current portfolio as of 051213

White = CIRM Funded

Dark Gray = Pending ICOC Vote, GWG Recommended

Light Gray = Pending ICOC Vote, Not GWG Recommended

CURRENT CIRM TRANSLATION PORTFOLIO

AWARD # PI, Institution	PROGRAM	GOAL*	DISEASE/INJURY	APPROACH
CANCER: HEMATOLOGIC MALIGNANCY				
DR1-01430 Carson, UCSD	Disease Team I	IND	AML, CLL	A monoclonal antibody (anti-ROR1) and a small molecule (JAK2 inhibitor) targeting CLL and AML cancer stem cells,respectively
DR1-01485 Weissman, Stanford	Disease Team I	IND	AML	Monoclonal antibody against CD47 – "Don't eat me" antigen that is expressed on leukemia stem cells
TR2-01789 Jamieson, UCSD	Early Translation II	DC	CML	Small molecule pan BCL-2 inhibitor targeting cancer stem cells
TR2-01816 Müschen, CHLA	Early Translation II	DC	AML, ALL	Small molecule inhibitor of BCL6 targeting cancer stem cells
CANCER: SOLID TUMOR				
DR2A-05309 Ribas, UCLA	Disease Team Therapy Development	IND, Ph 1	Melanoma	Autologous HSC genetically modified to produce an anti-tumor T cell receptor and a PET reporter gene
DR1-01477 Slamon, UCLA	Disease Team I	IND	Colon, ovarian cancers, glioblastoma	Small molecules specific for each of two drug targets in cancer stem cells
DR1-01421 Aboody, City of Hope	Disease Team I	IND	Glioblastoma	Allogeneic hNSC line to target tumor, engineered ex vivo to deliver carboxylesterase to locally convert CPT-11 to more potent SN-38
TR2-01791 Kasahara, UCLA	Early Translation II	DC	Glioblastoma	Allogeneic hMSC to target tumor, engineered to produce replication competent retrovirus encoding a prodrug activator to locally convert a pro-drug to a potent chemotherapeutic
TR3-05641 Forman, Beckman Inst.	Early Translation III	DC	Glioblastoma	A mixture of autologous central memory T cells engineered to each express a chimeric antigen receptor (CAR) targeting one of three proteins on glioma-initiating cancer stem cells
NEUROLOGIC DISORDERS: INJURY				
DR1-01480 Steinberg, Stanford	Disease Team I	IND	Stroke	Allogeneic hESC-derived NSC
TR3-05628 Tuszynski, UCSD	Early Translation III	DC	Spinal Cord Injury	hESC-derived neural stem cells in a scaffold
TR3-05606 Kriegstein, UCSF	Early Translation III	DCF	Spinal Cord Injury	hESC-derived progenitors of inhibitory interneurons
TR2-01767 Cummings, UC irvine	Early Translation II	DCF	Traumatic Brain Injury	Allogeneic hESC-derived NSC
TR2-01785 Havton, UC Irvine	Early Translation II	DCF	Spinal Cord Injury (conus medullaris, cauda equina)	hESC-derived motor and autonomic precursor neurons
NEUROLOGIC DISORDERS: NEURODEGENERATIVE DISEASES				
DR2A-05320 Svendson, Cedars-Sinai	Disease Team Therapy Development	IND, Ph 1	ALS	Allogeneic neural progenitor cells genetically modified with GDNF
DR2A-05416 Capela, Stem Cells Inc.	Disease Team Therapy Development	IND	Alzheimer's Disease	Neural stem cell transplantation for neuroprotection
DR2A-05415 Wheelock, UC Davis	Disease Team Therapy Development	IND, Ph 1	Huntington's Disease	MSC genetically engineered to express BDNF
TRX-01471 Goldstein, UCSD	Early Translation	DC	ALS	hESC derived astrocyte precursor cells
TR2-01841 Thompson, UC Irvine	Early Translation II	DC	Huntington's Disease	Allogeneic hESC-derived neural stem or progenitor cells
TR1-01267 Snyder, Sanford-Burnham	Early Translation I	DC	Parkinson's Disease	The best hNSC derived from either tissue, ESC, or iPSC
TR2-01856 Zeng, Buck Inst.	Early Translation II	DC	Parkinson's Disease	Allogeneic hPSC-derived dopaminergic neurons
TR3-05603 Lane, UC Irvine	Early Translation III	DC	Autoimmune Disease / Multiple Sclerosis	Human pluripotent stem cell-derived neural progenitor cells
TR3-05617 Schultz, Scripps	Early Translation III	DC	Autoimmune Disease / Multiple Sclerosis	Small molecule that acts on oligodendrocyte precursors in the CNS to induce differentiation to oligodendrocytes to stimulate remyelination
TR3-05676 Yeo, UCSD	Early Translation III	DCF	ALS	Small molecule that corrects proposed aberrant RNA "signature" in iPSC- derived neurons from patients with defects in RNA processing
TR3-05577 Goldstein, UCSD	Early Translation III	DCF	Alzheimer's Disease	Small molecule identified through screens on purified hiPSC-derived brain cells from patients that have rare and aggressive hereditary forms of Alzheimer's Disease
TR3-05669 Schubert, Salk	Early Translation III	DCF	Alzheimer's Disease	Small molecule for neuroprotection & neurogenesis identified using hESC-derived neural precursors
TR2-01778 Gage, Salk	Early Translation II	DCF	Parkinson's Disease	Small molecule identified by screening in a neuron/astrocyte/microglia co-culture differentiated from patient-derived iPSCs

