INTRODUCTION

Fabry disease is a progressive, multisystem disorder caused by mutations in the GLA gene that result in deficient or absent lysosomal alpha-galactosidase A (α-Gal A) activity.

- In α-Gal A deficiency, results in accumulation of Gb3 in kidney cells triggers pathological injury.

- Continuous exposure to lysosomal enzymes can trigger multiple inflammatory pathways.

- The accumulation of Gb3 in kidney cells triggers pathological injury.

- Approved therapies for Fabry disease include enzyme replacement therapy (ERT) and migalastat – a small molecule chaperone to GLA.

- This study prospectively examined the multisystemic efficacy of migalastat in both ERT-experienced and ERT-naïve subjects over an 8.6-year period.

METHODS

- The post-hoc analysis integrated data from the double-blind, randomised, placebo-controlled FACTS trial (the randomised, open-label, active-controlled ATTRACT study and the open-label extension studies AT005 and AT006; Figure 1). Full inclusion and exclusion criteria for the studies and definitions of the outcomes are found in the statistical analysis can be found in Section S2 of the supplementary materials.

- The primary objective was the comparison between migalastat and ERT.

- Renal outcomes: doubling of serum creatinine levels from the start of the study.

- Cardiovascular events: myocardial infarction; new symptomatic arrhythmia, renal insufficiency and other.

- Overall events: any adverse event of any severity.

- RESULTS

- All patients had multiorgan involvement (81% of patients had multiorgan involvement compared with all others having ≥2 organs affected) at baseline.

- Among the 97 subjects enrolled in the ATTRACT trial and its open-label extension, 53 ERT-naïve patients (81%) met clinical criteria (based on LDSP) for reimbursement for migalastat in Australia.

- Eligibility for reimbursement in Australia was defined as: patients must demonstrate substantial Fabry-related organ damage to be eligible for reimbursement.

- Among the 97 subjects enrolled in the ATTRACT trial and its open-label extension, 53 ERT-naïve patients (81%) met clinical criteria (based on LDSP) for reimbursement for migalastat in Australia.

- CONCLUSIONS

- EGF R remained stable in ERT-naïve and ERT-experienced subjects with Fabry disease with migalastat compared favourably with historical reports of ERT.

- Overall, the patient population in the current analysis is representative of the Fabry disease patient population in Australia and the majority met the clinical criteria for migalastat reimbursement.

REFERENCES

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- All patients enrolled in the ATTRACT trial and its open-label extension, 53 ERT-naïve patients (81%) met clinical criteria (based on LDSP) for reimbursement for migalastat in Australia.

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