Parkinson's Disease Fact Sheet

CIRM funds many projects seeking to better understand Parkinson's disease and to translate those discoveries into new therapies.

Description

Parkinson's disease is a neurodegenerative disease that affects approximately a million people in the United States and seven million people around the world. Symptoms include tremors, slow movement, muscle rigidity, balance issues and lack of facial expressions. Parkinson's disease occurs when the neurons or nerve cells in the portion of the brain that controls movement die off. These neurons send signals by releasing a chemical called dopamine, and are referred to as dopaminergic neurons. No cure exists for the disease and current medications become less effective over time.

Stem cell scientists are taking two general approaches to target Parkinson's disease. The first approach involves understanding the disease and looking for new drugs to treat it. CIRM grantees have taken skin cells from people with Parkinson's disease, reprogrammed them back to an embryonic-like state, turning them into the kind of stem cell that can be transformed into any other cell in the body, then coaxing those cells to become dopaminergic neurons that are lost to the disease. Those cells showed signs of the disease in the lab dish, and were distinctly different from the same cells created from healthy people.

CLINICAL STAGE PROGRAMS

Brain Neurotherapy Bio

Dr. Krystof Bankiewiecz and his team are using a gene therapy approach to promote the production of a protein called GDNF, which is best known for its ability to protect dopaminergic neurons, the kind of cell damaged by Parkinson's Disease. The approach seeks to increase dopamine production in the brain, alleviating PD symptoms and potentially slowing down the disease progress.

- To learn more about this clinical trial

Video: Progress and Promise in Developing a Cure for Parkinson's Disease

Being able to study human Parkinson's disease cells in a lab dish is a major milestone. Now, scientists can expose those cells to different drugs to find the ones that eliminate signs of the disease. If scientists find drugs that treat the disease in a lab dish, they will then test those same drugs in animal models and develop the most promising into a therapy for people with the disease. Several teams of CIRM-funded researchers are using stem cell techniques to create Parkinson's disease cells in the lab dish and then screening them for new drugs.

Other groups are creating dopamine-producing cells in the lab dish with the hope that they could replace the neurons that are damaged in people with the disease. See below for a list of a CIRM-funded projects related to Parkinson's disease.

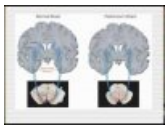
CIRM Grants Targeting Parkinson's Disease

Researcher name	Institution	Grant Title	Grant Type	Award Amount
Lei Wang	Salk Institute for Biological Studies	Genetic Encoding Novel Amino Acids in Embryonic Stem Cells for Molecular Understanding of Differentiation to Dopamine Neurons	New Faculty I	$2,587,742
Andres Bratt-Leal	Aspen Neuroscience	Autologous therapy for Parkinson's disease: single cell RNAseq for in depth characterization of transplanted cells	Progression Award - Discovery Stage Research Projects	$177,579
Michele Calos	Stanford University	Site-specific integration of Lmx1a, FoxA2, & Otx2 to optimize dopaminergic differentiation	Tools and Technologies II	$1,592,897
Birgitt Schuele	Parkinson's Institute	Editing of Parkinson's disease mutation in patient-derived iPSCs by zinc-finger nucleases	Tools and Technologies II	$1,327,983
Stuart Lipton	Scripps Research Institute	Drug Development of Inhibitors of Inflammation Using Human iPSC-Derived Microglia (hiMG)	Quest - Discovery Stage Research Projects	$1,658,123
Daniel Lim	University of California, San Francisco	Development and preclinical testing of new devices for cell transplantation to the brain.	Tools and Technologies II	$1,795,891
Susan McConnell	Stanford University	Identification and characterization of human ES-derived DA neuronal subtypes	Basic Biology I	$1,404,853
David Schaffer	University of California, Berkeley	Engineering Defined and Scaleable Systems for Dopaminergic Neuron Differentiation of hPSCs	Tools and Technologies II	$1,340,816
Xianmin Zeng	Buck Institute for Age Research	Banking transplant ready dopaminergic neurons using a scalable process	Early Translational II	$4,983,013
R. Jeremy Nichols	Parkinson's Institute	Understanding the role of LRRK2 in iPSC cell models of Parkinson's Disease	Basic Biology III	$1,482,822
Fred Gage	Salk Institute for Biological Studies	Crosstalk: Inflammation in Parkinson's disease (PD) in a humanized in vitro model	Early Translational II	$2,472,839
Stuart Lipton	Sanford Burnham Prebys Medical Discovery Institute	hESC-derived NPCs Programmed with MEF2C for Cell Transplantation in Parkinson's Disease	Disease Team Therapy Planning I	$96,448
Zhuohua Zhang	Sanford Burnham Prebys Medical Discovery Institute	Derivation of Parkinson's Disease Coded-Stem Cells (PD-SCs)	New Cell Lines	$1,556,448
Marcel Daadi	Sanford Burnham Prebys Medical Discovery Institute	Neural Stem Cell-Based Therapy For Parkinson's Disease	Disease Team Therapy Planning I	$63,952
Su Guo	University of California, San Francisco	Identifying small molecules that stimulate the differentiation of hESCs into dopamine-producing neurons	SEED Grant	$542,619

