

Clinical-Stage Programs by Disease Area

C I R M

Modality Details	Indication	Prevalence	Partnering Status	Phase 1	Phase 2	Phase 3	Add'l Phase 3	BLA Filed
Bone & Cartilage								
AAV gene therapy	Knee Osteoarthritis	Non-Rare	Venture-Backed Company					
Cancer								
AAV gene therapy	Glioma	Rare	Venture-Backed Company	#				
allogeneic NSC-based OV retrovirus	Glioma	Rare	Outlicensed Public					
	Glioma	Rare	Unpartnered Academic	#				
autologous CAR-T	Glioblastoma	Rare	Unpartnered Academic					
autologous CAR-T	Glioblastoma	Rare	Unpartnered Academic					
autologous CAR-T	DIPG, SDMG	Ultra-Rare	Unpartnered Academic					
autologous CAR-T	Malignant Pediatric Brain Tumors	Ultra-Rare	Unpartnered Academic					
allogeneic CAR-NK	AML, Myelodysplastic Syndrome	Rare	Public Company					
allogeneic CAR-T	r/r AML	Rare	Unpartnered Academic					
autologous CAR-T	Hepatoblastoma & Hepatocellular Carcinoma	Rare	Venture-Backed Company	#				
allogeneic CAR-T	ccRCC	Non-Rare	Public Company					
autologous CAR-T	Melanoma	Non-Rare	Unpartnered Academic					
Cardiovascular								
allogeneic cell therapy	DMD Cardiomyopathy	Rare	Public Company		★			✓
AAV gene therapy	PKP2-ARVC	Rare	Public Company					
allogeneic PSC-derived CM	Chronic iLVD	Non-Rare	Unpartnered Academic					
Hematology								
autologous CAR-T	AL Amyloidosis	Rare	Public Company	# ★				
biologic (antibody)	SCID	Ultra-Rare	Public Company	#				
autologous GM HSC	Sickle Cell Disease	Rare	Outlicensed Nonprofit		★			
autologous GM HSC	ADA-SCID	Ultra-Rare	Outlicensed Seed	# ★				
autologous GM HSC	Art-SCID	Ultra-Rare	Unpartnered Academic	# ★				
allogeneic PSC-derived CAR-T	SLE, Lupus Nephritis	Non-Rare	Public Company					
autologous GM HSC	Sickle Cell Disease	Rare	Unpartnered Academic	#				
allogeneic depleted HSC + conditioning Ab	Fanconi Anemia	Ultra-Rare	Unpartnered Academic	#				
allogeneic Treg-like	GvHD	Rare	Venture-Backed Company					
autologous GM T cells	IPEX Syndrome	Ultra-Rare	Unpartnered Academic					
allogeneic Treg-like	Immune Reconstitution	Non-Rare	Unpartnered Academic					
allogeneic depleted HSC	Severe Aplastic Anemia	Ultra-Rare	Unpartnered Academic					
Infectious Disease								
autologous CAR-T	HIV	Non-Rare	Outlicensed Nonprofit	#				
autologous CAR-T	HIV	Non-Rare	Unpartnered Academic					
Metabolic								
autologous GM HSC	Cystinosis	Ultra-Rare	Outlicensed Public	#	#			
autologous GM B cells	MPS I	Rare	Venture-Backed Company					
Neurological Disorder								
allogeneic PSC-derived interneuron	Drug-resistant unilateral MTLE	Non-Rare	Public Company	#		★		
AAV gene therapy	Parkinson's Disease	Non-Rare	Public Company					
allogeneic PSC-derived interneuron	Drug-resistant bilateral MTLE	Non-Rare	Public Company	#				
autologous MSC	ALS	Rare	Public Company				★	
autologous PSC-derived NPC	Parkinson's Disease	Non-Rare	Venture-Backed Company	#				
allogeneic PSC-derived NPC	Parkinson's Disease	Non-Rare	Venture-Backed Company	#				
allogeneic NPC	ALS	Rare	Unpartnered Academic	#				
allogeneic MSC on matrix	Spina Bifida-Myelomeningocele	Rare	Outlicensed Seed	#				
ASO	SCN2A-related Disorders	Ultra-Rare	Unpartnered Academic					
AAV gene therapy	CMT4J	Ultra-Rare	Partnered Non-Profit	#				
AAV gene therapy	Pitt Hopkins Syndrome	Ultra-Rare	Venture-Backed Company	#				
allogeneic PSC-derived NSC	Huntington's Disease	Rare	Outlicensed Seed	#				
allogeneic CAR-Treg	Multiple Sclerosis	Non-Rare	Venture-Backed Company	#				
AAV gene therapy	FOXP1 Syndrome	Ultra-Rare	Unpartnered Non-Profit	#				
Ophthalmology								
AAV gene therapy	Retinitis Pigmentosa	Rare	Venture-Backed Company					
allogeneic NPC	Retinitis Pigmentosa	Rare	Unpartnered Academic	#				
allogeneic PSC-derived RPE	Geographic Atrophy	Non-Rare	Venture-Backed Company					
allogeneic RPESC-RPE	dry AMD	Non-Rare	Seed Stage Company	#				
autologous PSC-derived RPE	AMD: RPE Atrophy	Non-Rare	Seed Stage Company					
autologous limbal SC	Limbal Stem Cell Deficiency	Non-Rare	Unpartnered Academic					
Other								
biologic (acellular vessel)	Dialysis Access	Non-Rare	Public Company				★	
AAV gene therapy	Danon Disease	Ultra-Rare	Public Company		★			
autologous muscle precursor	Dysphagia	Non-Rare	Outlicensed Venture-Backed	#				
allogeneic HLA-matched HSC + kidney	End Stage Kidney Disease	Non-Rare	Seed Stage Company	#				
allogeneic HSC + kidney	End Stage Kidney Disease	Rare	Unpartnered Academic	#				
allogeneic HLA-mismatched HSC + kidney	End Stage Kidney Disease	Non-Rare	Unpartnered Academic					
allogeneic HLA-mismatched HSC + kidney + Treg	End Stage Kidney Disease	Non-Rare	Unpartnered Academic					

