Human Neural Progenitors Secreting Glial Cell Line-Derived Neurotrophic Factor (CNS10-NPC-GDNF) for the Treatment of Amyotrophic Lateral Sclerosis

Grant Award Details

Grant Type: Clinical Trial Stage Projects
Grant Number: CLIN2-09284
Project Objective: Phase 1/2a clinical trial

Investigator:

<table>
<thead>
<tr>
<th>Name</th>
<th>Clive Svendsen</th>
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<tbody>
<tr>
<td>Institution</td>
<td>Cedars-Sinai Medical Center</td>
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<td>Type</td>
<td>PI</td>
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Disease Focus: Amyotrophic Lateral Sclerosis, Neurological Disorders

Human Stem Cell Use: Adult Stem Cell

Award Value: $6,154,067

Status: Active

Grant Application Details

Application Title: Human Neural Progenitors Secreting Glial Cell Line-Derived Neurotrophic Factor (CNS10-NPC-GDNF) for the Treatment of Amyotrophic Lateral Sclerosis
Public Abstract: Therapeutic Candidate or Device
CNS10-NPC-GDNF - a neural progenitor cell secreting GDNF

Indication
ALS

Therapeutic Mechanism
This therapy will replace damaged astrocytes. The new astrocytes will release paracrine factors. As the cells have been modified to release GDNF they will also provide this factor to dying motor neurons.

Unmet Medical Need
There is no treatment or cure for ALS. Thus there is a huge unmet medical need.

Project Objective
Phase 1/2a clinical trial

Major Proposed Activities
Assess clinical safety of the therapeutic product

Statement of Benefit to California:
ALS is a devastating disease and there are over 6,000 cases in CA. If this treatment works it will provide one of the only ways to slow down motor neuron disease progression. This illness costs the state of California millions of dollars in healthcare costs and immense suffering to those Californians affected by the disease.