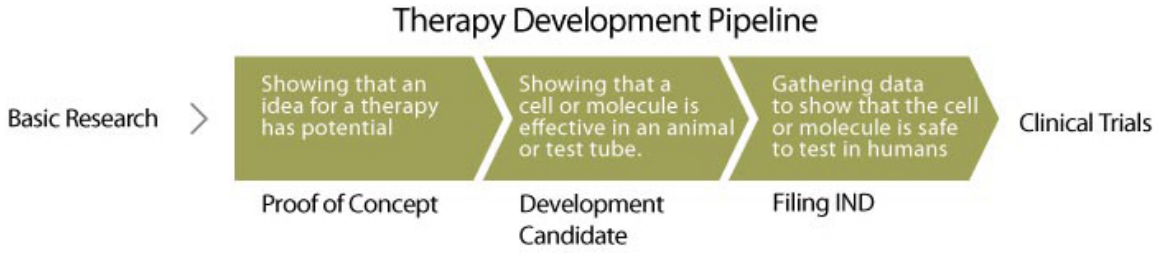
Funding therapies: Fueling hope

CIRM has 77 projects worth $620 million focusing on bringing new therapies to the clinic for 38 diseases. 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. In future years, the agency will fund increasing amounts of new therapy development in additional diseases.



Disease	$M	Proof of Concept	Development Candidate	IND	Phase1/II
		Project Goal			
Blood Diseases		→	→	→	→
B-Thalassemia Genetically engineer a patient's blood-forming stem cells to correct the gene that is defective in people with B-Thalassemia. Then re-inject those cells into the patient.	$9.4			→	→
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.	$6.6		→		
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.	$9.2			→	
Bone Disorders		→	→	→	→
Osteoporosis 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			→	→
Treat adult (mesenchymal) stem cells derived from bone marrow with parathyroid hormone for eventual transplantation at the site of injury.	$1.9	→			
Spinal fusion Starting with a patient's adult stem cells harvested from an area around blood vessels, treat them with a protein that encourage the stem cells to become bone, and seed them on a synthetic scaffold.	$5.4		→		

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Cancer		→	→	→	→
Leukemia Testing an antibody that blocks a protein on leukemia stem cells, dubbed the "don't eat me signal," that inhibits the immune cells that would normally destroy a cancer cell.	$20		→	→	
Testing six existing drug candidates, three small molecule drugs and three antibodies, that block the ability of leukemia stem cells to survive and replicate.	$20		→	→	
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.	$3.3		→		
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.	$20			→	→
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		→		
Solid tumor (Colon, Ovarian) Testing small molecules that attack two different targets on cancer stem cells.	$20		→	→	
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.	$3.1		→		
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.	$6.8		→		
Take a patients skin cells and convert them to an embryonic-like state. Mature those cells into cartilage precursors and use those to repair a person's damaged joint.	$1.7	→			
Diabetes & Complications		→	→	→	→
Diabetes Place insulin-producing cells generated from embryonic stem cells into a capsule. Place the capsule in people with diabetes who no longer produce their own insulin.	$10			→	→

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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.	$20		→	→	
Diabetic ulcers Place adult (mesenchymal) stem cells on a synthetic scaffold where they can grow into a layer of skin for transplantation.	$4.5		→		
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	$15.9		→	→	
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		→		
Retinitis Pigmentosa Create the specific cells that give rise to the retina starting with donor neural stem cells, with the eventual hope of replacing the damaged retina.	$3.9		→		
Take donor stem cells and mature them into retinal cells that can be injected into the eye	$17.3			→	→
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.	$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.	$20		→	→	
Remove some of the patient's blood-forming stem cells and genetically modify them with a technology called zinc fingers 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.	$14.6		→	→	
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		→		
Multiple Diseases		→	→	→	→
Multiple diseases Alter a gene that induces a person's own stem cells to repair tissue so that it is active in stem cells in a sustained way over time.	$5.8		→		

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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.	$2.3	→			
Generate a drug to correct the altered dystrophin protein that results in the disease symptoms	$6		→	→	
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.	$5.2		→		
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.	$17.8			→	→
Mature human embryonic stem cells into the precursor of a cell that protects motor neurons and inject those into patients.	$10.9			→	
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.	$1.7	→			
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.	$1.9	→			
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.	$1.7	→			
Inject neural stem cells into a region of the brain that is affected by Alzheimer's disease	$20		→	→	
Autism Create reprogrammed stem cells (iPS) from people with autism, then mature those into neurons and test drugs that alleviate symptoms.	$1.5	→			
Canavan Disease Create reprogrammed stem cells (iPS cells), then mature them into intermediate neural stem cells and genetically modify them to correct the inherited defect.	$1.7	→			
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.	$1.7	→			
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.	$18.9			→	→

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Huntington's Disease 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.	$3.8		→		
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.	$3.6		→		
Create reprogrammed stem cells (iPS) from people with 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.	$2.3	→			
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.	$6.0		→		
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.	$20			→	
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.	$4.7		→		
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.	$1.6	→			
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.	$5.7		→		
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.	$20			→	
Trauma Mature embryonic stem cells into neural stem cells with the goal of transplanting the cells at the site of injury.	$1.7	→			
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.	$20			→	
Harvest the patient's own heart stem cells, then grow them on the lab until there is sufficient quantity to inject back intro 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.	$4.8		→		
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.	$4.9		→		

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Heart Failure Mature human embryonic stem cells into heart muscle, and modify those cells so they won't be rejected by the immune system when transplanted into a damaged heart.	$1.9	→			
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				→
Blood vessel growth Develop a way of reprogramming cells of the body directly into cells that make up blood vessels.	$2.3	→			
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	→			
Liver Disease		→	→	→	→
Liver Failure Mature embryonic stem cells into liver cells and transplant those into the diseased liver.	$5.2		→		
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 children.	$1.8	→			
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.	$11.7		→	→	
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.	$20				→
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		→		
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		→		
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.	$5.5		→		
Vascular Disease		→	→	→	→
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.	$14.2				→