

Preliminary results of intravenous trehalose for the treatment of Spinocerebellar Ataxia type 3 (SCA3)

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Background: Trehalose is a disaccharide with protein stabilizing and autophagy enhancing properties. It showed efficacy in reducing abnormal protein aggregation in animal models of several human poly A- and poly Q- mediated hereditary neurological disorders. In animal models of SCA3, oral trehalose showed reduction of lesion size in a lentivirus model and improved motor function in an ongoing study in transgenic animals. The safety and pharmacokinetics of high dose IV trehalose in humans was recently studied in patients with Oculopharyngeal Muscular Dystrophy (OPMD).

Objective: To report preliminary results of a phase 2 trial of trehalose 9% IV in SCA3 patients.

Design and Methods: This is a proof of concept, phase 2 study to determine and compare the safety, tolerability and efficacy of 52 weekly IV administration of Trehalose 9% IV solution, 13.5 gr vs 27 gr in SCA3 patients. Primary endpoints will be the SARA score with several secondary endpoints (NESSCA scale, 9 hole peg test, 8 meters walk). Safety and tolerability is assessed by various clinical and laboratory tests.

Results: We have recruited 15 clinically and genetically confirmed SCA 3 patients who are currently under treatment. No drug-related adverse events were noted. This is in accordance with the reported safety profile of our OPMD study. Efficacy data is very limited data and at the moment is not reportable.

Conclusions: Based on these preliminary findings, trehalose 9% IV solution seems to be safe in humans. A further planned phase 2b multi-center clinical trial in SCA3 will be discussed.