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Pluripotent Cells: A tool to model development and to deliver definitive cure for pediatric diseases

Vittorio Sebastiano
Stanford School of Medicine, USA

The discovery of methods to convert somatic cells into induced pluripotent stem cells (iPSC) is probably one of the most revolutionizing breakthroughs in Regenerative Medicine within the last decade. iPSC are functionally equivalent to Embryonic Stem Cells (ESC) and have the ability, defined as Pluripotency, to generate any cell type of the adult body. Unlike ESC though, iPSC can be derived from the tissues of any individual, raising the possibility of producing custom-tailored cells for the study and treatment of virtually any disease. Furthermore, they are amenable to genetic manipulations, including homologous recombination (HR), which allows the in situ correction of the disease-causing mutation and avoids several safety risks associated with conventional vector-based gene therapy involving random integration such as non physiological gene expression and cancer formation. Although these prospects are exciting, several hurdles are associated with iPSC technology. Questions arise about the safety of the reprogramming and gene targeting methodologies, which involve extended culture periods, differentiation efficiency, and quality of iPSC-derived cells. These questions need to be answered before translation of iPSC-based technologies to the clinic. Research in the Sebastiano lab focuses on all aspects of this technology, spanning from nuclear reprogramming, to genome editing and *in vitro* differentiation. The ultimate goal of the lab is to develop clinical therapeutics using patients-specific iPSCs, by both modeling genetic and developmental disorders *in vitro* and by developing stem cell therapies that will be soon utilized in the clinic. Proof of principle studies and data on Epidermolysis Bullosa and DiGeorge Syndrome will be presented and discussed.

Biography

Vittorio Sebastiano is a Stem Cell and Developmental Biology Expert. His research focuses on dissecting the mechanisms that regulate nuclear reprogramming with the goal to use patient specific stem cells for the development of clinically relevant platforms for the treatment and the modeling of pediatric orphan diseases. He has been among the pioneers in gene therapy by *in situ* specific genome modification of iPSC derived from patients affected by Sickle Cell Anemia and Epidermolysis Bullosa. Currently, he is utilizing iPSCs to model developmental disorders and he is implementing clinical platforms to develop cell therapeutics for Precision Medicine.

vsebast@stanford.edu

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