

Autism Fact Sheet

CIRM funds many projects seeking to better understand autism and to translate those discoveries into new therapies.

Description

Autism is not one specific condition but a range of developmental disorders called Autism Spectrum Disorders (ASD) that can lead to difficulties with verbal and non-verbal communication, impaired social skills and other behavioral problems.

According to the Centers for Disease Control and Prevention 1 in 68 children has ASD, with boys four times more likely than girls to be affected. The precise cause of ASD is not known, however, there seems to be a strong genetic component to it.

While behavioral therapy has been shown to help those with ASD there is no cure. Some medications have shown effectiveness in easing symptoms but none treat the underlying cause of the problem.

CIRM funds several research projects investigating how the brain normally matures during development by studying stem cells as they differentiate or change into different types of nerves. This work could lead to a better understanding of how diseases such as autism form, and to future therapies.

Several CIRM-funded research projects are also using stem cells derived from people with ASD to learn more about how the symptoms of ASD arise and also to screen for drugs. These types of projects start with cells taken from people with ASD. Even if those cells come from the skin they have the same genes as cells in the brain that show symptoms in the disease. These cells can then be reprogrammed into an embryonic-like state called an induced pluripotent stem cell (iPSC), and matured into brain cells. Those resulting brain cells are genetically identical to brain cells in the person who donated the tissue.







CIRM-funded scientists have shown that these autism-like cells behave very differently in a lab dish than normal cells. Studying those cells can help scientists learn what goes wrong in the disease and guide them toward new therapies. What's more, the scientists can expose those cells to drugs and see which ones alleviate symptoms in the lab dish.

CIRM Grants Targeting Autism

Researcher name	Institution	Grant Title	Grant Type	Award Amount
Alysson Muotri	University of California, San Diego	A drug-screening platform for autism spectrum disorders using human astrocytes	Early Translational IV	\$1,656,456
David Segal	University of California, Davis	MSC delivery of an artificial transcription factor to the brain as a treatment for Angelman Syndrome	Quest - Discovery Stage Research Projects	\$1,055,319
Stuart Lipton	Scripps Research Institute	Drug Development for Autism Spectrum Disorder Using Human Patient iPSCs	Quest - Discovery Stage Research Projects	\$1,827,576
David Segal	University of California, Davis	AAV9-Cas13 gene therapy for Angelman syndrome	Quest - Discovery Stage Research Projects	\$1,364,903
Alysson Muotri	University of California, San Diego	Developing a drug-screening system for Autism Spectrum Disorders using human neurons	Early Translational II	\$1,376,198

Fred Gage	Salk Institute for Biological Studies	Development of Induced Pluripotent Stem Cells for Modeling Human Disease	New Cell Lines	\$1,737,720	
Fen-Biao Gao	Gladstone Institutes, J. David	MicroRNAs in Human Stem Cell Differentiation and Mental Disorders	SEED Grant	\$748,800	
Theo Palmer	Stanford University	Development of small molecule screens for autism using patient-derived iPS cells	Tools and Technologies II	\$1,797,606	
Marius Wernig	Stanford University	Cellular tools to study brain diseases affecting synaptic transmission	Tools and Technologies II	\$1,664,382	
Yi Sun	University of California, Los Angeles	Studying neurotransmission of normal and diseased human ES cell-derived neurons in vivo	Basic Biology III	\$1,382,400	
Anirvan Ghosh	University of California, San Diego	Investigation of synaptic defects in autism using patient-derived induced pluripotent stem cells	Basic Biology III	\$843,597	
Joachim Hallmayer	Stanford University	Induced pluripotent stem cells from children with autism spectrum disorders	Tissue Collection for Disease Modeling	\$530,265	
					Total: \$15,985,222.00

CIRM Autism Videos

 <p>Reversing Autism in the Lab with help from Stem Cells and the Tooth Fairy</p>	 <p>Autism and Stem Cells: A Mom's Perspective</p>	 <p>Seminar Intro: Autism, Toxicology, Environmental Health and Stem Cell Science</p>	 <p>Eric Roberts: Pesticides and Autism</p>
 <p>Michael McMaster: Human Embryonic Stem Cells for Predictive Toxicology</p>	 <p>Tracy Woodruff: So Many Chemicals...So Little Time</p>		

News and Information

- Autism blogs on the CIRM Stem Cellar
- One scientist's quest to understand Autism using stem cells (Interview with UCSD's Alysson Muotri)
- Summary and Recommendations of CIRM Autism Workshop [pdf]

Resources

- NIH: Autism Information Page
- CDC: Autism Facts
- Find a clinical trial near you: NIH Clinical Trials database

- National Autism Association
- Autism Society of America
- US Autism and Asperger Association
- Autism Speaks
- Family Caregiver Alliance
- National Family Caregivers Association

Find Out More:

[Stem Cell FAQ](#) | [Stem Cell Videos](#) | [What We Fund](#)

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