GM604 Phase 2A Randomized Double-blind Placebo Controlled Pilot Trial in ALS

Study Focus:
Study Objectives are: 1. To test the safety and tolerability of GM604 in a population of ALS patients. 2. To test for changes in ALS biomarkers before and after treatment. 3. To determine preliminary effects of injections of GM604 on measures of ALS disease biomarkers and clinical progression.

Disease:
Amyotrophic Lateral Sclerosis (ALS), Sporadic ALS, Familial ALS

Study Category:
Drug Trial

Study Status:
Active, not yet recruiting

Phase:
Phase II

Type:
Interventional Trial with active agents/drugs & a placebo

Study Summary:
This pilot trial is designed to test proof of principle, i.e. determine if a 2-week IV bolus treatment with this agent can (1) change ALS protein expression (target biomarkers and efficacy biomarkers) after treatment (2) have preliminary effects measures of ALS disease clinical progression.

GM604 is an endogenous human embryonic stage neural regulatory and signaling peptide that controls the development, monitoring and correction of the human nervous system. Neurological diseases are multisystem, multifactorial, and single target drugs are ineffective. Genervon’s Master Regulators play a significant role in embryonic/fetal nervous system development and are potent disease modification drug candidates modulating many pathways including inflammation, apoptotic, hypoxia. The study drug is a regulatory peptide with a sequence identical to one of the active sites of human Motoneuronotrophic Factor and is manufactured by solid phase synthesis. Preclinical research indicates it to be a neuro-protective agent in animal models of ALS, motor neuron diseases, PD, other neuro-degenerative diseases and stroke. GM604 controls and modulates over many known and significant ALS genes with positive effects interactively and dynamically through multiple pathways, and up to twenty-two biological processes, including neuro-protection, neurogenesis, neural development, neuronal signaling, neural transport, and other processes. GM6 is not a cocktail of drugs, but one master regulator peptide drug that functions through multiple pathways. Genervon hypothesized that studying the biomarkers of protein expressions of these ALS genes such as SOD1 and the protein expression of substances such as tau, NF-H, Cystatin C which were indications of degeneration of neuron in the CSF collected from ALS patients will provide information of the possible GM604’s mechanism of action in treating ALS.

Participant Duration:
12 weeks

# of Subjects:
12

Enrollment Start Date:
07/01/2013

Date Study Added to alscortium.org:
05/29/2013

Funding Source:
Genervon Biopharmaceuticals, LLC

Study Chair(s)/Principal Investigator(s):
Genervon Biopharmaceuticals, LLC
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Clinicaltrials.gov ID:
NCT01854294

NEALS Affiliated?
No

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Eligibility Criteria

Gender:
Male & Female

Minimum Age:
18

Min Vital Capacity (% predicted normal): 65%

Time since Symptom Onset:
Less than 24 months

Time since Diagnosis:
Not applicable

Can participants use Riluzole?
Yes

Other Eligibility Criteria:

Inclusion Criteria:
1. Patients with ALS: Familial and Sporadic ALS, with symptom onset ≤ or equal to 24 months.
2. At least 18 years of age
3. Subjects meet the El Escorial criteria of definite criteria for a diagnosis of ALS.
4. Subjects can be on a stable dose of riluzole for at least a month or not taking or initiating riluzole for the duration of the trial.
5. Not on any experimental medication for the last 1 month or five times the half-life of experimental medication.
6. At screening, must have a Forced Vital Capacity (FVC) ≥ 65% of predicted capacity for age, height and gender.
7. Have fully completed informed consent form
8. Ability to comply with study procedures
9. Women of child-bearing age must be on birth control. Pregnancy test should be done in women in child bearing age.
10. Medically safe to have lumbar puncture to collect CSF

Exclusion Criteria:
1. History of liver disease, severe renal failure, diabetes, coronary heart disease, cancer
2. Clinically significant EKG abnormality at screening
3. Any comorbid condition which would make completion of the trial unlikely
4. FVC < 65%
5. Presence of a bleeding disorder
6. Allergy to local anesthetics
7. Problem with CSF pressure
8. Topical or other skin infection at the lumbar puncture site
9. BMI > 32 kg/m2
10. Medical or surgical conditions in which a lumbar puncture is contraindicated
11. Use of any anti-platelet or anticoagulant drugs, such as plavix, aggrenox, ticlid, warfarin or coumadin