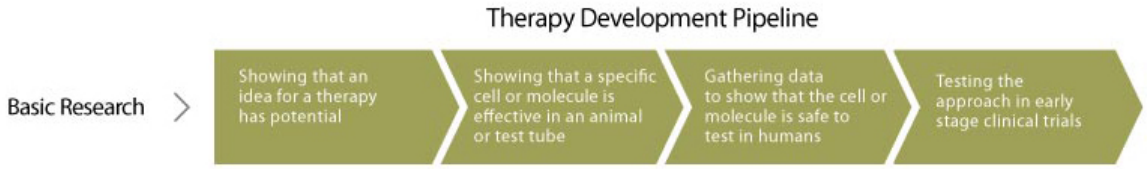
Funding therapies: Fueling hope

CIRM has projects funding all stages of translating basic discoveries into new therapies. These projects have the end goal of generating **proof of concept** for their therapeutic approach, finding a **candidate** drug or cell type to be developed into a therapy or filing an **investigational new drug (IND)** application with the Food and Drug Administration to begin clinical trials. We also fund several projects with approved clinical trials.



→ Approved clinical trial

		Proof of Concept	Development Candidate	IND	Phase I/II
Disease	***$M***	***Project Goal***			
Birth Defects					
Spina Bifida Use placental stem cells to complement the repair to the spinal canal made during fetal surgery.	$2.2		→	→	
Blood Diseases		→	→	→	→
B-Thalassemia Genetically engineer a patient's blood-forming stem cells to re-activate a fetal form of hemoglobin. Then re-inject those cells into the patient.	$6.4			→	→
Fanconi anemia Reprogram a patient's cells into induced pluripotent stem (iPS) cells and mature them into blood-forming stem cells. Then use a genetic tool to replace the defective hemoglobin gene with a normal gene.	$6.0		→		
Sickle cell disease Genetically engineer a patient's blood-forming stem cells to correct the gene that is defective in the disease. Then re-inject those cells into the patient.	$21.9			→	→
Correct the genetic defect in the patient's own blood forming stem cells then return those cells to the patient.	$1.7	→			
Hemophilia B Create reprogrammed iPS cells from the patient's own cells. Correct the gene defect in these cells, mature them into liver cells and return them to the patient.	$2.3	→			
Bone Disorders		→	→	→	→
Osteonecrosis Use a drug to make a patient's own mesenchymal stem cells better at repairing and replacing bone. The drug developed directs the stem cells to the bone surface and to form new bone.	$20			→	→
Osteoporosis Treat adult (mesenchymal) stem cells derived from bone marrow with parathyroid hormone for eventual transplantation at the site of injury.	$1.9	→			
Fractures Alter a gene that induces a person's own stem cells to repair bone in hard to heal fractures.	$6.5	→	→		

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		Project Goal			
Recruit stem cells from within the bone to heal fractures.	$5.2		→		
Starting with a patient's adult stem cells harvested from an area around blood vessels, treat them with a protein that encourages the stem cells to become bone, and seed them on a synthetic scaffold.	$5.4		→		
Cancer		→	→	→	→
Leukemia Initially tested six existing drug candidates, three small molecule drugs and three antibodies that block the ability of leukemia stem cells to survive and replicate. Now using one antibody, ROR1, in clinical trials.	$24.2		→	→	→
Testing a small molecule drug that blocks a protein called BCL-6 that cancer stem cells need in order to survive.	$3.6		→		
Testing a small molecule drug that blocks a protein called BCL-2 that cancer stem cells need in order to survive.	$3.1		→		
Malignant Melanoma Use gene modification of a patient's own cells to make them better at seeking out and destroying cancer. They plan to give the patients modified blood forming stem cells as well as modified mature T-cells.	$19.9			→	→
Brain Tumors Engineer donor neural stem cells so they carry a gene precursor of an anti-cancer drug. Those cells naturally migrate to the site of a tumor. Then inject a compound that converts the precursor drug to the active drug and kills the tumor.	$18		→	→	
Develop donor adult stem cells (mesenchymal cells) engineered to carry a gene that kills tumors. The cells naturally migrate to the site of the tumor.	$3.4		→		
Remove a patient's T cells and engineer them so that they home in on brain cancer stem cells. Re-inject those cells into the patient hoping that the T cells will identify and destroy those cells.	$5.2		→	→	
Prostate Cancer Test a drug that blocks the prostate cancer from spreading.	$4.0		→		
Solid tumor Testing small molecules that attack two different targets on cancer stem cells.	$26.9			→	→
Testing an antibody that blocks a protein on cancer stem cells, dubbed the "don't eat me signal," that inhibits the immune cells that would normally destroy a cancer cell.	$26.5			→	→
Cartilage Disorders		→	→	→	→
Arthritis (osteoarthritis) Starting with embryonic stem cells or reprogrammed stem cells (iPS cells) mature them into progenitors of cartilage and implant them into the defective joint.	$10.8		→	→	
Determine which variant of the small molecule drug PRO1 is best able to get a patient's own adult (mesenchymal) stem cells to mature into cartilage.	$8.8		→	→	
Take a patients skin cells and convert them to an embryonic-like state. Mature those cells into cartilage precursors and use those to	$1.7	→			

