Metabolismo, Sistemas Modelo y Terapias para la ELA. Tercer Encuentro Internacional de Investigación en ELA en España

Metabolism, Model Systems and Therapies for ALS

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ABSTRACT

Murine models of ALS

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Motor neuron disease, also known as amyotrophic lateral sclerosis, is a relentless mid-life onset form of neurodegeneration that is currently incurable. It starts focally and leads to paralysis and death, typically within 3 years of diagnosis. Mouse models are critical to further our understanding of disease processes. To try to faithfully recapitulate disease pathogenesis, we have a long-term interest in creating knock in (KI) mouse models of ALS.

Here, together with information on other models that have been produced by others, we present data from KI models of three key ALS genes (Sod1, Tardbp and Fus) developed by us over the last few years: the Sod1D83G mouse; the FUS Delta14 mouse; an allelic set of Tardbp (TDP-43) mutants. However, there are still many challenges, particularly how to improve translation into the clinic.

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