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Amyotrophic Lateral Sclerosis and therapy development: Where are we and where are we going?

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Amyotrophic lateral sclerosis (ALS) is a fatal neurodegenerative disorder that causes injury and death of lower motor neurons in the brainstem and spinal cord, and of upper motor neurons in the motor cortex. The progressive failure of the neuromuscular system usually leads to death from respiratory failure. ALS occurs in both familial and sporadic forms, which are clinically undistinguishable, with 90% of cases being sporadic in origin and about 2% being linked to mutations in the gene encoding for the enzyme superoxide dismutase 1 (SOD1). For the past 20 years ALS research has focused on the study of SOD1 disease models, which still have not led to the development of any effective therapy and at present the only FDA approved drug available to patients is Riluzole. However, the past decade has seen the discovery of new genes associated to ALS, i.e. TARDBP, FUS and C9ORF72, which have opened new perspectives and have led to the development of new disease models. At the same time the breakthrough that skin fibroblasts from patients could be 'reprogrammed' to create pluripotent stem cells and neuronal progenitors could be isolated from post-mortem tissues and differentiated into neurons and astrocytes have provided us with a unique and invaluable model of sporadic disease. These cells can be used for high throughput screenings such as microarray analysis, drug test and shRNA library screenings. The combination of these new tools is likely to mark the beginning of a new era in ALS research, leading to the discovery of new therapeutic targets.

Biography

Laura Ferraiuolo graduated in Biotechnology with a dissertation on Amyotrophic Lateral Sclerosis (ALS) in 2004. In 2005 she joined the Department of Neuroscience at the University of Sheffield, UK, and won the MND Association Prize studentship that funded her PhD under the supervision of Prof. Pamela Shaw. She was awarded the Peake Fellowship at the Sheffield Institute for Translational Neuroscience from 2009 to 2011, to investigate the crosstalk between motoneurons and astrocytes in ALS using microarray analysis and cellular models. In 2012 Laura joined Dr. Kaspar's group after winning the prestigious Marie Curie Fellowship. She has reviewed several manuscripts and grants to date.

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