Efficacy and Safety of NFC-1 in Adolescents With Genetic Disorders Impacting mGluR and ADHD

This study is not yet open for participant recruitment. (see Contacts and Locations)

Verified May 2016 by Medgenics, Inc.

Sponsor:
Medgenics, Inc.

Information provided by (Responsible Party):
Medgenics, Inc.

ClinicalTrials.gov Identifier:
NCT02777931

First received: May 13, 2016
Last updated: May 18, 2016
Last verified: May 2016

History of Changes

- Full Text View
- Tabular View
- No Study Results Posted
- Disclaimer
- How to Read a Study Record

Purpose
This is a randomized, double-blind, placebo-controlled, parallel-group study of NFC-1 versus placebo in adolescents with ADHD who have genetic disorders impacting mGluRs.

<table>
<thead>
<tr>
<th>Condition</th>
<th>Intervention</th>
<th>Phase</th>
</tr>
</thead>
<tbody>
<tr>
<td>Attention Deficit Disorder With Hyperactivity</td>
<td>Drug: NFC-1</td>
<td>Phase 2</td>
</tr>
<tr>
<td></td>
<td>Drug: Placebo</td>
<td>Phase 3</td>
</tr>
</tbody>
</table>

Study Type: Interventional

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

Official Title: A Multicenter, 6-week, Double-blind, Randomized, Placebo-controlled, Parallel-design Study to Assess the Efficacy and Safety of NFC-1 in Adolescents (Ages 12-17) With Genetic Disorders Impacting Metabotropic Glutamate Receptors and ADHD

Resource links provided by NLM:
Further study details as provided by Medgenics, Inc.:

Primary Outcome Measures:
- Change from baseline in ADHD-rating scale (ADHD-RS-5) Total Score [Time Frame: Baseline to Visit 8 (Week 6)] [Designated as safety issue: No]

Secondary Outcome Measures:
- Change from baseline in Clinical Global Impression - Global Improvement (CGI-I) Scale. [Time Frame: Baseline to Visit 8 (Week 6)] [Designated as safety issue: No]

Estimated Enrollment: 90

Study Start Date: June 2016

Estimated Study Completion Date: November 2016

Estimated Primary Completion Date: October 2016 (Final data collection date for primary outcome measure)

### Arms
<table>
<thead>
<tr>
<th>Arms</th>
<th>Assigned Interventions</th>
</tr>
</thead>
<tbody>
<tr>
<td>Experimental: NFC-1</td>
<td>Drug: NFC-1</td>
</tr>
<tr>
<td>Doses of NFC-1 will be administered as 100, 200, or 400 mg twice daily as capsules for oral administration.</td>
<td>NFC-1 is supplied as size 2 hard gelatin capsules. Other Name: NFC1</td>
</tr>
<tr>
<td>Matching placebo capsules.</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Placebo Comparator: Placebo</th>
<th>Drug: Placebo</th>
</tr>
</thead>
<tbody>
<tr>
<td>Matching placebo capsules</td>
<td>Matching placebo capsules</td>
</tr>
<tr>
<td>Other Name: Control</td>
<td>Other Name: Control</td>
</tr>
</tbody>
</table>

Detailed Description:
This is a randomized, double-blind, placebo-controlled, parallel-group study of adolescents with ADHD who have genetic disorders impacting mGluRs. Approximately 90 subjects will receive randomized treatment with NFC-1 or placebo. Dosing will be optimized during the first 4 weeks of treatment, based on clinical response and tolerability, and maintained for an additional 2 weeks.

Eligibility

Ages Eligible for Study: 12 Years to 17 Years
Genders Eligible for Study: Both
Accepts Healthy Volunteers: No

Criteria
Inclusion Criteria:
Subject has ADHD as defined by the Diagnostic and Statistical Manual of Mental Disorders, 5th edition (DSM-5) and Version 5 of the Attention Deficit Hyperactivity Disorder Rating Scale (ADHD-RS-5) ≥ 28 at Baseline with or without conventional ADHD therapy.

Subject has an intelligence quotient (IQ) > 79, based on the Wechsler Abbreviated Scale of Intelligence, second edition (WASI-II).

Subject has been genotyped previously and determined to have disruptive mutations in genes within the glutamate receptor metabotropic (GRM)-network as determined by the presence of copy number variations (CNVs) (GRM biomarker-positive subjects). The confirmation of a subject's positive status will be provided by the sponsor.

Subject is judged to be in general good health, other than having ADHD, based on medical history, physical examination, vital signs measurements, laboratory safety tests, and the Columbia Suicide Severity Rating Scale (C-SSRS) performed at the Screening Visit and/or prior to administration of investigational product (IP).

Subject has no clinically significant abnormality on electrocardiogram (ECG) performed at the Screening Visit and/or prior to administration of IP such as serious arrhythmia, bradycardia, tachycardia, cardiac conduction problems, or other abnormalities deemed to be a potential safety issue.

Parent/legal guardian and subject understand the study procedures and agree to the subject's participation in the study as indicated by parental/legal guardian signature on the subject informed consent form and subject signature on the assent form.

Exclusion Criteria:

- Subjects with prior diagnosis of comorbid major psychiatric disorders (ie, aside from ADHD), including major depression, bipolar disease, schizophrenia, pervasive development disorder, and intellectual disability.

- Subject is currently taking a prohibited medication and/or is unwilling to wean off current ADHD medication to participate in the study.

- Subject has a history of any illness that in the opinion of the study investigator might confound the results of the study or poses an additional risk to the subject by his or her participation in the study.

- Subject has a known history or presence of syncope, cardiac conduction problems (eg, clinically significant heart block), exercise-related cardiac events including syncope and pre-syncope, or clinically significant bradycardia.

- Subject has a history of stroke, chronic seizures, or major neurological disorder which, in the opinion of the investigator, would interfere with the subject's ability to participate and/or be evaluated in the study.

Contact: Medgenics

Locations

United States, Maryland

The Clinical Trials Center at Kennedy Krieger Institute
Baltimore, Maryland, United States, 21205

**Sponsors and Collaborators**
Medgenics, Inc.

**Investigators**

**Study Director:**  Liza Squires, M.D.  Medgenics, Inc.

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**More Information**

**Responsible Party:**  Medgenics, Inc.

**ClinicalTrials.gov Identifier:**  NCT02777931  

**History of Changes**

**Other Study ID Numbers:**  MDGN-NFC1-ADHD-201

**Study First Received:**  May 13, 2016

**Last Updated:**  May 18, 2016

**Health Authority:**  United States: Food and Drug Administration

Additional relevant MeSH terms:

- Attention Deficit Disorder with Hyperactivity
- Attention Deficit and Disruptive Behavior Disorders
- Mental Disorders
- Neurodevelopmental Disorders

ClinicalTrials.gov processed this record on May 22, 2016