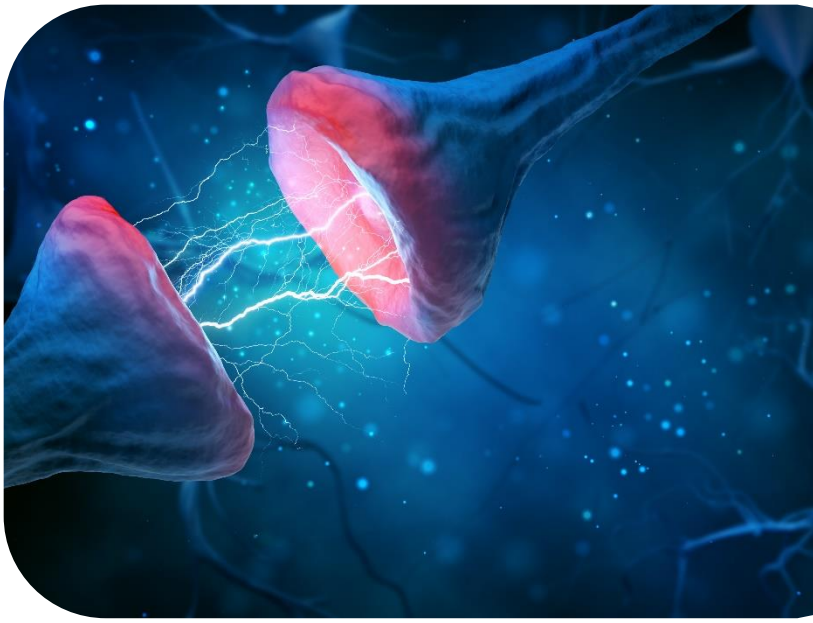
>> Parkinson's Disease

Parkinson's disease (PD) is a debilitating, incurable condition characterized by degeneration of dopaminergic neurons in the substantia nigra. Recently, improved access to and protocols for induced pluripotent stem cell (iPSC) culture are providing researchers with better tools for PD disease modeling and therapeutic discovery. Axion's Maestro platform provides sensitive functional analysis of dopaminergic neurons, enabling scientists to gain deep insight into disease mechanisms and assess the efficacy of potential therapeutics.



Learn how the Maestro can support Parkinson's disease research with **these selected publications:**

Recombinant pro-CTSD (cathepsin D) enhances SNCA/α-synuclein degradation in α-synucleinopathy models

Prieto Huarcaya S, Drobny A, et al. Autophagy. (2022)

Parkinson's disease (PD) involves intracellular buildup of SNCA/α-synuclein in the brain, and recent evidence suggests autophagy and lysosomal protease deficiency, including cathepsin D (CTSD), contribute to the lack of SNCA clearance. Using patient-derived iPSCs, researchers assess the potential of recombinant human CTSD (rHsCTSD) to enhance SNCA clearance as a therapeutic strategy.

Highlights:

- Treatment of PD patient-iPSC-derived neurons with rHsCTSD reduces SNCA accumulation.
- Using the Maestro to evaluate potential safety, the rHsCTSD treatment demonstrates no detrimental effects on neural activity.
- SNCA degradation and restored autophagy and lysosomal function are also observed *in vivo*, suggesting CTSD treatment may be a promising therapeutic approach.

Read more >>

iPSC modeling of young-onset Parkinson's disease reveals a molecular signature of disease and novel therapeutic candidates

Laperle AH, Sances S, et al. Nature Medicine. (2020)

Young-onset Parkinson's disease (YOPD) accounts for approximately 10% of Parkinson's cases, yet most of these patients have no familial history or identified genetic mutations. Additionally, iPSCs derived from sporadic PD patients often do not exhibit the same phenotypes; therefore, researchers developed a YOPD model to better understand disease mechanisms and severity.

Highlights:

- YOPD differentiated neurons exhibit α-synuclein accumulation and dysregulation of lysosomal proteins.
- Maestro is used to characterize functionality and maturation of iPSC-derived dopaminergic neurons.
- Activation of lysosomal pathways reduces α-synuclein accumulation both *in vitro* and *in vivo*, suggesting therapeutic potential.

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Human autologous iPSC-derived dopaminergic progenitors restore motor function in Parkinson's disease models

Song B, Cha Y, et al. The Journal of Clinical Investigation. (2020)

Stem cell therapy presents a promising approach to treat Parkinson's disease (PD) and other neurodegenerative diseases; however, clinical translation requires optimized, scalable protocols. Investigators present an iPSC dopaminergic neuron differentiation and purification protocol, then assess these neurons in an *in vivo* PD model.

Highlights:

- Novel "spotting" differentiation protocol and selection via quercetin results in high-yield and high-quality dopaminergic neurons.
- Differentiated neurons are characterized via the Maestro to validate function and maturation.
- When implanted in a rodent PD model, these neurons effectively integrate and restore motor function with no evidence of tumorigenesis.

Read more >>

iPSC-derived dopamine neurons reveal differences between monozygotic twins discordant for Parkinson's disease

Woodard CM, Campos BA, et al. Cell Reports. (2014)

Monozygotic twin studies present a useful platform to study neurodegenerative diseases, especially with improved access to and protocols for iPSC development. The authors generate iPSC-derived dopaminergic neurons from twins discordant for Parkinson's disease (PD) to assess differences in neural function and potential therapeutic strategies.

Highlights:

- Mutant neurons exhibit increased accumulation of α-synuclein and decreased dopamine production compared to wild-type (WT) neurons.
- Functional characterization of mutant neurons with Maestro demonstrates significantly reduced activity and delayed development compared to WT neurons.
- Genetic correction via overexpression of WT *GBA* in the mutant neurons reduces α-synuclein accumulation and restores enzymatic activity to WT levels.

Read more >>