SANFORD REPORT

Monthly Newsletter of Sanford Stem Cell Clinical Center



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New Director

We are excited to announce Catriona Jamieson, MD, PhD, as the new director of the Sanford Stem Cell Clinical Center at UC San Diego Health. Dr. Jamieson served as the deputy director of the Sanford Center from 2014 to 2018. She is ideal for this position due to her years of experience in clinical trials and cancer stem cell research. She endeavors to develop programs that will accelerate the translation of stem cell-related discoveries into clinical trials and anticipates that the goals of the Sanford Center will be clinically driven over the next five years. Dr. Jamieson is also professor of medicine and chief of the Division of Regenerative Medicine at UC San Diego, director of the UC San Diego Health CIRM Alpha Stem Cell Clinic, and deputy director of the UC San Diego Moores Cancer Center.

Sally Temple, PhD

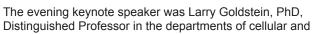
Larry Goldstein, PhD



5th Annual Division of Regenerative Medicine Symposium

The Sanford Center sponsored the UC San Diego Division of Regenerative Medicine's 5th Annual Symposium on March 15, 2019 at the Sanford Consortium for Regenerative Medicine.

Sally Temple, PhD, professor of biomedical science at Albany Medical College, was the morning keynote speaker and discussed, "Stem cells in the adult human retinal pigment epithelium and their therapeutic potential." Dr. Temple is also the co-founder and scientific director of The Neural Stem Cell Institute.



molecular medicine and neurosciences at UC San Diego. He presented, "Using stem cells to probe the secrets of Alzheimer's disease." Dr. Goldstein is also Director Emeritus of the Sanford Stem Cell Clinical Center and scientific director of the Sanford Consortium for Regenerative Medicine.

Clinical Trial Gives Patient Her Life Back

In 2006 at the age of 28, Sandra was living a healthy lifestyle and traveling the world until she was



diagnosed with myelofibrosis, a type of chronic leukemia. The disease was incurable and doctors advised managing her symptoms as her health continued to decline.

That's until 2011 when she came to UC San Diego and met Catriona Jamieson, MD, PhD. Dr. Jamieson enrolled Sandra in a clinical trial for a drug that targets cancer stem cells and within months the cancer started to regress.

Sandra is currently living a happy and normal life. She continues to share her story with others and spoke at the 5th Annual DRM Symposium on March 15, 2019.

Recent Publications



Alysson Muotri, PhD

Professor, Departments of Pediatrics, and Cellular and Molecular Medicine Co-Director, UCSD Stem Cell Program

January 2019

Setd5 haploinsufficiency alters neuronal network connectivity and leads to autistic-like behaviors in mice Nature Research



Martin Marsala, MD

Professor, Department of Anesthesiology Director, Sanford Surgical Training Center

March 2019

A scalable solution for isolating human multipotent clinicalgrade neural stem cells from ES precursors *Stem Cell Research & Therapy*

Breast Cancer Clinical Trial

UC San Diego Health and Oncternal Therapeutics, Inc. have launched a Phase Ib clinical trial to assess the safety and effectiveness of cirmtuzumab, in combination with a chemotherapy medication called paclitaxel, to treat

metastatic or locally advanced breast cancer.





Barbara Parker, MD

Rebecca Shatsky, MD

Cirmtuzumab is a novel monoclonal antibody developed at UC San Diego, with support from CIRM and the UC San Diego Health CIRM Alpha Stem Cell Clinic at the Sanford Stem Cell Clinical Center. It targets ROR-1, a cell surface protein present on tumors, and blocks the growth and survival of breast cancer cells.

Barbara Parker, MD, professor of medicine and breast oncologist, is co-principal investigator of the trial with Rebecca Shatsky, MD, assistant clinical professor of medicine, medical oncologist and breast cancer specialist at Moores Cancer Center.

For more information on the Phase Ib breast cancer clinical trial, contact the UC San Diego Health CIRM Alpha Stem Cell Clinic at 844-317-STEM (7836) or alphastemcellclinic@ucsd.edu.

Clinical Trial Portfolio

Indications	Product	Phase I	Phase II	Phase III
Cancer	UCSD/Oncternal - cirmtuzumab and paclitaxel for breast cancer		Phase lb	
	UC San Diego – cirmtuzumab for CLL		Phase I	
	UC San Diego – cirmtuzumab for CLL (extension)		Phase I	
	Oncternal – cirmtuzumab and ibrutinib for CLL/SLL/MCL			Phase Ib/II
	Fate – NK100 for solid tumors		Phase I	
	PersImmune – autologous T-cells for MDS (collection)		Phase I	
	PersImmune – autologous T-cells for MDS (treatment)		Phase I	
	Aivita – AV-GBM-1 for glioblastoma			Phase II
Neurological Degenerative, Trauma, and Brain	DiscGenics – allogeneic cell therapy for DDD		Phase I	
	Neuralstem – neural stem cells for paralysis due to SCI		Phase I	
	Asterias – AST-OPC1 for spinal cord injury			Phase I/II
	Asterias – AST-OPC1 for spinal cord injury (follow-up)			Phase I/IIa
Diabetes	ViaCyte – VC-01™ for type 1 diabetes			Phase I/II
	ViaCyte – VC-01™ for type 1 diabetes (follow-up)		Phase I	
	ViaCyte – VC-02™ for type 1 diabetes			Phase I/II
	ViaCyte – VC-02™ for type 1 diabetes (follow-up)			Phase II
Rare Diseases	UCSD/Avrobio – AVR-RD-04 for cystinosis			Phase I/II
Cardiovascular	Mesoblast – MPCs for chronic heart failure			

CIRM Grant Portfolio

Principal Investigator	Grant #	Start Year	Target Disease	Amount	
Catriona Jamieson, MD, PhD	AC1 - 07764	2015	Alpha Clinic	\$8M	
Larry Goldstein, PhD	TRAN1 - 08552	2016	ALS	\$5.6M	
Stephanie Cherqui, PhD	CLIN1 - 09230	2016	Cystinosis	\$5.2M	
Karen Christman, PhD	TRAN1 - 09814	2017	Peripheral Artery Disease	\$3.1M	
Ezra Cohen, MD	TRAN1 - 10258	2017	Cancer	\$5.8M	
Thomas Kipps, MD, PhD	CLIN2 - 10192	2017	B-Cell Cancer	\$18.3M	
Catriona Jamieson, MD, PhD	TRAN1 - 10540	2018	Acute Myeloid Leukemia	\$2.7M	
Dan Kaufman, MD, PhD	TRAN1 - 10587	2018	Acute Myeloid Leukemia	\$5.2M	
Mark Tuszynski, MD, PhD	DISC2 - 10665	2018	Spinal Cord Injury	\$2.1M	
Eric Adler, MD	DISC2 - 11131	2018	Danon Disease	\$1.4M	

Total \$57.4M



Thank You to Our Donor

Phase III

We thank our donor, Denny Sanford, for the transformational gift to establish the Sanford Stem Cell Clinical Center. This gift has led to numerous research breakthroughs, several stem cell-derived therapies, and new clinical trials treating a range of cancers and diseases.