Pharmaceutica Analytica Acta
ISSN: 2153-2435

Uchiumi and Tanuma, Pharmaceut Anal Acta 2012, 3:10
DOI: 10.4172/2153-2435.1000196

Short Communication Open Access

Anticipation of a Novel Gene Therapy Inspired by a Concept of iPS Cells

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Abstract

This year Nobel Prize in Physiology or Medicine was dedicated to two researches who have established and developed the study in the field of stem cells. At present, there seems to be still several problems to have to overcome before applying the techniques with iPS (induced pluripotent stem) cells to the practical use in the medical treatments. However, the concept of the iPS cells has brought a great hope not only in medicine, especially in regenerative therapies, but also in the development of new drugs. Here, from the point of view of medicine, we propose an another possibility to apply the concept of iPS cells to the new therapy of diseases, such as cancer.

Keywords: Gene therapy; iPS cells; Regenerative therapy; Reprogramming of cells; Transcription factors

The principle of the establishment of iPS cells is based on the cell biology to reprogram a differentiated cell to a state that the cell can differentiate into variety of cell types [1]. Various chemical compounds will be subjected to screening systems to identify or develop drugs that induce cell pluripotency [2]. However, the establishment of the iPS cells was originally demonstrated by the introduction of the set of four gene expression vectors into somatic cells [1,3]. It is frequently referred as Yamanaka factors, comprising of OCT3/4, SOX2, KLF4, and c-MYC. And it should be noted that all the encoded proteins are known as functional transcription factors (TFs) with DNA binding activities [4]. The concept of establishing iPS cells is extremely important to have indicated that only four TFs are required to manipulate cells convert into iPS cells, implying that certain alterations of transcriptional state in a cell can reinforce the cell to go back to its progenitors.

Up to present, a lot of efforts have been taken on the studies of cancer, and it was revealed that many factors, including proto-oncogene or tumor suppressor gene products and signal transduction proteins, are involved in the generation of tumors [5]. Given that cancer cells are originated from healthy cells, isn't it possible to reverse the history of cells into healthy state by introducing several TF-encoding gene expression vectors? Sooner or later, comprehensive studies of cancer by genomics or proteomics will indicate right combinations of the TF-encoding genes to regenerate normal cells from cancer cells. If introduction of these TF-encoding genes will make malignant cancer cells to stop growth and to go back into its progenitor like state, the only treatment that physicians need to do for the patients will be to deliver the TF-encoding gene expression vectors into cancer cells. This might be practically possible after the establishment of the gene-transfer system that is suitable for each individual cancer cell.

This proposal for the cancer therapy is only an example from the unlimited iPS cell-based concept. All researchers including us, especially who are studying life sciences, had better applaud the establishment of iPS cells that will make impossible to possible.

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Received November 23, 2012; **Accepted** December 17, 2012; **Published** December 21, 2012

Citation: Uchiumi F, Tanuma S (2012) Anticipation of a Novel Gene Therapy Inspired by a Concept of iPS Cells. Pharmaceut Anal Acta 3: 196. doi:10.4172/2153-2435.1000196