CIRM-funded Externally funded

Prevalence: Rare disease is defined as a disease with under 200K patients in the US; ultra-rare disease is defined as under 10K patients in the US.

= Phase 1/2 Trial; ★ = Pivotal Trial

AAV: adeno-associated virus; NSC: neural stem cell; OV: oncolytic virus; CAR: chimeric antigen receptor; NK: natural killer; PSC: pluripotent stem cell; CM: cardiomyocyte; GM: gene-modified; HSC: hematopoietic stem cell; Ab: antibody; Treg: regulatory T cell; MSC: mesenchymal stem cell; NPC: neural progenitor cell; ASO: anti-sense oligonucleotide; RPE: retinal pigment epithelium; RPESC: retinal pigment epithelial stem cell; SC: stem cell; HLA: human leukocyte antigen; DIPG: diffuse intrinsic pontine glioma; SDMG: spinal diffuse midline glioma; AML: acute myeloid leukemia; r/r: relapsed/refractory; ccRCC: clear cell renal cell carcinoma; DMD: Duchenne muscular dystrophy; PKP2-ARVC: PKP2 mutation-associated arrhythmogenic right ventricular cardiomyopathy; iLVD: ischemic left ventricular dysfunction; SCID: severe combined immunodeficiency; ADA-SCID: adenosine deaminase severe combined immunodeficiency; Art-SCID: Artemis-deficient severe combined immunodeficiency; SLE: systemic lupus erythematosus; GvHD: graft vs host disease; IPEX: immunodysregulation polyendocrinopathy enteropathy X-linked; HIV: human immunodeficiency virus; MPS I: mucopolysaccharidosis type I; MTLE: mesial temporal lobe epilepsy; ALS: amyotrophic lateral sclerosis; SCN2A: sodium voltage-gated channel alpha subunit 2; CMT4J: Charcot Marie tooth disease type 4J; FOXG1: forkhead box G1; AMD: age-related macular degeneration

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Allogeneic								
allogeneic cell therapy	DMD Cardiomyopathy	Rare	Public Company			★		✓
allogeneic PSC-derived interneuron	Drug-resistant unilateral MTLE	Non-Rare	Public Company	#		★		
allogeneic PSC-derived interneuron	Drug-resistant bilateral MTLE	Non-Rare	Public Company	#				
allogeneic PSC-derived NPC	Parkinson's Disease	Non-Rare	Venture-Backed Company	#				
allogeneic NPC	ALS	Rare	Unpartnered Academic	#				
allogeneic NPC	Retinitis Pigmentosa	Rare	Unpartnered Academic	#				
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allogeneic HLA-mismatched HSC + kidney	End Stage Kidney Disease	Non-Rare	Unpartnered Academic					
allogeneic HLA-mismatched HSC + kidney + Treg	End Stage Kidney Disease	Non-Rare	Unpartnered Academic					
Allogeneic, Gene-Modified								
allogeneic CAR-T	ccRCC	Non-Rare	Public Company					
allogeneic CAR-T	r/r AML	Rare	Unpartnered Academic					
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Genetic Therapy								
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AAV gene therapy	FOXG1 Syndrome	Ultra-Rare	Unpartnered Non-Profit	#				
AAV gene therapy	Glioma	Rare	Venture-Backed Company	#				
retrovirus	Glioma	Rare	Unpartnered Academic	#				
ASO	SCN2A-related Disorders	Ultra-Rare	Unpartnered Academic					
Biologic (Excluding Genetic Therapy)								
biologic (acellular vessel)	Dialysis Access	Non-Rare	Public Company				★	
biologic (antibody)	SCID	Ultra-Rare	Public Company	#				

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