## **CIRM Active Awards Portfolio**

Disease	Cell Type	Therapeutic or Technology	Institution	Stage	Project Objective
Immune Disease, Severe Combined Immunodeficiency, X-linked (X-SCID)	Adult Stem Cell	Antibody, Biologic, Therapeutic Approach	Stanford	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
Melanoma,Skin cancer	Adult Stem Cell	Gene Therapy (All), Gene-modified, personalized cell therapy, Therapeutic Approach	UCLA		Clinical Trial, Phase 1 or Phase 1/2
Osteonecrosis	Adult Stem Cell	Small molecule therapy, Therapeutic Approach	UC Davis	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
Sickle Cell Disease	Adult Stem Cell	Gene Therapy (All), Gene-modified, personalized cell therapy, Therapeutic Approach	UCLA	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
Age-related macular degeneration	Embryonic Stem Cell	Donor cell therapy, Other Components, Therapeutic Approach	USC	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
Solid Tumors	Cancer Stem Cell	Small molecule therapy, Therapeutic Approach	UCLA	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
Spinal Cord Injury	Embryonic Stem Cell	Donor cell therapy, Therapeutic Approach	Asterias	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
HIV/AIDS	Adult Stem Cell	Gene Therapy (All), Gene-modified, personalized cell therapy, Therapeutic Approach	СОН	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
Type 1 diabetes	Embryonic Stem Cell	Device, Donor cell therapy, Therapeutic Approach	ViaCyte	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
HIV-related Lymphoma,HIV/AIDS	Adult Stem Cell	Gene Therapy (All), Gene-modified, personalized cell therapy, Therapeutic Approach	UC Davis	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
Immune Disease,X-linked Chronic Granulomatous Disease	Adult Stem Cell	Gene Therapy (All), Gene-modified, personalized cell therapy, Therapeutic Approach	UCLA	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
Heart disease associated with Duchenne muscular dystrophy	Adult Stem Cell	Donor cell therapy, Therapeutic Approach	Capricor	Trial Phase 2	Clinical Trial, Phase 2
Kidney Failure	Adult Stem Cell	Biologic, Therapeutic Approach	Humacyte	Trial Phase 3	Clinical Trial, Phase 3
Pulmonary Hypertension,Vascular Disease	Adult Stem Cell	Donor cell therapy, Therapeutic Approach	Cedars-Sinai	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
Colon Cancer	Cancer Stem Cell	Antibody, Biologic, Therapeutic Approach	Forty Seven	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
Retinitis Pigmentosa	Adult Stem Cell	Donor cell therapy, Therapeutic Approach	jCyte	Trial Phase 2	Clinical Trial, Phase 2
Kidney Failure	Adult Stem Cell	Donor cell therapy, Therapeutic Approach	Stanford	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
Type 1 diabetes	Adult Stem Cell	Personalized cell therapy, Therapeutic Approach	Caladrius Biosciences	Trial Phase 2	Clinical Trial, Phase 2
Amyotrophic Lateral Sclerosis	Adult Stem Cell	Gene Therapy (All), Gene-modified, donor cell therapy, Therapeutic Approach	Cedars-Sinai	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
Immune Disease,Severe Combined Immunodeficiency, X-linked (X-SCID)	Adult Stem Cell	Gene Therapy (All), Gene-modified, personalized cell therapy, Therapeutic Approach	St. Jude	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
Stroke	Adult Stem Cell	Gene Therapy (All), Gene-modified, donor cell therapy, Therapeutic Approach	SanBio, Inc.	Trial Phase 2	Clinical Trial, Phase 2

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Alpha Thalassemia Major	Adult Stem Cell	Donor cell therapy, Therapeutic Approach	UCSF	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
Amyotrophic Lateral Sclerosis	Adult Stem Cell	Gene Therapy (All), Gene-modified, personalized cell therapy, Therapeutic Approach	BrainStorm	Trial Phase 3	Clinical Trial, Phase 3
Blood Cancer	Adult Stem Cell	Donor cell therapy, Therapeutic Approach	Angiocrine	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
B cell cancers,Leukemia	Cancer Stem Cell	Antibody, Biologic, Therapeutic Approach	UCSD	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
Leukemia, Acute Myeloid (AML)	Adult Stem Cell	Donor cell therapy, Therapeutic Approach	Nohla Therapeutics	Trial Phase 2	Clinical Trial, Phase 2
Type 1 diabetes	Embryonic Stem Cell	Device, Donor cell therapy, Therapeutic Approach	ViaCyte	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
Kidney Failure	Adult Stem Cell	Biologic, Therapeutic Approach	Humacyte	Trial Phase 3	Clinical Trial, Phase 3
Leukemia, Acute Myeloid (AML)	Cancer Stem Cell	Antibody, Biologic, Therapeutic Approach	Forty Seven	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
Brain Cancer	Adult Stem Cell	Gene Therapy (All), Gene-modified, personalized cell therapy, Therapeutic Approach	сон	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
Arthritis,Osteoarthritis	Adult Stem Cell	Small molecule therapy, Therapeutic Approach	Calibr	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
Blood Cancer,Bone Marrow Transplant and Viral Infection	Adult Stem Cell	Donor cell therapy, Therapeutic Approach		Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
Multiple Myeloma	Adult Stem Cell	Gene Therapy (All), Gene-modified, personalized cell therapy, Therapeutic Approach	Poseida Therapeutics, Inc.	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
Kidney Failure	Adult Stem Cell	Donor cell therapy, Therapeutic Approach	Medeor Therapeutics	Trial Phase 3	Clinical Trial, Phase 3
Sickle Cell Disease	Adult Stem Cell	Donor cell therapy, Therapeutic Approach	сон	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
Severe Combined Immunodeficiency, Artemis deficient (ART-SCID)	Adult Stem Cell	Gene Therapy (All), Gene-modified, personalized cell therapy, Therapeutic Approach	UCSF	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
B cell cancers,Leukemia	Adult Stem Cell	Gene Therapy (All), Gene-modified, personalized cell therapy, Therapeutic Approach	Stanford	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
Beta Thalassemia	Adult Stem Cell	Gene Therapy (All), Gene-modified, personalized cell therapy, Therapeutic Approach	T T	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2
Immunodeficiency, Adenosine deaminase-deficient (ADA-SCID)	Adult Stem Cell	Gene Therapy (All), Gene-modified, personalized cell therapy, Therapeutic Approach	Orchard Therapeutics Limited	Trial Phase 2	Clinical Trial, Phase 2
Lung Cancer	Adult Stem Cell	Antibody, Biologic, Gene Therapy (All), Gene-modified, personalized cell therapy, Therapeutic Approach	UCLA	Trial Phase 1	Clinical Trial, Phase 1 or Phase 1/2