**Response of Patients With Fabry Disease With the Amenable GLA Mutation p.N215S to Treatment With Migalastat**


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**INTRODUCTION**

- Fabry disease is a devastating, rare, and progressive X-linked storage disorder caused by mutations in the GLA gene, resulting in the deficient activity of α-galactosidase A (α-Gal A) in lysosomes.
- Renal dysfunction progresses over time in a majority of male patients with Fabry disease and can lead to end-stage renal disease.
- Accumulation of α-Gal A substrate can lead to functional impairments in the kidneys.
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**Key Inclusion Criteria**

- Male and female patients aged 16-76 years diagnosed with Fabry disease with responsive GLA mutations based on a preliminary GLA-HEK293 cell assay
- Treatment initiation with ERT (≤3 months before baseline) and stable ERT dose (±10% allowed) for 3 months before baseline
- ERT naïve patients
- Patients taking angiotensin-converting enzyme inhibitors, angiotensin receptor blockers, or nitric oxide donors (or none) at baseline for 3 months before the screening visit

**METHODS**

**Study Design**

**ATTRACT (AT1001-012, NCT01218659)**: a phase 3, randomized, open-label study to assess the efficacy and safety of migalastat in patients with Fabry disease with amenable GLA mutations who were previously treated or not treated with ERT.

**Baseline Disease Severity and Characteristics**

- Age, plasma globotriaosylsphingosine (lyso-Gb₃) levels:
  - Patients with the p.N215S mutation had higher median lyso-Gb₃ levels before the screening visit

**RESULTS**

- The ITT amenable population consisted of 53/57 (34 migalastat; 19 ERT) patients
- There was a reduction in median GFR and stabilization of plasma lyso-Gb₃ levels from baseline to month 18

**CONCLUSIONS**

- Migalastat may offer promise as an oral treatment alternative for male and female patients with Fabry disease with amenable mutations.

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