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**Human Neural Stem Cells (hNSCs) for neuroprotection in perinatal hypoxic-ischemic brain injury (HII)-Pre-IND-enabling Studies**

**Grant Award Details**

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Human Neural Stem Cells (hNSCs) for neuroprotection in perinatal hypoxic-ischemic brain injury (HII)-Pre-IND-enabling Studies

**Grant Type:** Therapeutic Translational Research Projects

**Grant Number:** TRAN1-11628

**Project Objective:** Establishing route, timing, and dose of administration of human neural stem cell candidate, along with preliminary safety information, to conduct a pre-IND meeting.

**Investigator:**

<b>Name:</b>	Evan Snyder
<b>Institution:</b>	Sanford Burnham Prebys Medical Discovery Institute
<b>Type:</b>	PI

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**Disease Focus:** Brain Injury, hypoxic, ischemic, Neurological Disorders, Pediatrics

**Human Stem Cell Use:** Adult Stem Cell

**Award Value:** \$4,963,684

**Status:** Active

**Grant Application Details**

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**Application Title:** Human Neural Stem Cells (hNSCs) for neuroprotection in perinatal hypoxic-ischemic brain injury (HII)-Pre-IND-enabling Studies

**Public Abstract:****Translational Candidate**

An established stable human neural stem cell line unmanipulated genetically & propagated under defined conditions

**Area of Impact**

Perinatal asphyxia (also called hypoxic-ischemic injury), a major untreatable cause of cerebral palsy & cognitive disability

**Mechanism of Action**

hNSCs rescue the penumbra, the part of the brain lesion following perinatal asphyxia that still has viable though endangered cells. Such rescue includes preserving tissue; host neuron growth; revascularization; inhibiting inflammation & scarring. Anatomic & behavioral improvement results. If strategically administered, hNSCs can supply their neuroprotective molecules in a manner that synergizes with standard-of-care, hypothermia, which is only marginally effective but must be offered to babies.

**Unmet Medical Need**

Perinatal hypoxic-ischemic brain injury is an untreatable common cause of CP & disability. Hypothermia (HT) is standard-of-care for this condition although it is only marginally-effective. Any new trial must include HT. We will coordinate hNSC administration to synergize with HT & improve outcome.

**Project Objective**

Pre-IND meeting, ultimately a Phase 1b/2a trial

**Major Proposed Activities**

- Ascertain the proper timing of hNSC administration in relation to hypothermia to achieve synergy
- Determine the manufacturing specifications & biodistribution of the hNSCs in anticipation of IND-enabling studies
- Preparation of a pre-IND package

**Statement of Benefit to California:**

Perinatal asphyxia occurs in 2-4/1000 births. Despite hyperthermia (which is only marginally effective), 80% of asphyxiated infants develop neurologic signs with 10-20% remaining significantly impaired (e.g., CP; disability; epilepsy). The cost to California economy is \$1M/child in terms of lifelong medical & rehabilitative care; the impact on family dynamics is 2-5-fold greater than that. We believe stem cell-based interventions can improve these outcomes.

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