



## **Orchard Therapeutics and UCLA receive \$19 million in latest round of funding from California's stem cell agency**

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Orchard Therapeutics Limited and researchers from the Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research at the University of California, Los Angeles, led by Orchard Scientific Advisory Board member Dr. Donald Kohn have been awarded \$19 million by the [California Institute for Regenerative Medicine's](#) governing board (CIRM).

The grant will fund a new clinical trial for Severe Combined Immunodeficiency caused by adenosine deaminase deficiency, commonly known as ADA-SCID or "bubble baby" disease. ADA-SCID is a rare inherited disorder of the immune system. ADA-SCID is caused by mutations in the gene encoding for the enzyme adenosine deaminase, which result in a severe deficiency in white blood cells and life-threatening infections. In the absence of treatment, ADA-SCID is fatal within the first months of life.

To date, over 40 ADA-SCID patients have been treated in clinical trials with autologous *ex-vivo* lentiviral gene therapy at UCLA, Los Angeles and at the Great Ormond Street Hospital (GOSH) in London, UK. All patients have survived (100% overall survival) and the treatment has been shown to restore patients' immune function, with a favourable safety profile.

The treatment uses the patient's own stem cells which are modified with a functioning copy of the missing or faulty gene before being transplanted back into the patient's body. The use of the patient's own cells (autologous) removes the need to search for a matching stem cell donor, which can take months or even years.

"We are very grateful for this funding and for all the patients who have already gone through our clinical trials," said Dr. Kohn, a professor of pediatrics in the UCLA David Geffen School of Medicine, professor of microbiology, immunology and molecular genetics in Life Sciences at UCLA, member of the UCLA Children's Discovery and Innovation Institute at Mattel Children's Hospital.

Dr. Alexander Pasteur, Orchard's interim Chief Executive Officer, added "funding from CIRM will allow UCLA and Orchard Therapeutics to accelerate the development program with *ex-vivo* autologous lentiviral gene therapy, which could potentially greatly benefit patients."

### **About ADA-SCID**

ADA-SCID is a rare inherited disorder of the immune system. The incidence of ADA-SCID is currently estimated between 1 in every 375,000 to 660,000 live births according to literature sources. The actual incidence could be higher. ADA-SCID is caused by mutations in the gene encoding for the adenosine deaminase enzyme, which result in a severe deficiency in white blood cells and life-threatening infections.

### **About UCLA**

UCLA is an international leader in the breadth and quality of its academic, research, health care, cultural, continuing education and athletics programs. With more than 43,300 undergraduate and graduate students, it is the largest university in California. The UCLA College and 12 professional schools offer more than 5,000 courses, 124 undergraduate majors and 91 minors, 98 master's programs, and 109 doctoral and professional programs. Seven alumni and six faculties have been awarded the Nobel Prize. UCLA performs well in a wide variety of national and international university rankings.