Phase 2A Study of GM 608 in Mild to Moderate Parkinson Disease (GAP-PD)

This study is currently recruiting participants.

Verified May 2013 by Genervon Biopharmaceuticals, LLC

Sponsor:
Genervon Biopharmaceuticals, LLC

Collaborator:
Columbia University

Information provided by (Responsible Party):
Genervon Biopharmaceuticals, LLC

ClinicalTrials.gov Identifier:
NCT01850381

First received: May 7, 2013
Last updated: May 8, 2013
Last verified: May 2013
History of Changes

Purpose

GM608 is an endogenous human embryonic stage neural regulatory and signaling peptide that controls the development, monitoring and correction of the human nervous system. The study drug is an oligopeptide 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 PD, other neuro-degenerative diseases and stroke. This trial is designed to test proof of principle, i.e. determine if a 2-week treatment with this agent can restore the non-functioning nigral dopaminergic neurons in PD over a 3 month period, during which the placebo-treated arm is expected to have little or no worsening of the total UPDRS (Unified Parkinson's Disease Rating Scale)score compared to baseline.

Study Objectives are:

1. To compare the safety and tolerability of GM608 with placebo in a population of patients with early PD.
2. To field test the study procedures for feasibility and efficiency
3. To determine if there is any hint that injections of GM608 might slow the rate of clinical worsening of PD.

Condition	Intervention	Phase
Parkinson's Disease	Drug: GM608	Phase 2

Study Type: Interventional

Study Design: Allocation: Randomized
Endpoint Classification: Safety/Efficacy Study
Intervention Model: Parallel Assignment
Masking: Double Blind (Subject, Caregiver, Investigator, Outcomes Assessor)
Primary Purpose: Treatment

Official Title: GM 608 in A Phase IIA Pilot Double-blinded, Randomized, Placebo Controlled Trial in Mild to Moderate Parkinson Disease

Resource links provided by NLM:

Genetics Home Reference related topics: Parkinson disease Perry syndrome

MedlinePlus related topics: Degenerative Nerve Diseases Parkinson's Disease

U.S. FDA Resources

Further study details as provided by Genervon Biopharmaceuticals, LLC:

Primary Outcome Measures:

- The change from the mean total UPDRS score of the combined screening and baseline visits to the total UPDRS score at the Week-12 visit, comparing treated with placebo.Compare safety and tolerability with placebo. [Time Frame: Baseline, week 2, week 6, week 12]
  [Designated as safety issue: Yes]

Secondary Outcome Measures:

- Change in total UPDRS between the mean screening-baseline visits and end of Week 2 (at visit 6 after dosing), and week 6, comparing the two arms of the study encompassing the entire cohort of 6 subjects. [Time Frame: Baseline, week 2, week 6]
  [Designated as safety issue: No]
- Change in UPDRS sub-scores (Mental, Activities of Daily Living, Motor) between the mean screening-baseline visits and ends of Week 2 (at visit 6 after dosing), 6, and 12, comparing the two arms of the study encompassing the entire cohort of 6 subjects. [Time Frame: Baseline, week 2, week 6, week 12] [Designated as safety issue: No]
- Time to the development of sufficient disability to require a change in symptomatic therapy. [Time Frame: 12 weeks]
  [Designated as safety issue: No]
- Proportion of subjects requiring additional symptomatic treatment due to disability. [Time Frame: 12 weeks]
  [Designated as safety issue: No]
- Change in Schwab & England ADL score from baseline to Week 2 (at visit 6 after dosing), 6, or 12. [Time Frame: Baseline, week 2, week 6, week 12] [Designated as safety issue: No]
- Change in Hoehn & Yahr score (H&Y), Beck Depression Inventory (BDI), and Montreal Cognitive Assessment (MOCA) scores from baseline to Week 2 (at visit 6 after dosing), 6, or 12. [Time Frame: Baseline, week 2, week 6, week 12] [Designated as safety issue: No]

Other Outcome Measures:

- Secondary analyses will consider a comparison of slopes using a mixed-model approach with treatment as a fixed effect and subject-specific slopes as a random effect. [Time Frame: 12 weeks] [Designated as safety issue: No]

Estimated Enrollment: 6

Study Start Date: May 2013
Estimated Study Completion Date: June 2014
Estimated Primary Completion Date: December 2013 (Final data collection date for primary outcome measure)

Arms	Assigned Interventions
Active Comparator: GM608 320 mg of GM608 will be administered intravenously as a slow bolus, once a day for 3 times a week for 2 weeks.	Drug: GM608 For GM608: 320 mg/dose reconstituted with bateriostatic saline, IV (in the vein) bolus over 1 min, on Monday, Wednesday, Friday for two consecutive weeks. For Placebo, matching volume of bacteriostatic saline, IV (in the vein) bolus over 1 min, on Monday, Wednesday, Friday for two consecutive weeks. Other Names: • GM602 • GM6 • GM604 • MNTF 6mer
Placebo Comparator: Matching Placebo (Bacteriostatic saline) matching placebo will be administered intravenously as a slow bolus, once a day for 3 times a week for 2 weeks.	

Show Detailed Description

Eligibility

Ages Eligible for Study: 30 Years and older
Genders Eligible for Study: Both
Accepts Healthy Volunteers: No

Criteria

Inclusion Criteria:

- with mild-moderate idiopathic PD diagnosed based on UK PD Brain Bank criteria.
- Age > 30
- Motor UPDRS Score ≥ 15
- Hoehn & Yahr stage <3
- Diagnosis of PD <10 years
- Have fully completed informed consent form
- May be on antiparkinsonian medications of an MAO-B inhibitor, an anticholinergic, or amantadine, but not levodopa or dopamine agonist

Exclusion Criteria:

- Patients with atypical parkinsonism (such as suspected PSP, MSA or CBD) and secondary parkinsonism (such as NPH, drug-induced, or vascular parkinsonism).
- Patients with uncertainty as to having classical Parkinson disease, such as those who might have scans without evidence of dopaminergic deficit (SWEDDs)
- Patients not willing to give an informed consent
- Patients who are on a dopaminergic medication (levodopa or dopamine agonist)
- Presence of a medical or psychiatric comorbidity that can compromise participation in the study

Contacts and Locations

Please refer to this study by its ClinicalTrials.gov identifier: NCT01850381

Contacts

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Locations

United States, New York

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Principal Investigator: Stanley Fahn, MD

Sponsors and Collaborators

Genervon Biopharmaceuticals, LLC

Columbia University

Investigators

Principal Investigator: Stanley Fahn, MD Columbia University Medical Center/NY Presbyterian Hospital

More Information

No publications provided

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ClinicalTrials.gov Identifier:	NCT01850381 History of Changes
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Study First Received:	May 7, 2013
Last Updated:	May 8, 2013
Health Authority:	United States: Food and Drug Administration

Keywords provided by Genervon Biopharmaceuticals, LLC:

Parkinson Disease	human Motoneuronotrophic Factor
Efficacy	neuroprotective agent
Safety	neurodegenerative diseases
Tolerability	rate of clinical progression of PD
Phase 2A	UPDRS
Pilot trial	Proof of principle

Additional relevant MeSH terms:

Parkinson Disease
Parkinsonian Disorders
Basal Ganglia Diseases
Brain Diseases
Central Nervous System Diseases
Nervous System Diseases
Movement Disorders
Neurodegenerative Diseases
Neuroprotective Agents
Protective Agents
Physiological Effects of Drugs
Pharmacologic Actions
Central Nervous System Agents
Therapeutic Uses

ClinicalTrials.gov processed this record on June 06, 2013