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repair a person's damaged joint.					
Diabetes & Complications					
Diabetes Mature embryonic stem cells into cells that are the progenitors for the pancreas cells that produce insulin. Encapsulate them in a material that will protect them from immune rejection when transplanted into patients, where they mature into the pancreatic cells lost in the disease.	$54.4				
Diabetic ulcers Place adult (mesenchymal) stem cells on a synthetic scaffold where they can grow into a layer of skin for transplantation.	$9.6				
Eye Disease					
Macular Degeneration Mature embryonic stem cells into a cell type in the eye that degrades in macular degeneration, then surgically implant those cells under the retina to replace the damaged cells	$35				
Mature reprogrammed stem cells from skin (iPS cells) into the cell type in the eye that degrades in macular degeneration.	$5.9				
Start with either reprogrammed adult cells (iPS cells) or stem cells from the eye, and mature those cells into the cell type in the eye that degrades in macular degeneration. Then engineer the cells so that they make a factor that turns down the overactive immune system that is thought to cause the disease.	$5.5				
Mature human embryonic stem cells into sheets of retinal cells that can be transplanted into the eye to treat blindness.	$4.0				
Retinitis Pigmentosa Take donor stem cells and mature them into retinal cells that can be injected into the eye	$18.9				
Cornea damage Develop a way to grow enough corneal stem cells, known as limbal stem cells, in the lab so there are enough to transplant as a possible therapy.	$2.2				
Genetic Disease					
Lysosomal storage disease Transplant neural stem cells into the brains of children who have a genetic condition that damages the neurons of their brains.	$4.3				
Heart Disease					
Heart Failure Turn embryonic stem cells into what are called cardiomyocytes, the kind of cells that can become heart muscle for direct transplantation into patients.	$19.9				
Harvest the patient's own heart stem cells, then grow them on the lab until there is sufficient quantity to inject back into the heart muscle.	$5.6				
Mature human embryonic stem cells into heart muscle, and use those cells as a patch to repair damage after a heart attack.	$4.4				
Develop a way of reprogramming heart cells directly into functional heart muscle as to repair damage after a heart attack.	$6.3				
Isolate mesenchymal stem cells from bone marrow, and transplant those on a scaffold to repair damage after a heart attack.	$4.6				
Mature human embryonic stem cells into heart muscle, and modify	$1.9				