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NEUROLOGIC DISORDERS: PEDIATRIC				
TR2-01832 Shi, Beckman	Early Translation II	DCF	Canavan Disease	Autologous iPSC-derived neural or oligodendrocyte progenitors, genetically modifed to correct mutant aspartoacylase (ASPA) gene
TR2-01814 Muotri, UCSD	Early Translation II	DCF	Autism Spectrum Disorder (ASD)	Neurons from ASD (and control) iPSC for phenotype screening, assay development and validation, drug screening and biomarker identification
TR2-01749 Alvarez-Buylla, UCSF	Early Translation II	DCF	Refractory epilepsy	hESC-derived progenitors of inhibitory interneurons
TR3-05476 Schwartz, CHOC	Early Translation III	DC	Lysosomal Storage Disease	Immune matched human neural stem cells transplantation subsequent to hematopoietic stem cell transplantation
EYE DISORDERS				
DR2A-05739 Klassen, UC Irvine	Disease Team Therapy Development	IND, Ph 1/2	Retinitis Pigmentosa	Allogenic retinal progenitor cells
DR1-01444 Humayun, USC	Disease Team I	IND	Age-related macular degeneration (dry form)	Allogeneic functionally polarized hESC-derived RPE monolayers on synthetic substrate implanted sub-retinally
TR1-01219 Friedlander, Scripps	Early Translation I	DC	Age-related macular degeneration (dry form)	Autologous iPSC-derived RPE (generated without integrating vectors)
TR2-01768 Deng, UCLA	Early Translation II	DCF	Corneal Injury	Ex vivo expansion of corneal epithelial stem/progenitor cells, also known as limbal stem cells
HIV / AIDS				
DR1-06893 Symonds, Calimmune	Disease Team I	Ph 1/2	HIV/AIDS	Autologous HSC transduced ex vivo with a lentiviral vector engineered to express an shRNA against CCR5 & a fusion inhibitor.
DR1-01490 Zaia, City of Hope	Disease Team I	IND	AIDS Lymphoma	Autologous HSC transduced ex vivo with non-integrating vector engineered to express a zinc finger nuclease targeting CCR5
TRX-01431 Chen, UCLA	Early Translation	DC	AIDS Lymphoma	Autologous HSC transduced ex vivo with a lentiviral vector engineered to express shRNAs against CCR5 & another target in the HIV life cycle.
TR2-01771 DiGiusto, Beckman	Early Translation II	DC	AIDS Lymphoma	Autologous HSC genetically modified with multiple anti-HIV resistance genes and a drug resistance gene
DIABETES & COMPLICATIONS				
SP1-06513 Foyt, ViaCyte Inc.	Strategic Partnership I	IND, Ph 1/2	Diabetes: Type 1	Allogeneic hESC-derived pancreatic cell progenitors in a device implanted subcutaneously
DR1-01423 Brandon, ViaCyte Inc	Disease Team I	IND	Diabetes: Type 1	Allogeneic hESC-derived pancreatic cell progenitors in a device implanted subcutaneously
TR2-01787 Isseroff, UC Davis	Early Translation II	DC	Chronic Diabetic foot ulcers	Allogenic hMSC on a dermal regeneration scaffold
BLOOD DISORDERS				
SP2-06902 Recommended for Funding by GWG	**Strategic Partnership II**	**IND, Ph 1**	**β-thalassemia**	**Autologous HSC genetically modified ex vivo using a novel gene-editing technology to re-activate fetal gamma-globin expression**
SP1-06477 Davidson, Bluebird Bio	Strategic Partnership I	IND, Ph 1/2	β-thalassemia	Autologous HSC genetically modified ex viv owith lentiviral vector encoding a therapeutic form of the β-globin gene
DR2A-05365 Shizuru, Stanford	Disease Team Therapy Development	IND, Ph 1/2	Conditioning regimen for allogeneic HSC transplantation for X-SCID	MAb that depletes endogenous HSC
DR1-01452 Kohn, UCLA	Disease Team I	IND	Sickle Cell Disease	Autologous HSC, genetically corrected ex vivo by lentiviral vector mediated addition of a hemoglobin gene that blocks sickling
TR1-01273 Verma, Salk	Early Translation I	DC	Fanconi Anemia, XSCID	Autologous iPSC-derived HSC genetically corrected *ex vivo* by homologous recombination
TR3-05535 Cowan, UCSF	Early Translation III	DC	SCID-A	Autologous HSC genetically corrected ex vivo by lentiviral vector mediated delivery of the Artemis gene
BONE DISORDERS				
SP2-06906 Not Recommended for Funding by GWG	Strategic Partnership II	IND, Ph 1/2	Spinal fusion	Combination product of a ceramic scaffold and human bone marrow stromal cells that are stimulated to form bone
DR2A-05302 Lane, UC Davis	Disease Team Therapy Development	IND, Ph 1/2	Osteoporosis	Synthetic molecule, LLP2A-Ale, to enhance homing of endogenous bone marrow MSCs to bone surface
TR1-01249 Longaker, Stanford	Early Translation I	DC	Bone fractures	Recombinant lyposomal Wnt3a to stimulate endogenous stem cells to repair bone
TR2-01821 Peault, UCLA	Early Translation II	DC	Spinal fusion	Autologous adult perivascular stem cells (MSC) and an osteoinductive protein (CLL) on a FDA-approved acellular scaffold
TR2-01780 Gazit, Cedars-Sinai	Early Translation II	DCF	Osteoporosis-related vertebral compression fractures	MSC in combination with PTH (parathyroid hormone)

