MSC delivery of an artificial transcription factor to the brain as a treatment for Angelman Syndrome

Grant Award Details

MSC delivery of an artificial transcription factor to the brain as a treatment for Angelman Syndrome

Grant Type: Quest - Discovery Stage Research Projects

Grant Number: DISC2-09032

Project Objective: MSC-delivered artificial transcription factor to the brain as a treatment for Angelman Syndrome

Investigator:

<table>
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<tr>
<th>Name:</th>
<th>David Segal</th>
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<tr>
<td>Institution:</td>
<td>University of California, Davis</td>
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<td>Type:</td>
<td>PI</td>
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Disease Focus: Autism, Neurological Disorders

Human Stem Cell Use: Adult Stem Cell

Award Value: $1,087,572

Status: Active

Grant Application Details

Application Title: MSC delivery of an artificial transcription factor to the brain as a treatment for Angelman Syndrome
**Public Abstract:**

**Research Objective**

Mesenchymal stem cells will be used to deliver an artificial transcription factor to neurons in the brain to treat a genetic disease.

**Impact**

It could lead directly to a treatment for Angelman Syndrome, but the approach could be used to alter gene expression in almost any brain disorder. It could overcome the brain delivery bottleneck.

**Major Proposed Activities**

- Prepare the MSC delivery system (month 1 – month 6)
- Rescue and analysis of on-target molecular phenotypes in “YFP-mice” (month 6 – month 12)
- Rescue and analysis of the behavioral phenotypes in “AS-mice” (month 12 – month 24)
- Analysis of the off-target molecular phenotypes in “YFP-mice” (month 18 – month 24)

**Statement of Benefit to California:**

Brain disorders are responsible for more years lost to disability than any other medical condition. For example, autism spectrum disorder (ASD) in the US is estimated to affect 1 in 68 children. The need for effective treatments can not be understated. Molecular therapeutics pioneered to understand and treat rare single-gene disorders such as Angelman Syndrome will provide the tools and methods that will ultimately be used to address the more common complex brain disorders.