ABSTRACT

Induced pluripotent stem cells (iPSC) are one of the key discoveries in cell biology of the last decade. These cells are pluripotent stem cells derived from differentiated somatic cells while having used only four exogenous transcription factors. Pluripotent cells, which can be derived from somatic cells carrying genetic mutation, have a great potential to be used in the testing of new drugs and in discovering molecular mechanisms of genetic disorders. iPSC derived from healthy cells can be used in regenerative medicine. Originally, retroviral vectors were used for delivering reprogramming transcription factors to cells. However such approach is not safe for medicinal use, because of the ability of retroviruses to integrate into the host genome. This fact initiated development of safer delivering methods of transcription factors into the cells.

In this work I present the overview of methods which have been used for reprogramming including the most common techniques used to test pluripotency. In addition, I will describe iPSC application options for therapy of genetically determined hematological disorders (sickle cell anemia, β-thalassemia, X-linked chronic granulomatous disease) and for modelling of their molecular mechanism (polycythemia vera).

Key words: iPSC, reprogramming, hematological disorders, cell therapy