SANFORD REPORT

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UC San Diego Health

Sanford Stem Cell Clinical Center

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Dr. Catriona Jamieson treats patients with blood cancers. The UCSD oncologist is also a researcher who helped prove that a recently approved cancer drug, fedratinib, was a good candidate for clinical testing. Jamieson is shown in her lab at the Sanford Consortium for Regenerative Medicine on October 4, 2019, in San Diego. Credit: Howard Lipin, The San Diego Union-Tribune



Dr. Alysson Muotri, a UCSD stem cell scientist, discusses how mini-brains, or brain organoids, can help understand and treat brain-based diseases.

Credit: Bradley J. Fikes, The San Diego Union-Tribune

In creating so-called minibrains, how close to a real human brain is too close?

By Bradley Fikes, The San Diego Union-Tribune

Do I exist? Do you exist? How do I know you exist?

These ancient questions have been given new urgency by 21st century science. On Friday, some of San Diego's top researchers met in La Jolla to discuss them. [Continued on next page]

A cancer drug's journey: Hope found, then lost, then found again

By Bradley Fikes and John Wilkens, The San Diego Union-Tribune

Cancer patients hold onto hope the way some people hold onto the lap bars of roller coasters: As if their lives depend on it.

And then they get ready for the ups and downs.

Theresa Blanda and Nancy Davidson started their rides after they were diagnosed with rare and debilitating blood cancers that enlarge the spleen and can progress to complications that are fatal.

Both volunteered for a clinical trial with a drug developed by a small San Diego biotech company. Unlike the vast majority of trials, which end in disappointment, this one worked for them. The drug, fedratinib, made them better.

So much better that Blanda returned to her accounting work in the defense industry. She made plans to marry. She dreamed about traveling.

Davidson stopped worrying about whether she would live long enough to see her son and daughter graduate from high school and college.

Then, five years into the trial, eight patients developed what appeared to be a dangerous neurological disorder that causes brain swelling and confusion. One died. Was the drug responsible?

U.S. regulators put a safety hold on the medication, and rather than investigate further, the French pharmaceutical company that owned fedratinib walked away. [Continued on next page]

In creating so-called minibrains, how close to a real human brain is too close? (Continued from Previous Page)

The topic was so-called mini-brains, pea-sized structures of human neurons grown from stem cells. Called human brain organoids by scientists, they are yielding important discoveries about autism, brain damage from Zika and other neurological conditions.

These mini-brains enable scientists to probe brain functioning in a way that's not ethical in healthy humans. said Dr. Alysson Muotri, a UC San Diego brain organoid researcher and a meeting leader.

But the mini-brains are becoming more complicated, giving rise to the theoretical possibility they might eventually acquire minds of their own, he said.

These mini-brains show no signs of consciousness at present, he said. And existing technological limitations make that very unlikely.

Muotri said mini-brain are limited in size because they don't develop blood vessels. That means that as the organoids grow in their cultures, the inner begin to die. They also don't develop all the types of cells found in a human brain.

But Muotri and colleagues recently demonstrated that these organoids can make brain waves similar to those of premature babies — something never done before. And as for the future, it's impossible to say. <u>Read more</u>

A cancer drug's journey: Hope found, then lost, then found again (Continued from Previous Page)

"I've never felt better and all of a sudden they're gonna just take this drug away from me?" Davidson said. "Where do I go from here?"

Dr. Catriona Jamieson, an oncologist and researcher at UC San Diego, wondered the same thing. She was running the UCSD trial site with Davidson and Blanda in it. When they stopped using the drug, they got sicker.

As months went by, other physicians involved in clinical trials saw similar setbacks. One, in Michigan, reported that 11 of his patients, all of whom had responded well to fedratinib, died after they were taken off it.

Jamieson went to John Hood, a scientist and entrepreneur who had helped invent the drug. "We need to do something," she said. <u>Read more</u>

Dr. Barbara Parker Receives Lifetime Achievement Award



Barbara Parker, MD

The 2019 American Cancer Society's Celebration of Cancer Care Champions recognizes and honors innovative research scientists in San Diego and cancer care professionals who have devoted a lifetime to the fight against cancer. Among those honored is Dr. Barbara Parker, who received the Lifetime Achievement Award.

Dr. Parker is professor of medicine and co-principal investigator of the 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. For more information on this clinical trial, contact the UC San Diego Health CIRM Alpha Stem Cell Clinic at alphastemcellclinic@ucsd.edu or click here.



Lawrence Goldstein Policy Fellows Announced

The ISSCR is pleased to announce the recipients of the inaugural Lawrence Goldstein Policy Fellowship, Mohamed Abou El-Enein, PhD (Charité Universitätsmedizin, Berlin, Germany) and Zubin Master, PhD (Mayo Clinic, Rochester, MN, U.S.).

This policy fellowship, named for long-time ISSCR member and policy stalwart Lawrence Goldstein, is designed to train scientists to become stem cell policy advocates. Recipients will gain hands-on experience in policy work including serving as ex-officio members of the ISSCR policy committee, participating in ISSCR advocacy outreach activities in their region of the world, and working with the committee on projects, policy messaging and communications.

Larry Goldstein, PhD

The Goldstein Policy Fellows come from Europe and the U.S. and have extensive interests in science

policy and regulatory issues. Dr. Abou El-Enein, the head of Clinical & Regulatory Affairs Platform at the Berlin Center for Advanced Therapies (BeCAT) and a Professor of Clinical Development of Regenerative Medicine is involved in designing and navigating the regulatory landscape of human clinical trials. Dr. Master is an Associate Professor of Biomedical Ethics and has experience in governmental health policy with Health Canada and has carried over those interests to his academic study on the translation of stem cell and regenerative medicine research to improve the health and treatment options for patients. Both fellows have published on unproven stem cell products.

"Drs. Abou El-Enein and Master are outstanding candidates to launch this program. They have demonstrated an academic interest in science policy and a commitment to the public service required to apply these policies and engage regulators. I look forward to working with them." said the fellowships namesake, Dr. Larry Goldstein. <u>Read more</u>