

Are We At The Beginning Of Stem Cell Revolution?

Jan. 15, 2021 by Irina Bilous , Andrii Buvailo

A decade ago, iPSC (induced Pluripotent Stem Cell) technology was viewed as science fiction. iPSC can be obtained from adult tissues by applying a set of molecules - transcriptional factors - that can reprogram cells' fate back. This Nobel Prize-winning technology opens a landscape of opportunities, as in contrast to embryonic stem cells, iPSCs production is not complicated by a number of ethical issues as in the case of embryonic stem cells. Furthermore, since iPSCs are developed from patient's own cells, the body won't typically develop a severe immune response against them. Today, besides creating therapies for incurable diseases, the pharmaceutical industry tends to adopt iPSCs for screening purposes that will dramatically decrease the cost of clinical trials.

One of many breakthroughs in iPSCs therapies was reported in the New England Journal of Medicine in May 2020, revealing how iPSCs were used to treat Parkinson in a 69 year-old man. The cells were derived from the patient's skin and then reprogrammed to produce the dopaminergic neurons. As a result the patient's severe symptoms were diminished and he was able to return to some of his usual activities.

The example above demonstrates only one of dozens of diseases to which iPSCs can be applied, including diabetes, heart diseases, and so on. But it is equally important that iPSC platforms can be used as part of phenotypic screening research for drug discovery. According to Evotec's CEO Werner Lanthaler, the use of iPSCs can help to "circumvent the translational errors that are in early [drug discovery] models" [from this video]. Evotec owns proprietary human iPSC platform used by leading pharma brands, e.g. Bristol-Myers Squibb, for research programs in this area.

Induced Pluripotent Stem Cells technology is becoming an integral part of other emerging therapies. For example, Fate Therapeutics and Jansenn will work together to identify CAR(NK) and CAR-T cells derived from iPSCs. Fate Therapeutics will lead the research leveraging its iPSC product platform capable for large scale production of CAR-T cells ready for the "off-the-shelf" use, a step ahead of the current "case-by-case" strategy of CAR-T cells preparation. This opportunity may bring new and cheaper options for patients and commercial benefits of up to 3 billion dollars for the companies.

iPSC technology is also a lucrative opportunity for anti-aging research and therapy developments, Blue Rock Therapeutics and Agex Therapeutics being notable examples. Blue Rock Therapeutics was

launched by Bayer and Versant Venture back in 2016. In 2019 Bayer acquired the remaining stake for \$240 M in cash. Blue Rock has technologies to create cell types for neurology, cardiology, and immunology. Additionally they are developing iPSCs to be used as new therapeutics modalities for local non-toxic therapeutics delivery.

Agex develops several cell-based therapies, currently in preclinical stage, focused on treatments of cardiac ischemia, certain age-related metabolic disorders, and restoring regenerative potential in a wide array of aged tissues.

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