The PRONTO Study, a Global Phase 2b Study of NEOD001 in Previously Treated Subjects With Light Chain (AL) Amyloidosis (PRONTO)

**Purpose**

This is a global, multicenter, Phase 2b, randomized, double-blind, placebo-controlled, two-arm, parallel-group efficacy and safety study of NEOD001 as a single agent administered intravenously in adults with AL amyloidosis who had a hematologic response to previous treatment for their amyloidosis (e.g., chemotherapy, autologous stem cell transplant [ASCT]) and have persistent cardiac dysfunction.

<table>
<thead>
<tr>
<th>Condition</th>
<th>Intervention</th>
<th>Phase</th>
</tr>
</thead>
<tbody>
<tr>
<td>AL Amyloidosis</td>
<td>Drug: NEOD001</td>
<td>Phase 2</td>
</tr>
<tr>
<td></td>
<td>Drug: Placebo</td>
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</tbody>
</table>

Study Type: Interventional

Study Design:
- Allocation: Randomized
- Intervention Model: Parallel Assignment
- Masking: Double Blind (Subject, Caregiver, Investigator, Outcomes Assessor)
- Primary Purpose: Treatment

Official Title: A Phase 2b, Randomized, Double-blind, Placebo-controlled Study of NEOD001 in Previously Treated Subjects With Light Chain (AL) Amyloidosis Who Have Persistent Cardiac Dysfunction

Resource links provided by NLM:
- MedlinePlus related topics: Amyloidosis
- Genetic and Rare Diseases Information Center resources: Multiple Myeloma, AL Amyloidosis
- U.S. FDA Resources

Further study details as provided by Prothena Therapeutics Ltd.:

Primary Outcome Measures:
- Cardiac best response as measured by NT-proBNP [Time Frame: Baseline to 12 months]

Secondary Outcome Measures:
- Change in Short Form-36 (SF-36) Questionnaire [Time Frame: Baseline to 12 months]
- Change in 6MWT [Time Frame: Baseline to 12 months]
- Renal best response as measured by proteinuria [Time Frame: Baseline to 12 Months]
- Change in Neuropathy Impairment Score- Lower Limb (NIS-LL) score [Time Frame: Baseline to 12 Months]
- Progression Free Survival [Time Frame: Baseline to 12 Months]
- To assess the safety of single agent NEOD001 by assessing vital signs, electrocardiogram (ECG), laboratory tests and AEs [Time Frame: Baseline to 12 months]

Estimated Enrollment: 100
Study Start Date: March 2016
Estimated Study Completion Date: January 2018
Estimated Primary Completion Date: January 2018 (Final data collection date for primary outcome measure)

<table>
<thead>
<tr>
<th>Arms</th>
<th>Assigned Interventions</th>
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<tbody>
<tr>
<td>Experimental:</td>
<td>NEOD001</td>
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<tr>
<td>Drug given IV every 28 days at 24mg/kg</td>
<td>NEOD001 is a monoclonal antibody directed at soluble and insoluble light chain aggregates</td>
</tr>
<tr>
<td>Placebo Comparator: Placebo</td>
<td>Drug: Placebo</td>
</tr>
<tr>
<td>Placebo</td>
<td>Saline Bag</td>
</tr>
</tbody>
</table>

**Eligibility**

Ages Eligible for Study: 18 Years and older (Adult, Senior)
Sexes Eligible for Study: All
Accepts Healthy Volunteers: No

**Inclusion Criteria:**
1. Age ≥18 years
2. Confirmed diagnosis of systemic AL amyloidosis
3. ≥1 prior systemic plasma cell dyscrasia therapy with at least a partial hematologic response
4. Cardiac involvement
5. NT-proBNP ≥650

**Exclusion Criteria:**
1. Non-AL amyloidosis
2. Meets the International Myeloma Working Group (IMWG) definition of Multiple Myeloma
3. NT-proBNP >5000
4. Received Plasma cell directed chemotherapy within 6 months
5. Received autologous stem cell transplant (ASCT) within 12 months

**Contacts and Locations**

Choosing to participate in a study is an important personal decision. Talk with your doctor and family members or friends about deciding to join a study. To learn more about this study, you or your doctor may contact the study research staff using the Contacts provided below. For general information, see Learn About Clinical Studies.

Please refer to this study by its ClinicalTrials.gov identifier: NCT02632786

**Contacts**

Contact: Wendy Currlin clinicaltrials@prothena.com
Contact: Tanya OShea, MSc clinicaltrials@prothena.com

**Sponsors and Collaborators**

Prothena Therapeutics Ltd.

**More Information**

Responsible Party: Prothena Therapeutics Ltd.
ClinicalTrials.gov Identifier: NCT02632786 History of Changes
Other Study ID Numbers: NEOD001-201
Study First Received: December 9, 2015
Last Updated: January 4, 2017

Keywords provided by Prothena Therapeutics Ltd.:
amyloidosis
ntprobnp
NEOD001
Prothena

Additional relevant MeSH terms:
neod001
amyloid
plasma cell dyscrasia
immunotherapy

Amyloidosis
Proteostasis Deficiencies
Metabolic Diseases

ClinicalTrials.gov processed this record on January 24, 2017