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Editor's Summary

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Disease mimicked in culture

The inherited disease spinal muscular atrophy (SMA), one of the most common neurological disorders causing death in childhood, is caused by mutations in both copies of the *SMN1* gene. Little is known about SMA pathogenesis, partly because it is unique to humans who have two versions of this gene — *SMN1* and *SMN2*; rodents and other lab model candidates have just one. Now a new technique has been developed that creates a tool for studying SMA disease pathology at the cellular level. Skin fibroblasts from a child with SMA (and for comparison from his unaffected mother) were used to generate induced pluripotent stem (iPS) cell lines. They form neural progenitor cultures that can produce differentiated neural tissue and motor neurons that maintain the disease phenotype. The cultures also responded to drugs known to elevate the mutated protein associated with the disease. Similar iPS technology may be of value in the study of other genetic disorders such as Huntington's disease.

NEWS AND VIEWS

Stem cells: Tailor-made diseased neurons

How can we investigate a disease affecting neurons, which cannot be isolated from patients for analysis? As the study of one neurological disorder shows, a first step might be to make patient-specific neurons.

Michael Sendtner

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ARTICLE

Induced pluripotent stem cells from a spinal muscular atrophy patient

Allison D. Ebert, Junying Yu, Ferrill F. Rose, Jr, Virginia B. Mattis, Christian L. Lorson, James A. Thomson & Clive N. Svendsen

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