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those cells so they won't be rejected by the immune system when transplanted into a damaged heart.					
Take heart stem cells from donors and grow them in a lab, then inject those cells into the heart in people who have had heart attacks.	$19.8				
Long QT syndrome Generate reprogrammed iPS cells from a patient. Mature those into heart cells and use those cells to test drugs to treat the condition.	$6.3				
Danon disease Take skin cells from a person with Danon disease and reprogram them into embryonic-like cells. Mature those into heart cells, and use those cells to screen for drugs that treat the disease.	$1.7				
HIV/AIDS					
Remove some of the patient's blood-forming stem cells and genetically modify them using a technology called siRNA to remove a protein from the cell surface that the HIV virus uses to enter the cell. Re-inject those cells hoping the patient will develop mature blood cells resistant to HIV infection.	$18.1				
Modify the patient's immune cells to directly kill cells infected by HIV.	$4.9				
Remove some of the patient's blood-forming stem cells and genetically modify them using a technology known as zinc fingers to alter a protein the virus needs to use to enter a cell making the cells immune to HIV.	$20.1				
Remove some of patients' blood-forming stem cells and genetically engineering them to carry multiple genes that help cells resist infection by HIV.	$3.1				
Immune Disease					
SCID Replace SCID patients' dysfunctional immune cells with healthy ones using a safer form of bone marrow transplant. Use an antibody to remove the bad immune cells instead of the dangerous chemotherapy and radiation used today.	$19.1				
Work to improve systems for correcting the defective gene in patients with the SCID to repair their dysfunctional immune system.	$1				
SCID-A Remove some of the patient's blood-forming stem cells and modify them to produce a protein that's missing in people with this disease. Reintroduce those cells so that the patient now has the missing protein.	$3.9				
Liver Disease					
Liver Failure Mature embryonic stem cells into liver cells and transplant those into the diseased liver.	$4.2				
Generate reprogrammed iPS cells from a patient with genetic liver disease. Correct the mutated gene in these patients, mature the cells into liver cells and transplant those cells back into the patient.	$1.8				
Convert skin, blood or fat cells into liver precursors and transplant those into people with liver failure.	$1.5				
Metabolic disease Take stem cells from the placenta that have liver function and use those cells to treat metabolic diseases of the liver in	$1.8				

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children.					
Muscle Disease					
Muscular Dystrophy Reprogram skin cells from the patient into induced pluripotent stem (iPS) cells, then genetically modify them to correct the defective gene. Then mature corrected cells into skeletal muscle precursor cells that can be transplanted.	$1.8				
Reprogram adult cells to become iPS cells and correct the gene mutation in those cells. Return those cells to the damaged muscle.	$2.2				
Repair limb girdle muscular dystrophy by reprogramming adult cells to become iPS cells and correct the gene mutation in those cells.	$2.3				
Age related muscular atrophy Remove muscle stem cells from older people who are losing muscle strength. Multiply those cells in the lab, then reinject them into a patient's muscles.	$1.8				
Incontinence Take skin cells from a person with incontinence and mature those into the smooth muscles that make up the bladder. Transplant those cells back into the person.	$5.0				
Neurological Disorders					
ALS (Lou Gehrig's Disease) Genetically modify nerve stem cells so that they produce a protein that can protect them from the cause of ALS after transplant as well as protect any remaining undamaged cells.	$17.0				
Mature human embryonic stem cells into the precursor of a cell that protects motor neurons and inject those into patients.	$9.8				
Take skin cells from people with ALS and convert them to an embryonic-like state. Mature those cells into neurons and use them to screen for drugs that treat signs of ALS in the cells.	$1.5				
Find drugs that improve ALS symptoms in iPS cells generated from patients with the disease.	$2.3				
Alzheimer's Disease Mature embryonic stem cells into three types of neural stem cells--either just the natural cells or cells modified to make them better able to mature into neurons or destroy proteins associated with Alzheimer's disease. Test those cells in animal models of the disease.	$3.6				
Take skin cells from people with a hereditary form of Alzheimer's disease and convert them into an embryonic-like state. Mature those cells into neurons and use them to screen for drugs that treat signs of Alzheimer's in the cells.	$1.8				
Mature human embryonic stem cells into neurons, and use those cells to find drugs that encourage the development of new neurons and protect the existing neurons.	$3.4				
Inject neural stem cells into a region of the brain that is affected by Alzheimer's disease	$8.9				
Autism Create reprogrammed stem cells (iPS) from people with autism, then mature those into neurons and test drugs that alleviate symptoms.	$3.1				
Canavan Disease Create reprogrammed stem cells (iPS cells), then mature them into intermediate neural stem cells and genetically	$1.7				

