IONIS FUSION trial with FUS ASO

A multicenter, double blind randomized placebo-controlled phase I-III study to evaluate the effect, safety, pharmacokinetics and pharmacodynamics of intrathecally administered ION363 in patients with ALS with a FUS mutation.

This phase I-III study is sponsored by IONIS Pharmaceuticals and investigates if lowering of FUS protein levels by antisense oligonucleotides against FUS mRNA results in a beneficial effect in ALS patients with a FUS mutation.

FUS mutations are a rare hereditary cause of ALS and in these patients a clustering of FUS protein is observed. ION363 is an antisense oligonucleotide or ASO that is intrathecally administered to diminish the synthesis of the FUS protein. A 100 mg dose is investigated in this study; after a few upload doses the medicine will be administered once every 3 months. In the first phase, 1 out of 3 patients will receive placebo. A second phase of the study is open label in which all patients will receive the active product. Rapid progressing patients can accelerated enter the second phase of the study. Worldwide, 50-60 patients will be included.

More information about this studie can be found on https://clinicaltrials.gov/ct2/show/NCT04768972?term=ion363&cond=ALS&draw=2&rank=1

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