Steven Finkbeiner	Gladstone Institutes, J. David	Common molecular mechanisms in neurodegenerative diseases using patient based iPSC neurons	Basic Biology IV	$1,395,184
Susan McConnell	Stanford University	Optimization of guidance response in human embryonic stem cell derived midbrain dopaminergic neurons in development and disease	SEED Grant	$607,363
Xinnan Wang	Stanford University	Misregulated Mitophagy in Parkinsonian Neurodegeneration	Basic Biology V	$1,174,943
Zhuohua Zhang	Sanford Burnham Prebys Medical Discovery Institute	Modeling Parkinson's Disease Using Human Embryonic Stem Cells	SEED Grant	$701,060
David Schaffer	University of California, Berkeley	Engineered Biomaterials for Scalable Manufacturing and High Viability Implantation of hPSC-Derived Cells to Treat Neurodegenerative Disease	Tools and Technologies III	$1,239,276
Stuart Lipton	Sanford Burnham Prebys Medical Discovery Institute	MEF2C-Directed Neurogenesis From Human Embryonic Stem Cells	Comprehensive Grant	$2,832,000
Jeanne Loring	Scripps Research Institute	Autologous cell therapy for Parkinson's disease using iPSC-derived DA neurons	Quest - Discovery Stage Research Projects	$2,299,786
Fred Gage	Salk Institute for Biological Studies	Molecular and Cellular Transitions from ES Cells to Mature Functioning Human Neurons	Comprehensive Grant	$2,749,293
Birgitt Schuele	Parkinson's Institute	CRISPR/dCas9 mutant targeting SNCA promoter for downregulation of alpha-synuclein expression as a novel therapeutic approach for Parkinson's disease	Quest - Discovery Stage Research Projects	$1,288,415
J. William Langston	Parkinson's Institute	Using patient-specific iPSC derived dopaminergic neurons to overcome a major bottleneck in Parkinson's disease research and drug discovery	Early Translational I	$3,698,646
Justin Cooper-White	Scaled Biolabs Inc.	A tool for rapid development of clinical-grade protocols for dopaminergic neuronal differentiation of Parkinson's Disease patient-derived iPSCs	Quest - Discovery Stage Research Projects	$657,528
Evan Snyder	Sanford Burnham Prebys Medical Discovery Institute	Developmental Candidates for Cell-Based Therapies for Parkinson's Disease (PD)	Early Translational I	$5,190,752
Vicki Nienaber	Zenobia Therapeutics	A new phenotypic screening platform that identifies biologically-relevant targets and lead compounds for the treatment of Parkinson's disease	Inception - Discovery Stage Research Projects	$112,500
David Schaffer	University of California, Berkeley	Directed Evolution of Novel AAV Variants for Enhanced Gene Targeting in Pluripotent Human Stem Cells and Investigation of Dopaminergic Neuron Differentiation	Tools and Technologies I	$918,000
Krystof Bankiewicz	University of California, San Francisco	MRI Guided Delivery of Neural Progenitor Cells Secreting GDNF for the Treatment of Parkinson's disease	Late Stage Preclinical Projects	$5,811,340

Krystof Bankiewicz	Brain Neurotherapy Bio	A Phase 1b Safety Study for MRI guided delivery of AAV2-GDNF for the treatment of Parkinson's disease	Clinical Trial Stage Projects	$5,510,462	
					Total: $59,270,573.00

CIRM Parkinson's Disease Videos

 **Lorenz Studer, Winner of the 2017 Ogawa-Yamanaka Stem Cell Prize**	 **Suzanne Peterson, Scripps - CIRM Stem Cell #SciencePitch**	 **Jessica Westfall, The Parkinson's Institute - CIRM Stem Cell #SciencePitch**	 **Jeanne Loring, Scripps - CIRM Stem Cell #SciencePitch: Parkinson's Disease**
 **Parkinson's: Ask the Stem Cell Expert | Xianmin Zeng, Buck Institute**	 **Greg Wasson, Parkinson's Action Network: Patient Advocate Presentation**	 **Parkinson's Disease: Advancing Stem Cell Therapies - 2011 CIRM Grantee Meeting**	 **Stem Cells and Parkinson's Disease**
 **Progress and Promise in Parkinson's**	 **Spotlight on Parkinson's Disease: Seminar by Jeff Bronstein, M.D., Ph.D.**	 **Spotlight on Parkinson's Disease: Seminar by Arnold Kriegstein, M.D., Ph.D.**	 **Spotlight on Parkinson's Disease: Seminar by Bruce Wisnicki**

News and Information

- CIRM Stem Cellar Blog coverage on Parkinson's Disease
- Stories of Hope: Parkinson's Disease
- Are Parkinson's Disease Stem-Cell Therapies Finally Ready for Clinical Trials? It Depends, Some Say (Parkinson's News Today)

Resources

- NIH: Parkinson's Disease Information
- Find a clinical trial near you: NIH Clinical Trials database
- Parkinson's Disease Trials Listed on ClinicalTrials.gov
- National Parkinson Foundation
- American Parkinson Disease Association
- Parkinson's Disease Foundation
- Michael J. Fox Foundation for Parkinson's Research
- The Parkinson's Institute
- Family Caregiver Alliance
- National Family Caregivers Association
- The *Movement* Disorders Society
- GForce-PD: A Global Effort to Bring Cell Based Therapies to Parkinson's Disease Patients

Find Out More:

Stem Cell FAQ | Stem Cell Videos | What We Fund

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