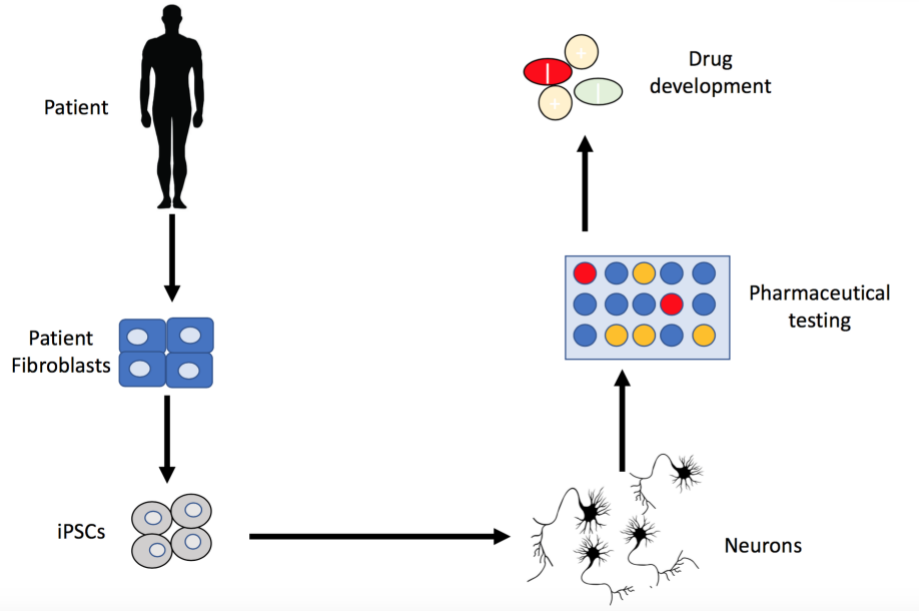
Stem Cells for Pharmaceutical Screening: A Step Away from Animal Research
By Jennifer Waldo

What if there was a way to find a cure for a devastating disease without the use of animal testing? Animals have long been used to screen pharmaceuticals before they are tested in people. Without the use of animals in labs, it would be nearly impossible to test pharmaceuticals for detrimental side effects. While animal models are still extremely important in testing new treatments, stem cells offer a new way to screen pharmaceuticals before subjecting animals to potentially harmful unknowns that do not always provide valuable insight.

Stem cells to be used for pharmaceutical testing can be created from human adults through an non-invasive process of obtaining a subset of skin cells called fibroblasts. These fibroblasts can then be genetically manipulated to become induced pluripotent stem cells (iPSCs) [1].

The use of iPSCs in research is an improvement on animal testing as iPSCs have many of the same internal functions and metabolic pathways as the patient they come from that animals models do not have. This is likely due to the large difference between a mouse and human, such as immune response and different metabolisms that make many diseases hard to reiterate in an animal model. Different species have different developmental pathways and will likely have different reactions to a pharmaceuticals based on how quickly it is metabolized. Other species may also have differing sensitivities to that pharmaceutical than a patient would [2]. This makes the outcomes of pharmaceutical screenings on animal models unreliable. For example, many treatments have provided "cures" in mouse models, but have failed when they are tested in people.

iPSCs have characteristics of cells from early development and can become any cell type in the body [1]. Due to the nature of iPSCs, they can be grown in a lab and be manipulated into making the cells that are most affected by the disease, such as a neuron for Huntington's disease [3]. Another bonus of these cells coming from patients is that they also contain the genetic information of the patient, such as disease associated traits. This means that if a patient has Huntington's, the cells that are taken from the patient will also have the traits of Huntington's. These disease-associated traits can then be evaluated after treatment with a potential pharmaceutical to see if the disease symptoms have been alleviated. One example would be looking for a decrease in the amount of the mutant Huntingtin protein—high levels of this proteins is known to play a major role in Huntington's disease [4].



The use of stem cells decreases the need to try unknown treatments on animals,

as the therapeutics can be tested in these cells and likely provide better insights. While this doesn't eliminate the need for animals within the science world, it does provide an important step away from animal research in a situation where it is not necessary. Stem cells allow for more rigorous pharmaceutical testing strategies that will likely allow for a more complete screen of pharmaceuticals prior to them reaching clinical trials.

1. Takahashi, K. and S. Yamanaka, *Induction of pluripotent stem cells from mouse embryonic and adult fibroblast cultures by defined factors.* Cell, 2006. **126**(4): p. 663-76.
2. Zuba-Surma, E.K., et al., *Stem cells as a novel tool for drug screening and treatment of degenerative diseases.* Curr Pharm Des, 2012. **18**(18): p. 2644-56.
3. Tousley, A. and K.B. Kegel-Gleason, *Induced Pluripotent Stem Cells in Huntington's Disease Research: Progress and Opportunity.* J Huntingtons Dis, 2016. **5**(2): p. 99-131.
4. Yang, S., et al., *CRISPR/Cas9-mediated gene editing ameliorates neurotoxicity in mouse model of Huntington's disease.* J Clin Invest, 2017. **127**(7): p. 2719-2724.