Diseases-in-a-Dish
By Thuy Dung Nguyen

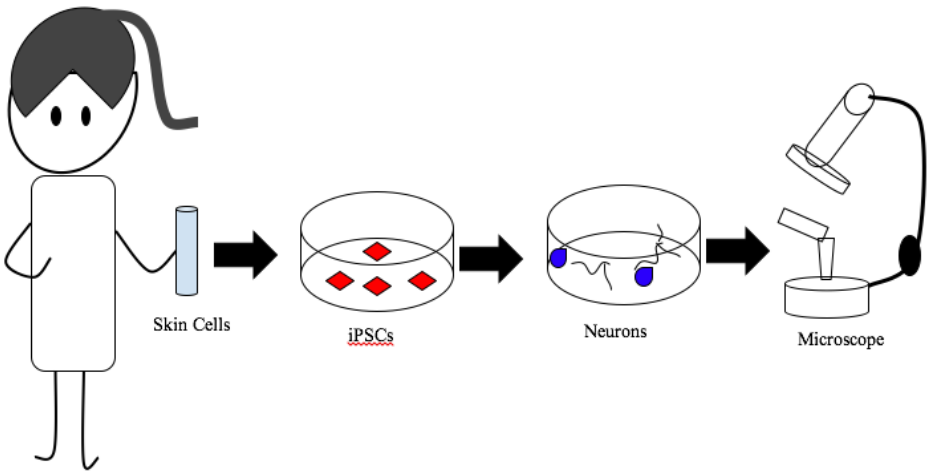
Imagine someone you know is diagnosed with cancer, Ebola, seizures, or another incurable disease. What if the diagnosis is one of the many unknown diseases for which there is still limited understanding of the disease? Will there ever be a cure? Will there still be hope?

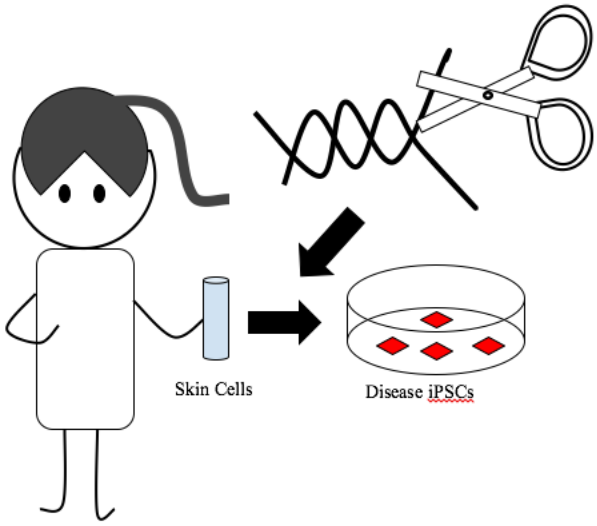
Though there may not be an immediate cure, there is something that scientists are doing to revolutionize the way we study and develop treatments for debilitating diseases.

Since the discovery of induced pluripotent stem cells (iPSCs) in 2006, the field of stem cell research is revolutionizing the way scientists study incurable diseases (1, 2). iPSCs have the ability to self-renew and develop into any cell type in the body. The potentials of iPSCs allow scientists to create cell models that represent diseases in a dish (3). Researchers can simply take a patient's skin cells, reprogram them into iPSCs, then grow them into liver cells, neurons or any cells of interest, offering an unlimited cell source to model and study a particular disease.

If the differentiated cell type came from that particular someone we mentioned earlier, the differentiated cells will have the same genetic materials as himself/herself. For example, if scientists use skin cells donated by that particular someone to differentiate them into neurons, these differentiated neurons will have characteristics and activities that will be just like neurons from that particular someone's brain if scientists were to take a direct sample through an invasive procedure. This will allow scientists to study the disease that particular someone diagnosed with in a dish and avoid any surgical procedure.

However, skin cell samples do not have to be from the patient who has the disease. Did you know the samples can be donated by you, or any healthy person? With skin cell samples from a healthy person, scientists can remodel them into any disease cell type. Let's look at the incurable Rett Syndrome as an example. If you want to visualize what Rett Syndrome is, just "Imagine the symptoms of Autism, Cerebral Palsy, Parkinson's, Epilepsy and Anxiety Disorder …..all in one little girl…." (4). Instead of obtaining a brain sample from a little girl with Rett Syndrome,





scientists can obtain skin cells from any healthy patient to study Rett Syndrome. Following reprogramming of healthy skin cells into iPSCs, scientists can remodel iPSCs to any disease cell through gene editing technologies. For example, Rett Syndrome is associated with mutations on the MECP2 gene. Scientists can introduce a specific mutation into the MECP2 gene to mimic the effect of the mutation found in Rett Syndrome patients (5). Once iPSCs carrying the mutation is differentiated into neurons, the differentiated neurons will behave similarly to neurons found in a Rett Syndrome patient's brain (5).

Therefore, skin cells from healthy patients can be just advantageous as skin cells from disease patients. They all allow a wide spectrum of possible diseases-in-a-dish modeling for further investigation and testing of potential therapeutic treatments for incurable diseases.

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