# UCLA scientist receives CIRM Discovery Grant to develop a cellular therapy for cancer using blood stem cells

Mirabai Vogt-James | Thursday, July 19, 2018

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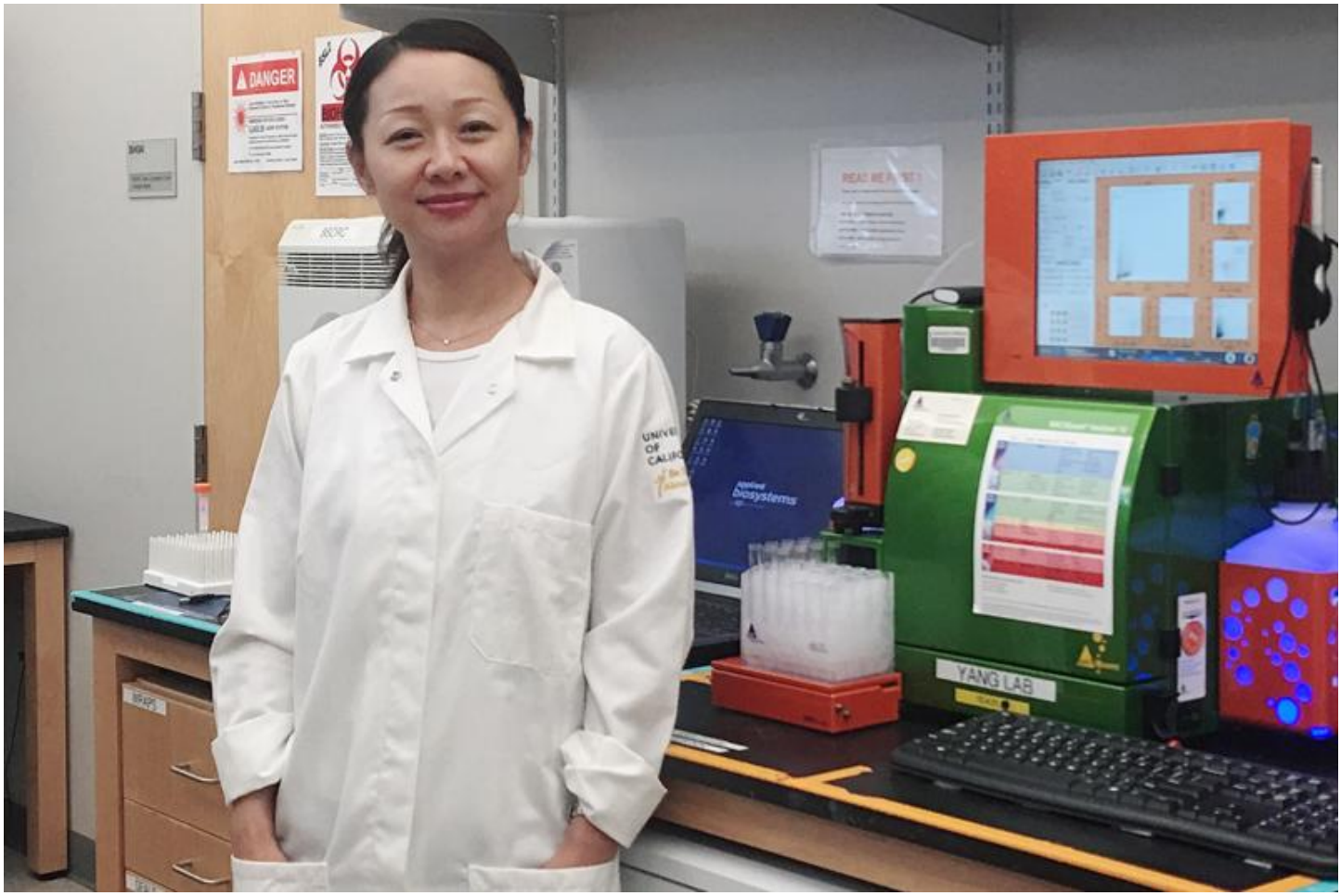


Lili Yang, a researcher from the Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research at UCLA has received a Quest Discovery Program award totaling approximately $1.4 million from the California Institute for Regenerative Medicine, the state stem cell agency.

The award was announced at a meeting of the Independent Citizens Oversight Committee, CIRM's governing body on July 19, 2018.

CIRM established the Quest Discovery Program to support the development of promising novel stem cell-based technologies that will be ready for translational studies within two years.

Yang's award will fund her efforts to develop a cellular therapy that could potentially be used to treat multiple cancers including solid tumors (such as melanoma, colon, lung, breast, and head and neck cancers) and blood cancers (such as leukemia, multiple myeloma, and myelodysplastic syndromes).

"The treatment protocol we will test with this funding has the potential to become an affordable, universal and off-the-shelf therapy for multiple cancers," said Yang, who is also a member of the UCLA Jonsson Comprehensive Cancer Center and an assistant professor in the UCLA Department of Microbiology, Immunology and Molecular Genetics.

Yang's novel approach will genetically modify blood-forming stem cells – which produce every type of blood cell, including the immune cells that can fight disease – to create large supplies of invariant natural killer T (iNKT) cells, a powerful subset of immune cells that have the remarkable capacity to target a broad range of cancers. To date, the clinical applications of iNKT cells have been greatly limited because they don't naturally exist in high numbers in the body; one drop of human blood contains around 10 million total blood cells but only around 10 iNKT cells; cancer patients typically have even less iNKT cells.

Using blood-forming stem cells from healthy donors, Yang's approach will first genetically modify the cells in two ways:

- One genetic modification will insert a receptor that will prompt the blood-forming stem cells to create only iNKT cells and not any other kind of T cell
- The second genetic modification will remove specific molecules from the blood-forming stem cells, prompting the stem cells to create iNKT cells that won't cause rejection when transplanted into a patient. This means the cells could come from any donor but still be universally compatible with any patient.

The genetically modified blood-forming stem cells will then be put into an artificial thymic organoid in collaboration with Gay Crooks, a professor of pathology and laboratory medicine and of pediatrics and co-director of the UCLA Broad Stem Cell Research Center, whose lab developed the organoid. This organoid mimics the natural functions of the thymus, which turns blood-forming stem cells into immune cells within the body. After 8 weeks, the blood-forming stem cells will produce iNKT cells that will be multiplied in the lab, tested for safety and then frozen.

Using this method, Yang and the research team estimate that about 1,000 to 10,000 doses of iNKT cells can be produced from a single blood stem cell donor.

The team plans to test the effectiveness of the iNKT cells in preclinical animal models of various types of human cancer. If the method proves successful, the team hopes to take the concept to clinical trials in the future and ultimately create a lasting supply of iNKT cells that are readily available to treat a large population of cancer patients.

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