell's genes in the lab, and then transferring the corrected cells to the patient.

Collectively, cell and gene therapies are also known as regenerative medicine.

Are you like the NIH?

Kind of. Like the NIH we fund research taking place in university labs, research institutes, and in industry. But unlike the NIH we only fund research in California, we only fund cell and gene therapy research, and we have a specific focus on funding research and clinical trials with a high likelihood of leading to cures.

Do you work in a lab?

No, we don't operate labs ourselves. We are a funding agency. We support research going on in labs at universities, research institutes, and companies throughout California. We also support students who work in those labs.

What do you mean you put therapies within reach of people in CA?

Most clinical trials take place at major medical centers, far from where many people live. The logistics of getting to clinical trial sites, staying overnight, or even hearing about relevant clinical trials can prevent people from participating. We have programs that bring clinical trials to where people are, and that provide financial support to people in California who are participating in clinical trials.

Aren't those kinds of therapies expensive?

They are, and that can be a barrier. We don't have an answer for that problem… yet. We are working with insurers and policy makers to find solutions for reimbursement challenges and to encourage them to make sure cell and gene therapies are covered in the state.

Cures are great, but is that really what state funds should support?

We think so. First of all, diseases cost more than finding a cure. People with debilitating diseases often can't work, have high healthcare needs, and require care from family or relatives who are then also unable to work. That all costs the state tax dollars. In addition, according to our most recent economic impact report, since our founding in 2004 CIRM investment has resulted in more than 50 new companies, created tens of thousands of jobs, and brought in millions in local and state tax revenues. Our research funding benefits the state directly, in addition to helping patients.

20 years, no cures?

At the best of times, therapies take 10-15 years from discovery to FDA approval, and that's when people are working in established fields of science. In 2004, when CIRM was first approved by voters, scientists barely knew how to work with stem cells in a lab and gene therapy was essentially at a standstill. Since then, the field has advanced to a point where late-stage clinical trials are already benefitting patients with some diseases and we expect some of our programs to be approved for patients this year. Given where the field was when we started, 20 years is pretty fast.

Words, terms, and definitions

The following language is intended to help navigate conversations with non-scientists. It can also help avoid jargon in written communications. Avoid defining jargon then continuing to use the jargon word. Instead, replace every instance of jargon with natural language.

Regulatory terms

Stages of Therapy Development

Discovery. The discovery stage focuses on generating new scientific knowledge and identifying promising biological targets or molecules. In this phase, researchers seek to understand the fundamentals of diseases, uncover how biological systems work, and screen for compounds or interventions that might influence these systems in beneficial

ways. Activities often include genetic studies, laboratory modeling, target identification, and the early development of potential therapies before proceeding to preclinical testing.

Preclinical. Preclinical research bridges the gap between basic scientific discoveries and their application in clinical settings. This stage evaluates how to take what is learned in discovery (such as a new molecule or biological insight) and develop it into real-world interventions—drugs, devices, diagnostics, or protocols. It involves rigorous testing in the laboratory and often in animal models, refinement of candidates, and preparations for human study. The goal is to ensure that interventions are effective and reasonably safe enough to proceed to clinical trials.

Clinical. The clinical stage is where potential interventions are tested in humans through clinical trials. Clinical trials determine whether a new potential treatment works in people and is safe and effective. Trials unfold in phases, beginning with safety assessments in small groups (phase 1), then expanding to larger populations (phase 2), to large trials (phase 3) that are rigorously evaluate effectiveness, side effects, and ultimately provide data for regulatory approval and, if successful, routine use in patients.

Investigational New Drug Application (IND)

An IND is an application to the FDA asking permission to begin testing the proposed therapy in humans. The IND generally contains extensive information from **preclinical research** that includes mechanism of action for the proposed therapy, evidence that it is safe and effective in animals, evidence that they can manufacture the therapy in a rigorous, consistent manner so all patients would receive exactly the same therapy, and other information.

Biologics License Application (BLA)

A BLA is an official request submitted to the FDA asking for permission to provide a new biological product, such as a cell or gene therapy, directly to patients. The application must include information about the manufacturer, the quality of the product, and data from laboratory and clinical studies. Once the FDA approves a BLA, a therapy can be made available to patients.

Regenerative Medicine Advanced Therapy (RMAT) designation

The FDA designates some proposed therapies in clinical trial to be a Regenerative Medicine Advanced Therapy. This designation is awarded to promising regenerative medicine therapies being tested in clinical trials for serious or life-threatening diseases. The designation can help a therapy developer (often, but not always, a company) get through FDA approvals and to patients more quickly.

Biology terms

Biomarker

Something doctors or scientists can test for — in blood, tissue, or other samples — to understand health, disease, or how well a treatment is working. For example, your cholesterol level is a biomarker for heart disease risk. In research and medicine, biomarkers help with early detection, diagnosis, and tracking treatment progress.

Stem cell

A stem cell is a cell that has the potential to divide and become any cell of that organ system. For example, a blood stem cell can divide and become any cell of the blood system (red blood cell, immune cell, platelet, etc). Some stem cells, known as pluripotent (*many potentials*) stem cells, can become any cell in the body. When used to treat disease, scientists work with stem cells in the lab to grow them into the desired cell type, then insert those into a patient to treat the disease.

Viral vector

A virus scientists use to deliver new genetic material into a person's cells. They take a harmless virus and insert genetic material that can fix faulty genes. When inside the human body, the vector delivers the helpful genetic material to diseased cells. This technology only works for diseases caused by known gene mutations.