SANFORD REPORT

Monthly Newsletter of Sanford Stem Cell Clinical Center

UC San Diego Health
Sanford Stem Cell Clinical Center

IN THIS ISSUE

The Sanford Stem Cell Clinical Center team supports outpatient clinic operations for our clinical trials at the Koman Family Outpatient Pavilion.

Dr. Catriona Jamieson and biotech executive John Hood
Credit: The San Diego Union-Tribune

Once shelved as too risky, blood cancer drug discovered in San Diego gets FDA approval

By Bradley Fikes, The San Diego-Union Tribune

A promising cancer drug, discovered in San Diego and later discarded for safety concerns, has at last been approved for use. The drug, fedratinib, treats certain kinds of a bone marrow cancer called myelofibrosis.

The U.S. Food and Drug Administration approved fedratinib, which will be sold by Celgene under the brand name Inrebic. It had originally been developed by San Diego's TargeGen, purchased by Sanofi in 2010 for up to $635 million.
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CIRM Awards Dr. Mark Tuszynski $6.2M Grant for Spinal Cord Injury

Mark Tuszynski, MD, PhD, professor of neurosciences at UC San Diego and Sanford Center faculty member, was awarded a $6.2 million grant by the California Institute for Regenerative Medicine (CIRM) to develop a neural stem cell therapy for spinal cord injury (SCI).

Dr. Tuszynski will use human embryonic stem cells to create neural stem cells (NSCs) which will then be grafted at the injury site. In preclinical studies, the NSCs have been shown to support the formation of neuronal relays at the site of SCI. The neuronal relays allow the sensory neurons in the brain to communicate with the motor neurons in the spinal cord to re-establish muscle control and movement.

International Space Station Project Dedicated to T. Denny Sanford

By Jackie Carr

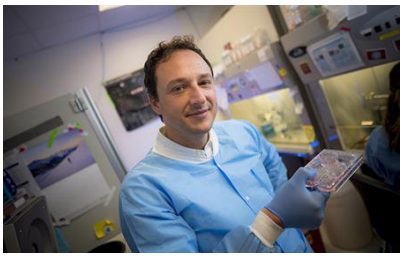
A research team from University of California San Diego, led by Alysson R. Muotri, PhD, seeks to boldly go where the first answers may be found: Launching a payload of stem cell-derived human brain organoids to the International Space Station (ISS) orbiting almost 250 miles above Earth, where researchers will document how these masses of cells organize into the beginnings of a functional brain.

The first-ever project of its type is dedicated to T. Denny Sanford, a longtime advocate of stem cell research whose partnership has supported
[Continued on next page]

Sanford Center Researchers Take Brain Organoids One Step Further

By Heather Buschman, PhD

Brain organoids — also called mini-brains — are 3D cellular models that represent aspects of the human brain in the laboratory. Brain organoids help researchers track human development, unravel the molecular events that lead to disease and test new treatments. They aren't prefect replicas, of course. Brain organoids do not replicate cognitive function, but researchers can check how an organoid's physical structure or gene expression changes over time or as a result of a virus or drug.



Credit: Erik Jepsen, UC San Diego

University of California San Diego researchers have now taken brain organoids one step further, achieving an unprecedented level of neural network activity — electrical impulses that can be recorded by multi-electrode arrays. Using data from babies born up to three-and-a-half months premature, the team developed an algorithm to predict their age based upon EEG patterns. The algorithm then read lab-grown brain organoids the same way, and assigned them an age.

The electrical impulse pattern for nine-month-old brain organoids revealed similar features to those of a premature infant who had reached full-term (40 weeks gestation). Read more

Science Translational Medicine Publishes Dr. Jeremy Rich's Study on Glioblastoma

By Heather Buschman, PhD

Glioblastoma is an aggressive form of brain cancer that infiltrates surrounding brain tissue, making it extremely difficult to treat with surgery. Even when chemotherapy and radiation successfully destroy the bulk of a patient's glioblastoma cells, they may not affect the cancer stem cells. This small population of tumor cells have the capacity to grow and multiply indefinitely, and can lead to tumor recurrence.

To study these tumors and test new therapies, researchers at University of California San Diego School of Medicine use mice tumor samples donated by patients who underwent surgery. With this approach, they recently discovered that treatment with both a targeted cancer therapy and the multiple sclerosis (MS) drug teriflunomide halts glioblastoma stem cells, markedly shrinks tumors and improves mouse survival.

The study is published August 7, 2019 in Science Translational Medicine.

"We used to think we'd find a single magic bullet to treat everyone with glioblastoma," said senior author Jeremy Rich, MD, professor of medicine at UC San Diego School of Medicine and director of neuro-oncology and director of the Brain Tumor Institute at UC San Diego Health. "But now we realize that we need to find out what drives each patient's unique tumor, and tailor our treatments to each individual."

In recent years, the desire to personalize cancer therapies has led to the development of several targeted cancer therapies. Read more

International Space Station Project Dedicated to T. Denny Sanford

(Continued from Previous Page)

partnership has supported Muotri's work as well as several key research entities, including the Sanford Consortium for Regenerative Medicine and UC San Diego Sanford Stem Cell Clinical Center.

Credit: UC San Diego

"On July 21, UC San Diego will partner with Space Tango to launch a payload of living brain organoids into space," said Erik Viirre, MD, PhD, professor of neurosciences and director of the Arthur C. Clarke Center for Human Imagination. "The study results will have enormous implications for space colonization and human health. We hope to determine if humanity can reach into the broader cosmos."

Credit: UC San Diego

"This groundbreaking experiment will evaluate more than 100 brain organoids, also known as 'mini-brains,' while in orbit," said Muotri, professor in the UC San Diego School of Medicine Departments of Pediatrics and Cellular and Molecular Medicine. "We will be able to continuously observe the formation of the neural tube, including cell migration, cell-cell interaction, cell division and death. This will be the first in a series of space flights to help us understand the intricacies of brain development, both in weightlessness and on Earth." Read more