UC San Diego
UC San Diego News Center

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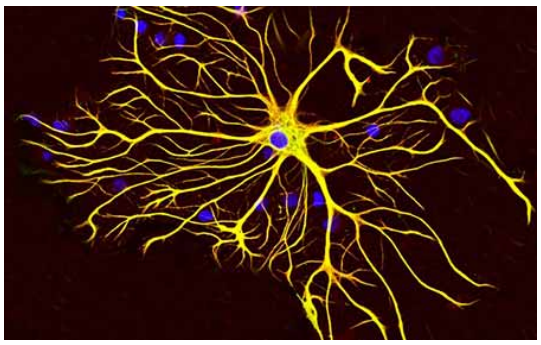
CIRM Grant to Fund Proposed Stem Cell Trials for ALS

The Independent Citizens Oversight Committee of the California Institute for Regenerative Medicine approved yesterday a $6.3 million grant to a research team from the University of California, San Diego School of Medicine and University of California, Davis to pursue a novel human embryonic stem cell-based therapy to rescue and restore neurons devastated by amyotrophic lateral sclerosis or ALS.



A human astrocyte grown in tissue culture. The blue stain indicates DNA inside the astrocyte's nucleus and in other nearby cells. Photo by Wikimedia/Greg Shaw

The team is headed by Lawrence Goldstein, PhD, Distinguished Professor in the departments of Neuroscience and Cellular and Molecular Medicine and director of both the UC San Diego Stem Cell Program and Sanford Stem Cell Clinical Center.

ALS is also known as Lou Gehrig's disease, named after the New York Yankee baseball player famously stricken by the disorder. The condition involves the progressive degeneration of spinal cord motor neurons, which control muscle movement, leading to paralysis and respiratory failure.

In Gehrig's day (he died in 1941, two weeks before his 38th birthday), a diagnosis of ALS meant a debilitating and lingering death. Not much has changed. While there are mild palliative treatments, there are currently no known effective treatments to slow or mitigate symptoms of ALS. A cure, said Goldstein, may be years away.

"A lot of people and institutions are working on ALS to understand it and resolve it at a cellular level, often targeting the motor neurons themselves. That's good," said Goldstein. "Our view is that it's critically important that we develop an aggressive set of cell therapy programs so that

we have multiple ‘shots on goal’ in parallel. We need to attack the disease from as many angles as possible.”

In this case, the angle of attack involves astrocytes, star-shaped glial cells in the brain and spinal cord that support and promote the health of neurons by, for example, providing nutrients or serving as impulse transmission regulators.

Goldstein and others argue that the decline and death of supporting astrocytes is central to the development and progression of ALS. They note that cellular and animal studies have shown that the introduction and augmentation of non-diseased astrocytes (and perhaps other cell types) in the vicinity of dying motor neurons significantly enhances the latter’s survival.

Building upon past research, scientists will conduct pre-clinical studies in advance of proposed human trials that would transplant neuronal stem cells derived from an established and well-understood human embryonic stem cell line into ALS patients. The neuronal stem cells would develop as astrocytes, provide new support for ailing motor neurons, thus extending their lives and function.

“Maintaining motor neuron survival would add years of fulfilling life to ALS patients,” Goldstein said.

The research will be conducted at the Alpha Stem Cell Clinic at UC San Diego Health, one of three alpha clinics in a statewide CIRM network intended to create long-term infrastructure for clinical trials of stem cell-related drugs and therapies in humans. The UC San Diego alpha clinic, the cell therapy arm of the Sanford Stem Cell Clinical Center, is currently conducting first-in-human stem cell clinical trials for spinal cord injury, diabetes and leukemia.

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