

The ALS Center for Cell Therapy and Regeneration Research at Johns Hopkins

The ALS Center for Cell Therapy and Regeneration Research at Johns Hopkins is committed to identifying the causes of the neurodegenerative disease Amyotrophic Lateral Sclerosis (ALS) and discovering new and effective treatment options.

At the ALS Center, Johns Hopkins researchers work with other investigators and clinicians within the Johns Hopkins ALS Clinic to aggressively take groundbreaking scientific discoveries and turn them into clinical applications that will improve the quality of life of those diagnosed with ALS.

The Center's research focuses on the use of stem cells to understand mechanisms of disease, in cell transplantation, and in the development of pharmaceuticals for treating ALS. However, the ultimate goal is to promote parallel research into ultimately preventing ALS altogether, as well as investigate the regrowth or repair of the nerve cells affected by the disease.

Our greatest limitation to treating Amyotrophic Lateral Sclerosis (ALS) is the lack of understanding what causes sporadic ALS. However, the discovery of human-induced pluripotent stem cells (iPSC) has multiple implications; including helping us understand how disease starts and progresses.

By using human induced pluripotent stem cells (iPSC) from our ALS patients, we are now studying mechanisms causing the development of ALS. We can turn these iPSC into ALS-relevant cells of the brain including motor neurons, astrocytes, and even muscle. This helps investigators understand the contributions of each of these cell types to the disease.

We have only begun to scratch the surface in understanding what causes ALS, what factors influence the timing of disease onset, its progression, and its heterogeneity. However, Johns Hopkins researchers are now making powerful discoveries in therapeutics for this disease.

Current needs for the ALS Center...

\$700/month—This is the monthly cost for maintaining 1 induced pluripotent stem cell (iPSC) line from a single patient. The ALS Center for Cell Therapy and Regeneration Research at Johns Hopkins maintains approximately 10 iPSC lines at one time for a total of approximately \$7000/month.

\$3000/Multielectrode Array Analysis: Multielectrode Array (MEA) is a new and powerful technique that allows us to record the electrical activity of human iPSC in a dish. This helps us recreate how human neurons from ALS patients might react to different drug compounds we could use for treatment. This cost is for a single experiment for 1 human iPSC line. On average, we study iPSC lines from 10 ALS patients at a time.

\$8000—Cell Culture incubator which keeps stem cells at a steady physiological temperature.

\$10,000—Cost to convert 1 ALS patient's blood cells into a human iPSC line. These iPSC are then used to create the motor neurons and astrocytes we use to study ALS.

\$15,000—The cost for purchasing enough ALS mice (\$300/mouse) for the completion of a single stem cell transplantation study or gene therapy study.