

# SANFORD REPORT

Monthly Newsletter of Sanford Stem Cell Clinical Center

UC San Diego Health  
Sanford Stem Cell Clinical Center

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## Establishment of Fellowship in Honor of Director Emeritus

The International Society for Stem Cell Research (ISSCR) recently honored Lawrence Goldstein, PhD, Distinguished Professor in the department of cellular and molecular medicine and neurosciences at UC San Diego and Director Emeritus at Sanford Stem Cell Clinical Center, at its annual conference in Los Angeles for his years of science policy advocacy. The organization also announced a new program in his name, the Lawrence Goldstein Science Policy Fellowship, which will train ISSCR members to become stem cell policy advocates.

"The society has long appreciated Larry's willingness to speak out in support of stem cell research and regenerative medicine, even in challenging social and political times," said ISSCR president Douglas Melton. "Larry has made a significant impact on the field and changed many hearts and minds. He exemplifies for scientists the important role our voices play in public debate, in ensuring that scientific evidence and facts are at the heart of any conversation about stem cells and their translation to medicine." [Continued on next page]

## Sanford Center Researcher Receives \$12 Million Grant to Launch Cystinosis Trial

The California Institute for Regenerative Medicine (CIRM) governing board awarded Stephanie Cherqui, PhD, associate professor of pediatrics at UC San Diego and Sanford Center faculty member, a grant of nearly \$12 million to conduct a clinical

trial for the treatment of cystinosis. Cystinosis is a rare metabolic disease that primarily affects children and young adults. It is characterized by the abnormal accumulation of an amino acid called cystine. Excess cystine damages cells and slowly destroys the organs of the body including the kidneys, liver, eyes, muscles and the brain. [Continued on next page]



## New Executive Director

We are excited to announce Michelle Ghani as the new executive director of the Sanford Stem Cell Clinical Center at UC San Diego Health. Michelle graduated from UC San Diego in 2003 with a bachelor of science in management science and a minor in psychology. She was recently accepted into a two year graduate program at Point Loma Nazarene University where she will be pursuing a master's degree in business administration.

Michelle brings over nine years of experience working at UC San Diego Health. She previously served as a fund manager and fiscal analyst in the business office of the Moores Cancer Center. She then took on the role of finance manager for the clinical trials office, specializing in both clinical trial budgeting and clinical trial financial management. For the last four years Michelle has been the division administrator for the Division of Regenerative Medicine providing fiscal, administrative, and operational support to faculty and staff. She will continue to support the Division in addition to support programs that will accelerate the translation of stem cell-related discoveries into clinical trials.



## Establishment of Fellowship in Honor of Director Emeritus (Continued from Previous Page)

For the last 25 years, Dr. Goldstein has been a faculty member and principal investigator of a biomedical research laboratory at UC San Diego School of Medicine. He also founded and directed the UCSD Stem Cell Program and the Sanford Stem Cell Clinical Center and is the founding scientific director of the Sanford Consortium for Regenerative Medicine. Goldstein co-chaired the scientific advisory board for California's Proposition 71 that established \$3 billion for stem cell medical research in the state. Throughout his career, Goldstein has advocated for science before local, state, and national policy makers on issues such as decisions about funding levels, and guidelines and ethical standards for research using stem cells, fetal tissue, and other matters.

The Lawrence Goldstein Science Policy Fellowship started accepting applications in June 2019. Information about submitting an application for the program is available at [ISSCR.org](http://ISSCR.org).

## Sanford Center Researcher Receives \$12 Million Grant to Launch Cystinosis Trial (Continued from Previous Page)

The clinical trial entails taking blood stem cells from patients with cystinosis, genetically-modifying the cells to carry a normal CTNS gene, and then returning the cells to the same patient. The modified blood stem cells are designed to create a new, healthy, blood system free of the disease.

"The Sanford Stem Cell Clinical Center helped accelerate the preclinical studies for this project by providing project management guidance, regulatory support, and invaluable staff training," said Cherqui. "Many principal investigators within the academic setting are unfamiliar with the rigorous process of going from bench to bedside. The Sanford Center provided my team with the resources and direction to meet the regulatory and reporting requirements necessary to bring this gene therapy to patients affected with cystinosis."

This is the 17<sup>th</sup> clinical trial overseen by the CIRM Alpha Stem Cell Clinic at UC San Diego Health. To learn about eligibility for this clinical trial, visit [ClinicalTrials.gov](http://ClinicalTrials.gov).



*Maria Millan, MD, President and CEO of CIRM, David Higgins, PhD, CIRM board member and patient advocate for Parkinson's disease, and Catriona Jamieson, MD, PhD, director of the UC San Diego Health CIRM Alpha Stem Cell Clinic and Sanford Stem Cell Clinical Center*

## Stem Cell Therapies and You: A Patient Advocate Event

In collaboration with California Institute for Regenerative Medicine (CIRM), the Sanford Stem Cell Clinical Center hosted Stem Cell Therapies and You: A Patient Advocate Event on May 28, 2019 at the the Sanford Consortium for Regenerative Medicine.

The TED-style talks and panel discussions provided an overview of research funded by CIRM, recent scientific advancements and current clinical trials at the UC San Diego Health CIRM Alpha Stem Cell Clinic. A patient enrolled in the Phase I breast cancer clinical trial at UC San Diego Health also shared her experience as a clinical trial participant and what it means to her to have the opportunity to participate in research.

For more information on the Phase I breast cancer clinical trial, contact the CIRM Alpha Stem Cell Clinic at 844-317-STEM (7836) or [alphastemcellclinic@ucsd.edu](mailto:alphastemcellclinic@ucsd.edu).

## Services at the ACTL

The Advanced Cell Therapy Laboratory (ACTL) at UC San Diego offers expert assistance and hands-on services to investigators who require cGMP systems and facilities. We aid in the transition from research and development to compliant and clinically-relevant manufacture of cell therapy products for IND-enabling studies or Phase I/II trials.

The ACTL is equipped with two manufacturing suites to enable product segregation and multi-lot production. Each suite consists of ISO 7 class cleanrooms with dedicated ISO 7/8 class ante-rooms for gowning. Each cleanroom is fitted with ISO 5 biosafety cabinets and CO2 incubators. Adjacent to the cleanrooms is a process development and Quality Control laboratory. The ACTL also has two nitrogen storage freezers suitable for long-term storage of cellular products in vapor-phase.

### ACTL Services

- Optimizing manufacturing processes, including scale-up, closed systems, and delivery formats
- Development of Standard Operating Procedures and Batch Production Records
- Cell product manufacturing, including cell enrichment with the CliniMACS System
- Cell product characterization, including standard microbiological release assays, flow cytometry and RT-qPCR assessment
- Secure storage of cell banks and cell products
- Materials risk assessment and selection
- Writing or review of CMC or supporting reports

