A Phase III Multicenter, Multinational, Randomized, Double-blind, Placebo-controlled Study to Evaluate the Efficacy and Safety of Patisiran (ALN-TTR02) in Transthyretin (TTR)-Mediated Polyneuropathy (Familial Amyloidotic Polyneuropathy-FAP)

The study is now open for enrollment in the United States. If you would like to be a participant in this study, please ask your physician to contact the Alnylam Clinical Trial Hotline (toll free in U.S. 866-330-0326). A summary of the study can be found below. For a more detailed description of the study and inclusion and exclusion criteria please visit www.clinicaltrials.gov (NCT01960348)

**Purpose of our Study**

The purpose of this study is to evaluate the safety and efficacy of patisiran in patients with Transthyretin (TTR)-Mediated Polyneuropathy (Familial Amyloidotic Polyneuropathy-FAP).

**Summary of Study Protocol**

**Primary Outcome:** The difference between the patisiran and placebo groups in the change from baseline of modified Neuropathy Impairment Score+7 (mNIS+7)

**Secondary Outcome:** The difference between patisiran and placebo in the change from baseline in (a) quality of life (b) motor function (c) autonomic function

<table>
<thead>
<tr>
<th>Condition:</th>
<th>Transthyretin (TTR)-Mediated Polyneuropathy (FAP)</th>
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</thead>
<tbody>
<tr>
<td>Intervention:</td>
<td>Patisiran or Placebo</td>
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<tr>
<td>Study Design:</td>
<td>Allocation: Randomized 2:1 Patisiran to Placebo</td>
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<td>Estimated Enrollment:</td>
<td>Approximately 200 patients</td>
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<td>Dosing Schedule:</td>
<td>Every 3 weeks for 18 months</td>
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**Key Inclusion Criteria**

- Male or female of 18 to 80 years of age (inclusive)
- Have a diagnosis of FAP with documented TTR mutation
- Have a NIS of 10 to 100 (inclusive)

**Key Exclusion Criteria**

- Has vitamin A levels below the lower limit of normal (LLN)
- Had a prior liver transplant or is planned to undergo liver transplant during the study period
- Has other known causes of sensorimotor or autonomic neuropathy (e.g., autoimmune disease, monoclonal gammopathy, etc.)