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CARTILAGE DISORDERS				
TR1-01216 D'Lima, Scripps	Early Translation I	DC	Focal cartilage defect, osteoarthritis	iPSC- or ESC-derived chrondrocyte progenitors implanted into chrondral defect or injected into OA joint
TR2-01829 Schultz, Scripps	Early Translation II	DC	Osteoarthritis	Optimized small molecule of lead molecule PRO1 that induces chrondrocyte differentiation of resident hMSC
TR3-05709 Athanasiou, UC Davis	Early Translation III	DCF	Articular cartilage defects	Autologous adult (dermis isolated) stem cell-derived tissue engineered product
CARDIOVASCULAR, VASCULAR DISEASE				
DR2A-05735 Smith, Capricor Inc.	Disease Team Therapy Development	Ph 2	Heart dysfunction after MI/Chronic heart failure	Allogeneic cardiac-derived stem cells
DR2A-05423 Laird, UC Davis	Disease Team Therapy Development	IND, Ph 1	Critical limb ischemia	Allogeneic MSC engineered to express VEGF delivered by intramuscular injection
DR2A-05394 Wu, Stanford	Disease Team Therapy Development	IND	End stage heart failure with LVAD	Allogeneic hESC-derived cardiomyocytes
TR3-05556 Wu, Stanford	Early Translation III	DC	Cardiovascular Disease	hESC-derived cardiomyocytes seeded in a tissue engineered patch
TR3-05593 Srivastava, Gladstone	Early Translation III	DC	Cardiovascular Disease	Direct reprogramming of endogenous cardiac fibroblasts into functional cardiomyocytes by gene transfer
TR3-05626 Boyd, UC Davis	Early Translation III	DC	Cardiovascular Disease	Allogeneic human bone marrow-derived MSCs embedded in a biological scaffold
TR3-05559 Xu, UCSD	Early Translation III	DCF	Cardiovascular Disease	hESC-derived cardiomyocytes genetically modified to evade allogeneic immune rejection
TR3-05568 Belmonte, Salk	Early Translation III	DCF	Cardiovascular, Vascular Disease	Multipotent vascular progenitors derived by direct conversion of somatic cells
TR3-05687 Adler, UCSD	Early Translation III	DCF	Cardiovascular Disease - Danon disease	Small molecule leads identified by correction of autophagy on Danon patient iPSC-derived lines
LIVER DISEASE				
TR2-01857 Zern, UC Davis	Early Translation II	DC	Liver Disease (acute liver failure and as a bridge following large liver resections)	Allogeneic genetically modified hESC-derived hepatocytes
TR3-05488 Miki, USC	Early Translation III	DCF	Liver Disease, Congenital	Human amniotic epithelial cell-derived hepatic cells
TR3-05542 Willenbring, UCSF	Early Translation III	DCF	Liver Disease, Chronic	Human induced hepatocyte-like cells
SKELETAL MUSCLE DISORDERS				
TRX-05426 Nelson, UCLA	Early Translation	DCF	Duchenne muscular dystrophy	Combination therapy of an antisense oligonucleotide that promotes exon skipping and a small molecule that enhances its efficiency
TR2-01756 Calos, Stanford	Early Translation II	DCF	Duchenne muscular dystrophy	Autologous skeletal muscle stem/precursor cells derived from human iPSC genetically modifed to correct the dystrophin gene
TR3-05501 Blau, Stanford	Early Translation III	DCF	Age-related Muscle Atrophy	Autologous human muscle stem/progenitor cells rejuvenated and expanded ex vivo using a combined bioengineering and small molecule treatment
OTHER DISORDERS				
DR1-01454 Lane, Stanford	Disease Team I	IND	Epidermolysis bullosa	Epidermal sheets from expanded autologous genetically corrected (to express wild type COL7A1) iPSC-derived keratinocytes
TR3-05569 Reijo Pera, Stanford	Early Translation III	DC	Urinary Incontinence	Autologous iPSC-derived smooth muscle precursor cells and smooth muscle cells, potentially delivered in a matrix

*** The Project Goal is:**
IND - file a complete IND with the FDA
DC - achieve a development candidate ready for IND-enabling preclinical development
DCF - show feasibility of a potential development candidate by achieving initial proof of concept