Imagine a world where any part of a person's body—from specific cells to entire organs—can be regenerated from skin cells alone. In this world, a patient's skin cells could be made into any cell type of interest in order to study diseases specific to that cell type. This way, the root causes of such diseases could be better studied in laboratories and highly specific drugs and therapies could be tested in order to find potential cures. A world like this would see a revolution in medical science and countless people could be rid of terrible diseases or have damaged body parts regenerated. Fortunately, this world could very well be ours in the near future due to recent advances in stem cell research.

Embryonic stem cells are cells that are capable of giving rise to any cell type in the body, a capability known as pluripotency. They also have the ability to grow indefinitely while maintaining pluripotency (1). These characteristics of embryonic stem cells are what endow them with the potential to be useful in understanding disease mechanisms, screening effective and safe drugs, and treating patients with various diseases and injuries (2). This is possible because embryonic stem cells can be cultured in a lab and then differentiated into a cell type of interest, which can then be studied or potentially used for cell replacement therapies. For a long time, scientists had to obtain stem cells from human embryos, destroying the embryo in the process (3). A recent discovery, however, showed that human skin cells can be reprogrammed to become stem cells—now called induced pluripotent stem cells or iPS cells—which can then be turned into any other type of cell through the process of differentiation (4). This demonstrates that differentiated cells can be successfully reprogrammed to become pluripotent stem cells, potentially bypassing the previous ethical concerns involved with embryonic stem cells research.

After the discovery of induced pluripotent stem cells, it remained unclear whether they could be generated directly from elderly patients with chronic disease and whether such patient-specific iPS cells could be differentiated into the particular cell types necessary to treat or study the patient's condition (5). A recent and remarkable advance in this direction generated iPS cells from skin fibroblasts of an 82-year-old woman diagnosed with a genetic form of amyotrophic lateral sclerosis (ALS), or Lou Gehrig's disease, and used them to generate healthy neurons. ALS is a terribly debilitating neurodegenerative disorder in which motor neuron loss in the spinal cord leads to progressive paralysis and death (6). (It is most well known as the disease that afflicts the renowned British theoretical physicist Stephen Hawking.) It was an extremely promising result that neither the patient's advanced age nor severe debilitation prevented scientists from successfully reprogramming her skin fibroblasts to make iPS cells. The patient-specific iPS cells generated from her skin cells were then directed to differentiate into healthy motor neurons and glia, neuron support cells (5). It was thus found that patient-specific iPS cells can respond appropriately to developmentally relevant cell signals, just like human embryonic stem cells. This demonstrates the feasibility of using the pluripotency of iPS cells to generate a potentially limitless supply of motor neurons with a patient's exact genotype, making them compatible with the patient's immune system, which has been a long sought after goal of regenerative medicine (5).

It has been very difficult to study genetically and environmentally complex diseases like ALS. Recent advances in stem cell research, however, are starting to tackle this problem using an iPS cell-based approach, wherein patient-specific iPS cells can be generated and then differentiated. In addition to helping scientists study these diseases, this approach may one day lead to potential cell replacement therapies, hopefully in the near future.

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References