Dear Colleague,

A new clinical trial for patients with the 22q11.2 deletion syndrome (22q11DS) has recently started. Centers in the United States and Canada are participating. Pediatric patients with 22q11DS and symptoms of anxiety, ADHD, and/or ASD are eligible. A brief outline of the study is provided in this leaflet.

> ***Only one (1) visit to the investigative site is required. The remainder of the study visits are conducted by telemedicine and/or home health nurses.***

We would appreciate you contacting the nearest study center if you have any patients that may be appropriate. Study centers can be located at: https://www.nobiastx.com/ or www.clinicaltrails.gov (Identifier: NCT05290493)

## Study Design

This is a phase 2, randomized, double-blind, placebo-controlled, crossover study of NB-001 (fasoracetam) in pediatric patients with 22q11DS.

## Participating Investigators/Trial Sites:

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**Lead Investigator*

Protocol NB-001-01

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## More Information

**Website** www.nobiastx.com



Protocol NB-001-01

# Now Enrolling

## A Randomized, Placebo-Controlled Crossover Trial to Assess the Safety and Efficacy of NB-001 in Children and Adolescents with 22q11.2 Deletion Syndrome

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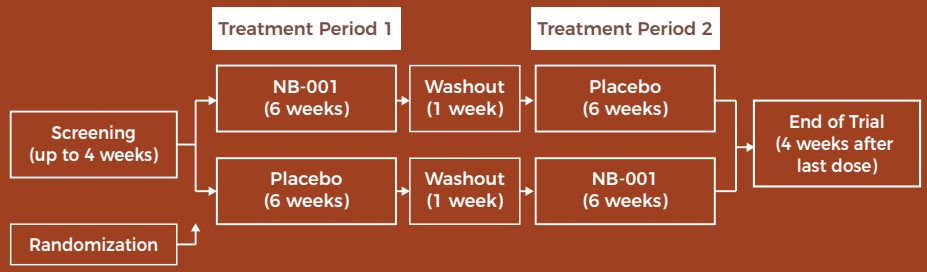
## Crossover Trial Schematic

**Double-Blind Treatment Phase: 13 weeks**




## Key Inclusion Criteria

**The patient must match *all* inclusion criteria:**

1. A genotype with a pathologic deletion in the 22q11 region confirmed by documentation (e.g., genetic test results).
2. 6 to 17 years old
3. A CGI-S scale score of ≥4 at Screening. Note that the Severity score of 4 could be from a composite of 2 or more sub-threshold scores.

   And either:

   a. Psychiatric symptoms in the clinical range for **at least 1 of 3 disorders**: anxiety disorder, attention deficit hyperactivity disorder (ADHD), or autism spectrum disorder (ASD)

   OR:

   b. Psychiatric symptoms in the **subclinical range for at least 2 of 3 disorders**: anxiety disorder, ADHD, or ASD
4. Contraception: Female patients of childbearing potential must use dual effective/highly effective methods of contraception during the trial.
5. Must provide written informed consent and assent (if applicable) and agree to comply with the trial protocol.

## Key Exclusion Criteria

**Patient must not match *any* of the exclusion criteria:**

1. A history of psychotic symptoms, current psychotic symptoms, or a diagnosis of a psychotic disorder based on clinical assessment.
2. A history of any illness that, in the opinion of the Investigator, might confound the results of the trial or pose an additional risk to the patient by participation in the trial.
3. Clinically significant unstable or uncontrolled endocrine, gastrointestinal, cardiovascular, hematological, hepatic, immunological, renal, respiratory, or genitourinary abnormalities or diseases.
4. Uncontrolled, active seizure(s), within the 3 months prior to Screening.
5. Known human immunodeficiency virus (HIV), a detectable viral load for hepatitis C, or hepatitis B surface antigen indicative of chronic active infection.
6. Pregnant or is a nursing mother.
7. Suicidal ideation and behavior, based on Investigator assessment of the completed Columbia-Suicide Severity Rating Scale.
8. Currently taking medication(s) at a dose that has not been stable for ≥3 months prior to Day 1 or psychotherapy that has not been stable for ≥3 months prior to Day 1. If the patient is taking medication(s) or receiving psychotherapy, the patient and parent/guardian must agree to continue the intervention(s) at the same dose and frequency through the End of Trial Visit.

### Medication

Two (2) 100-mg (or placebo) capsules will be administered orally BID with liquids. If the patient is unable to swallow a capsule whole, capsules may be opened, and the contents sprinkled on applesauce; total daily dose: 400 mg.

### Crossover Design

All patients will have two 6-week treatment periods separated by a 1-week washout period. Each patient will be randomly assigned to one of two treatment sequences: NB-001 followed by placebo (sequence A/P) or placebo followed by NB-001 (sequence P/A).

### Remote/Virtual Trial

The trial is designed to allow most visits to be conducted via telephone and/or video (i.e., telemedicine) or by home health nurse. An in-person visit is required at Screening unless site or government mandates restrict this due to coronavirus (COVID-19). Other in-person visit(s) may occur, only if necessary, based on clinical judgment.

### Open Label Extension (OLE)

Upon completion of the crossover protocol, patients *may* have the opportunity for extended treatment with NB-001 in an open-label extension (OLE).