

## Giving Opportunities

### **Post-Doctoral Fellowships** | \$2 million/\$125,000

Fellowship funds allow us to attract the most gifted graduate and post-doctoral fellows to work alongside faculty to gain traction on promising research problems. Their insights often lead to new discoveries and paths of inquiry.

### **Seed Grants** | \$250,000+

Seed funding for early-stage research launches new projects and allows accumulation of sufficient “proof of concept” data to successfully apply for follow-on funding from traditional funding sources such as the NIH. Competitive seed grant awards fuel pure innovation – the high-risk, high-reward research ideas that forge new realms of discovery and spark biomedical breakthroughs.

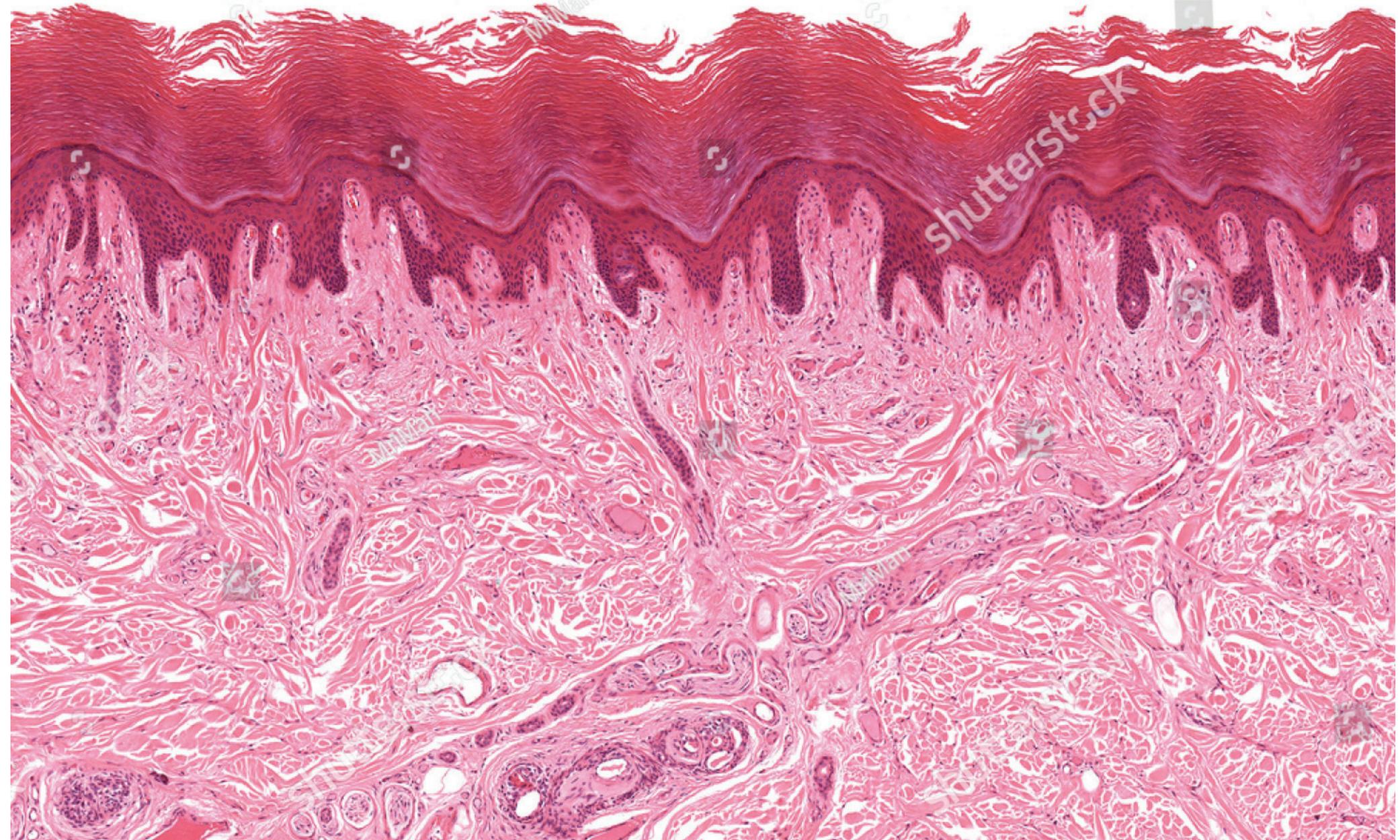
### **Disease-Specific Research** | Varying Gift Amounts

Research funds can be earmarked to accelerate any of the multiple research efforts currently underway, or help to launch a novel line of inquiry. Research funds allow faculty to pursue new opportunities, advance projects at critical stages, and expand current projects. Typically, the cost of a research project can range from several hundred thousand to more than a million dollars, but gifts of all amounts are appreciated to move the research forward.

### **General Program Support** | Any Amount

Discretionary support for Dr. Oro will enable him to strategically invest in the most promising research projects. General funds also support important technology development, to acquire valuable equipment, and employ the highly specialized technical support personnel needed to maintain it.

*microscopic cross section of human skin*



### **Contact Us**

Kat Walsch, MBA, *Senior Associate Director*

Medical Center Development  
3172 Porter Drive | Suite 210 | Palo Alto, California | 94304

650.724.9860 | [kwalsch@stanford.edu](mailto:kwalsch@stanford.edu)

## Therapeutic Reprogramming for Genetic Skin Diseases

**THE ORO LAB**

## LAB LEADER



### Anthony Oro, MD, PhD

The Eugene and Gloria Bauer  
Professor of Dermatology

Associate Director, Center for Definitive  
and Curative Medicine

Dr. Oro and his team develop novel cellular and molecular therapies for genetic skin diseases, hair loss conditions, and non-melanoma skin cancer. Members of the Oro Lab study the skin to answer unresolved questions in stem cell biology, cancer genetics, and metastasis.

