2015 Ice Bucket Call for Clinical Projects

RAP-ALS – *Rapamycin treatment for Amyotrophic Lateral Sclerosis*

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PROJECT VALUE	**426.825 euro**
DURATION	**24** months
PROJECT OBJECTIVES	In recent years different possible pathogenetic mechanisms for ALS has been postulated, i.e. the accumulation of altered proteins within the neurons and dysfunction of the immune response, which assumes neurotoxic characteristics rather than protective. In cellular and animal models Rapamycin has proved capable of promoting the removal of altered proteins and suppressing the inflammatory neurotoxic response. Rapamycin has never been tested in ALS patients and its ability to reach the central nervous system nor the best dosage for therapeutic purposes has never been verified. The main purpose of this clinical study is to verify Rapamycin ability to modify the expression of biological markers of inflammation in ALS patients compared to controls. Rapamycin safety and tolerability will be also evaluated in ALS patients; the lowest dose of the drug able to cross the blood-brain barrier and enter the central nervous system will be determined as well as some inflammation and immune response markers. The proposed study is a randomized phase II, single-blind, placebo-controlled, multi-centric trial, which is expected to enroll 63 ALS patients in eight Italian centers. The enrolled subjects will be divided into 3 groups, treated with two different doses of the drug. The treatment will last 18 weeks and follow-up post-treatment will last 36 weeks. This study could timely deliver relevant information about Rapamycin safety and tolerability in order to eventually develop future clinical trials to test the efficacy of the treatment in ALS patients. The trial will also provide important data about the role of autophagy and immune system in the pathogenesis of the disease