

STRATALS

Stratification of presymptomatic amyotrophic lateral sclerosis: the development of novel imaging biomarkers

Amyotrophic lateral sclerosis (ALS), also known as Charcot's disease, is a severe neurodegenerative disease occurring between the ages of 50 and 70 years. It causes progressive paralysis of the limbs as well as muscles of speech and swallowing. There is no effective treatment and death occurs by respiratory failure after 3 to 5 years. There is an urgency to develop new treatments, a limit being the extreme variability of the disease in its causes (there are hereditary and non-familial forms) or its clinical presentations. It is essential to characterize these different forms, in order to propose a personalized treatment. A pre-symptomatic period is now well characterized in subjects who carry a mutation responsible for familial forms of ALS and who have not yet developed the disease.

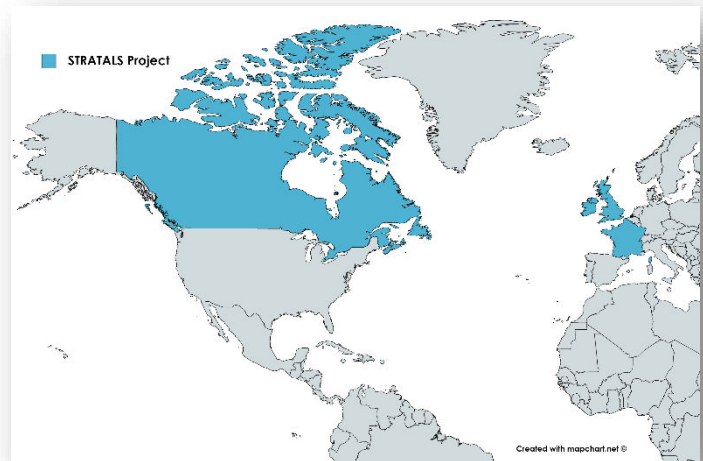
Recent developments in therapy, including gene therapy approaches, which are currently evaluated in symptomatic patients, open for the first time the possibility to prevent the disease or at least delay its onset. To achieve this goal, we need to develop new tools that will allow us to characterize this pre-symptomatic phase and also to monitor the effect of these new therapies. New imaging methods now offers the possibility of knowing when the first lesions begin and how they evolve over time. In this project, we will develop new imaging tools that allow even finer analysis of the changes occurring in the nervous system, especially in the spinal cord, which is the main site of abnormalities. We will apply these methods to individuals carrying a mutation causing ALS, whether they are pre-symptomatic or symptomatic, and will monitor their evolution over time.

Thanks to this project we will be able to better predict the evolution and to design the clinical trials with drugs under development or future therapies.

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