

---

**Generation of clinical grade human iPS cells**

**Grant Award Details**

---

Generation of clinical grade human iPS cells

**Grant Type:** New Cell Lines

**Grant Number:** RL1-00681

**Investigator:**

<b>Name:</b>	Zack Jerome
<b>Institution:</b>	University of California, Los Angeles
<b>Type:</b>	PI

---

**Disease Focus:** Amyotrophic Lateral Sclerosis, Cancer, Melanoma, Muscular Dystrophy, Neurological Disorders, Skeletal/Smooth Muscle disorders, Solid Tumors

**Human Stem Cell Use:** iPS Cell

**Cell Line Generation:** iPS Cell

**Award Value:** \$1,341,000

**Status:** Closed

**Progress Reports**

---

**Reporting Period:** Year 1

[View Report](#)

**Reporting Period:** Year 2

[View Report](#)

**Reporting Period:** Year 3

[View Report](#)

---

**Grant Application Details**

---

**Application Title:** Generation of clinical grade human iPS cells

**Public Abstract:** The therapeutic use of stem cells depends on the availability of pluripotent cells that are not limited by technical, ethical or immunological considerations. The goal of this proposal is to develop and bank safe and well-characterized patient-specific pluripotent stem cell lines that can be used to study and potentially ameliorate human diseases. Several groups, including ours have recently shown that adult skin cells can be reprogrammed in the laboratory to create new cells that behave like embryonic stem cells. These new cells, known as induced pluripotent stem (iPS) cells should have the potential to develop into any cell type or tissue type in the body. Importantly, the generation of these cells does not require human embryos or human eggs. Since these cells can be derived directly from patients, they will be genetically identical to the patient, and cannot be rejected by the immune system. This concept opens the door to the generation of patient-specific stem cell lines with unlimited differentiation potential. While the current iPS cell technology enables us now to generate patient-specific stem cells, this technology has not yet been applied to derive disease-specific human stem cell lines for laboratory study. Importantly, these new cells are also not yet suitable for use in transplantation medicine. For example, the current method to make these cells uses retroviruses and genes that could generate tumors or other undesirable mutations in cells derived from iPS cells. Thus, in this proposal, we aim to improve the iPS cell reprogramming method, to make these cells safer for future use in transplant medicine. We will also generate a large number of iPS lines of different genetic or disease backgrounds, to allow us to characterize these cells for function and as targets to study new therapeutic approaches for various diseases. Lastly, we will establish protocols that would allow the preparation of these types of cells for clinical use by physicians investigating new stem cell-based therapies in a wide variety of diseases.

**Statement of Benefit to California:** Several groups, including ours have recently shown that adult skin cells can be reprogrammed in the laboratory to create new cells that behave like embryonic stem cells. These new cells, known as induced pluripotent stem (iPS) cells should have, similar to embryonic stem cells, the potential to develop into any cell type or tissue type in the body. This new technology holds great promise for patient-specific stem-cell based therapies, the production of in vitro models for human disease, and is thought to provide the opportunity to perform experiments in human cells that were not previously possible, such as screening for compounds that inhibit or reverse disease progression. The advantage of using iPS cells for transplantation medicine would be that the patient's own cells would be reprogrammed to an embryonic stem cell state and therefore, when transplanted back into the patient, the cells would not be attacked and destroyed by the body's immune system. Importantly, these new cells are not yet suitable for use in transplantation medicine or studies of human diseases, as their derivation results in permanent genetic changes, and their differentiation potential has not been fully studied. The goal of this proposal is to develop and bank genetically unmodified and well-characterized iPS cell lines of different genetic or disease backgrounds that can be used to characterize these cells for function and as targets to study new therapeutic approaches for various human diseases. We will establish protocols that would allow the preparation of these types of cells for clinical use by physicians investigating new stem cell-based therapies in a wide variety of diseases. Taken together, this would be beneficial to the people of California as tens of millions of Americans suffer from diseases and injuries that could benefit from such research. Californians will also benefit greatly as these studies should speed the transition of iPS cells to clinical use, allowing faster development of stem cell-based therapies.

---

**Source URL:** <https://www.cirm.ca.gov/our-progress/awards/generation-clinical-grade-human-ips-cells>