

## Role of Oxidative Stress and Inflammation in Type 1 Gaucher Disease (GD1): Potential Use of Antioxidant/Anti-inflammatory Medications

**Status:** Recruiting

### Eligibility Criteria

**Sex:** All

**Age:** 18 Years and over

This study is also accepting healthy volunteers

#### Inclusion Criteria:

1. All participants must be 18 years or older. 2. All participants must understand and cooperate with requirements of the study in the opinion of the investigators and must be able to provide written informed consent. 3. Individuals with GD1 who are medically stable for participation in the study in the opinion of the investigator. 4. GD1 patients must be on a stable, specific ERT and/or SRT therapy at a specific dose (e.g. on a units/kg basis) for at least 2 years. 5. GD1 patients who have had a change in therapy, i.e. a change in dose or switch from one drug to another, can be enrolled after at least 6 months have elapsed since the change and is considered stable in the opinion of the clinician providing care to the patient. 6. Healthy subjects who will be frequency-matched for age. 7. All participants must not have taken antioxidants coenzyme Q-10, vitamin C, or vitamin E for 3 weeks prior to the study and during the course of the study.

#### Exclusion Criteria:

1. Medically unstable conditions in any group as determined by the investigators. 2. Concurrent disease; medical condition; or an extenuating circumstance that, in the opinion of the investigator, might compromise subject safety, study compliance, completion of the study, or the integrity of the data collected for the study. 3. Women who are pregnant or lactating or of child-bearing age who are not using acceptable forms of contraception. 4. History of asthma that is presently being treated. 5. Patients enrolled in another interventional study. 6. Allergy to N-acetylcysteine. 7. Patients who cannot or are unwilling to have blood drawn. 8. Inability to undergo MRI scanning, including but not limited to: unable to remain still in an MRI scanner for more than 30 minutes, claustrophobia, presence of paramagnetic substances or pacemakers in body, weight over 300 lbs. 9. Unable to adhere to study protocol for whatever reason.

### Conditions & Interventions

#### Interventions:

Drug: N-acetylcysteine

#### Conditions:

Gaucher Disease Type 1

#### Keywords:

Gaucher Disease, GD1, N-acetylcysteine, glutathione, GSH

### More Information

**Description:** The purpose of this study is to measure levels of blood and brain chemicals related to oxidative stress and inflammation in healthy volunteers and individuals with Type 1 Gaucher disease (GD1) to see if these levels are altered by GD1. We will also examine if there is a change in these blood and brain chemicals after participants begin taking oral N-acetylcysteine (NAC), which is available both as a prescription medication and a natural product that has antioxidant and anti-inflammatory effects.

**Contact(s):** Reena Kartha - rvkartha@umn.edu

**Principal Investigator:** James Cloyd

**Phase:** Phase 2

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