As a post-doctoral fellow, Dr. Oro was part of the team that established the link between basal cell carcinoma and the Hedgehog pathway, a cascade of biochemical signals that regulate cell growth and differentiation. Basal cell carcinoma is the most common cancer and serves as a model for treating other Hedgehog-dependent cancers like pancreatic, colon, lung, breast, and brain. Dr. Oro and his team showed that by blocking this pathway, they could reduce the size of tumors. They provided clinical evidence that led to FDA approval for the vismodegib, the first Hedgehog pathway inhibitor.

“To watch a lab discovery become a drug and then, as a physician, to treat patients with that drug is rare for a scientist and very rewarding,” Dr. Oro says. He continues to study the pathway to understand how tumors become resistant to treatment.

## Imagine if we could cure patients by reprogramming their cells.

**That’s exactly what Tony Oro and his team are doing.** Right now, more than 250 million people worldwide live with genetic diseases. Too often, physicians can only offer patients a lifetime of trial and error symptom management or palliative care to keep them comfortable without solving the underlying issue. Starting with genetic diseases, the Oro Lab is harnessing the regenerative power of stem cells to find better, more definitive solutions.

**Often, doctors know exactly what causes these diseases.** But definitive treatments are elusive, frustrating both doctors and patients. Pluripotent stem cells, which can be derived from adult tissue, hold the promise of lasting solutions because they can produce any type of cell the body needs to repair itself. These master cells exist within regenerative properties as embryonic stem cells. Dr. Oro and his team are developing ways to transform a patient’s own induced pluripotent stem cells (iPS) into therapies for some of the most heart-breaking and intractable diseases.

**Stem cell therapies hold the promise of lasting solutions.** For conditions with well-defined genetic causes, scientists begin by sequencing the patient’s genome to determine the faulty genes responsible for the condition. Doctors then take some of the patient’s skin cells and reprogram them into iPS cells. Then they use a specially engineered virus to deliver corrected DNA into each of the cells. After that, they convert the iPS back into skin cells. Instead of carrying information for the disease, these new skin cells carry information to repair and correct the condition. The novel technique is called therapeutic reprogramming.

**The impact can be definitive and curative.** More than a temporary Band-Aid, the corrected cells can alter the patient’s condition for years—possibly forever. Epidermolysis bullosa, commonly called EB, is one example where therapeutic reprogramming can be life-changing. Children born with this devastating skin disease fail to create the collagen that keeps a healthy person’s epidermis attached to lower layers of skin. With every bump, scratch, and rub, these children lose

the top layer of skin and receive painful blisters in its place. These kids rarely survive beyond their teens and spend their entire lives living like burn victims because, until now, physicians could only wrap them like mummies to try to prevent injury.

**Tony’s team is able to grow the corrected cells into sheets** of healthy skin that can replace damaged skin with precisely matched grafts—that produce the necessary collagen. These grafts can then be transplanted without risk of rejection because they’re created from the patient’s own cells.

**With our newly constructed Laboratory for Cell and Gene Medicine,** we’re working to expand the use of therapeutic reprogramming. This new facility means physician-scientists like Tony can work closely with stem cell biologists, geneticists, biomedical engineers, and other biotechnologists to manufacture clinical-grade therapies according to the FDA’s Current Good Manufacturing Practice (cGMP) standards. Because we have one of the few facilities like this in academia, our researchers can quickly test potential therapies safely and accelerate the translation from lab to clinic.

**Therapeutic reprogramming has tremendous potential.** Genetic conditions like sickle cell, liver disease, or hemophilia could potentially be treated—and someday eradicated—with cell-based therapies similar to the one Tony and his team have devised for EB patients. Same for wear and tear and other more abrupt injuries. What if iPS cells could coax the cartilage in an old runner’s knees to repair itself? Or repair spinal tissue after a person is injured in a car wreck?

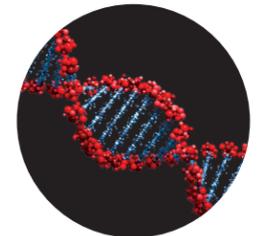
**You can help make these innovative therapies a reality for millions.** With your help, Tony and his team will investigate new ways to transform patients’ own cells into powerful treatment protocols. He will recruit and train tomorrow’s pioneers and expand the important work already being done by an interdisciplinary group of experts on Stanford’s campus. Your support will accelerate innovative projects that otherwise might go unfunded. And any one of them could make the difference for millions of people living with conditions that currently have no known treatments.



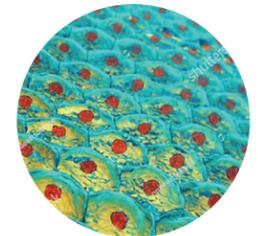
a small sample of skin cells are taken from an EB patient



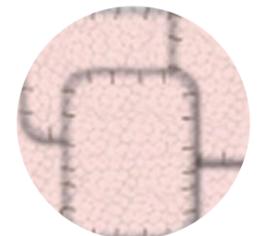
skin cells are converted into iPS cells



genetic defects are corrected using an engineered virus



corrected iPS cells are converted back into skin cells



which are grown into skin grafts perfectly matched to the patient