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modify them to correct the inherited defect.					
Epilepsy Mature embryonic stem cells into the very specific type of nerve cell found in the part of the brain thought to be malfunctioning in epilepsy.	$1.6	→			
Huntington's Disease Use donor mesenchymal stem cells to deliver a growth factor to patients' damaged and endangered nerves. The growth factor is called BDNF.	$17.9				→
Generate reprogrammed iPS cells from people with Huntington's disease. Mature those into the cell type damaged in the disease and screen for drugs to prevent that damage.	$0.6	→			
Genetically modified bone marrow stem cell (mesenchymal) to turn off the mutated Huntington's gene in preparation for eventual injection into the brain.	$2.8		→		
Maturing embryonic stem cells into neural stem cells with the goal of eventual transplantation into the brain.	$8.8		→	→	
Multiple Sclerosis Mature human embryonic stem cells into neural progenitor cells and use these cells to treat people with MS.	$4.8		→		
Develop a drug that promotes a patient's neural precursor cells to develop into the insulating sheath that is lost in people with MS.	$4.3		→		
Parkinson's Disease Test the effectiveness of different types of stem cells including neuronal and cells derived from embryonic stem cells in an animal model of Parkinson's disease.	$5.2		→		
Create reprogrammed stem cells (iPS) from people with various forms of Parkinson's disease and mature those cells into the neuronal type that degenerates in the disease. Then test drugs on those cells in the lab to find candidates that alleviate symptoms.	$3.7	→			
Mature embryonic stem cells into the type of neuron that degenerates in Parkinson's disease and develop ways of creating enough of those cells to be therapeutically useful.	$5.0		→		
Create reprogrammed stem cells from people with Parkinson's Disease and mature them into neurons and the surrounding glial cells that protect neurons. Then look for drugs that impact either type of cell.	$2.5	→	→		
Spinal Cord Injury Transplant nerve stem cells to treat spinal cord injuries in the neck, unlike prior studies that have worked on injuries in the back.	$14.3		→	→	→
Find the appropriate type of human neural stem cells to implant along with a scaffold at the site of injury in people with spinal cord injury.	$4.6		→		

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Mature human embryonic stem cells into a type of neuron that blocks the effects of other neurons. Transplant these into people with spinal cord injury to reduce pain and improve bladder function.	$1.5	→			
Spinal Muscular Atrophy Create reprogrammed stem cells (iPS cells) and mature them into motor neurons. Then use a small molecule drug to coax the neurons into producing more of the protein that is deficient in the disease.	$2.4		→		
Stroke Mature embryonic stem cells into neural stem cells that would be transplanted at the site of the stroke alone or embedded in a biodegradable scaffold.	$18			→	
Mature human embryonic stem cells into cells that produce neurons. Use these cells to treat damage caused by stroke.	$2.1	→			
Trauma Mature embryonic stem cells into neural stem cells with the goal of transplanting the cells at the site of injury.	$1.5	→			
Respiratory Disorder		→	→	→	→
Airway Stenosis Use the patient's own stem and progenitor cells to seed a tissue-engineered airway scaffold which is then implanted in the patient to treat a critical narrowing and potentially life-threatening narrowing of the windpipe or trachea	$4.4			→	
Skin Disease		→	→	→	→
Skin disease (epidermolysis bullosa) Reprogram skin cells from the patient into induced pluripotent stem cells, then genetically modify them to correct the genetic defect found in the disease. Mature the cells into sheets of skin that can be grafted onto the patient.	$11			→	
Vascular Disease		→	→	→	→
Blood vessel growth Develop a way of reprogramming cells of the body directly into cells that make up blood vessels.	$2.3	→			
Limb Ischemia Genetically modify donor mesenchymal stem cells so that they secrete the growth factor called VEGF, which is known to stimulate blood vessel growth.	$12				→
Mature iPS cells into the cells that give rise to blood vessels and investigate their potential for transplantation.	$2.3	→	→		