



Shaping Tomorrow's Nephrology: Insight-Driven Kidney Care

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FollowME Fabry Pathfinders Registry: Cardiac and Renal Effectiveness in a Multi-National, Multi-Center Cohort of Patients on Migalastat Treatment for 5 Years

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Objectives : The ongoing, real-world followME Fabry Pathfinders registry (EUPAS20599) is designed to evaluate natural history and treatment outcomes in Fabry disease (FD). Here, we present 5-year effectiveness and safety data for migalastat in patients with FD.

Methods : Data were collected and analyzed from patients ≥ 12 years of age with confirmed FD, migalastat-amenable GLA variants, an estimated glomerular filtration rate (eGFR) ≥ 30 mL/min/1.73m² and who had received migalastat for ≥ 4.9 years.

Results : As of August 2025, 156 patients (64.1% male) were included. Median migalastat exposure was 5.7 years (range: 4.9–7.5). At enrollment, median age was 56.0 years (range: 16–77); p.N215S was the most common GLA variant (n=41; 26.3%). The Chronic Kidney Disease Epidemiology Collaboration equation was used to calculate eGFR. At baseline, 56 (35.9%) of patients had eGFR (mL/min/1.73m²) ≥ 90 , 54 (34.6%) ≥ 60 – <90 , 10 (6.4%) ≥ 30 – <60 and 36 (23.1%) had missing values; 39 had a proteinuria category ≥ 1 and 27 had missing values. Female baseline median eGFR was 90.8 mL/min/1.73m² (n=43; range: 57.7–128.1) with median annualized rate of change (ARC) -1.40 mL/min/1.73m²/year (n=55; 95% confidence interval [95%CI] -2.04 , -0.72); baseline median left ventricular mass index (LVMI) was 85.5 g/m² (n=35; range: 47.0–232.5) and median LVMI ARC was -0.69 g/m²/year (n=46; 95%CI -2.36 , 1.68). Male baseline median eGFR was 85.8 mL/min/1.73m² (n=77; range: 37.8–133.1) with median ARC -1.51 mL/min/1.73m²/year (n=99; 95%CI -2.06 , -0.68); baseline median LVMI was 132.3 g/m² (n=55; range: 54.3–296.2) and median LVMI ARC was -1.53 g/m²/year (n=67; 95%CI -4.72 , 0.40). Combined, median eGFR ARC was -1.47 mL/min/1.73m²/year (n=154; 95%CI -1.99 , -0.98 ; Figure 1) and median LVMI ARC was -0.95 g/m²/year (n=113; 95%CI -2.36 , 0.23; Figure 2). Two patients (1.3%) had any treatment-related treatment-emergent serious adverse event; neither discontinued migalastat.



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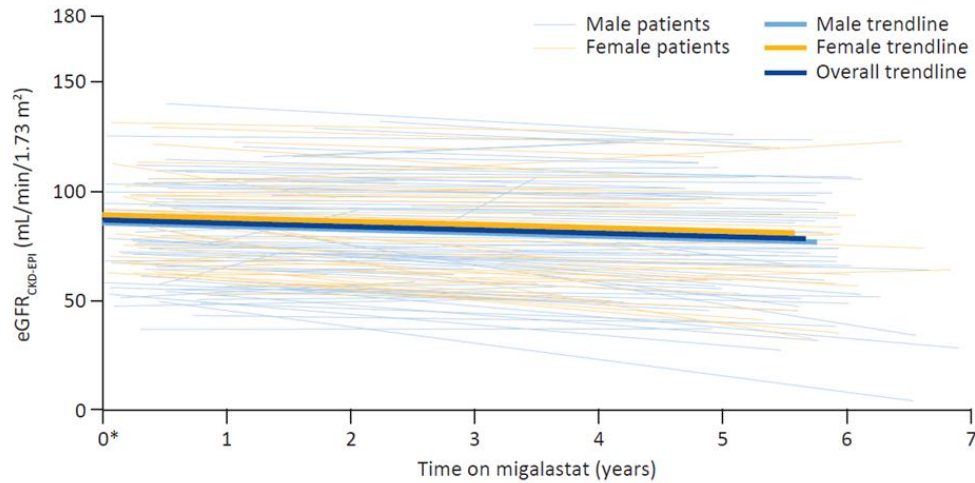


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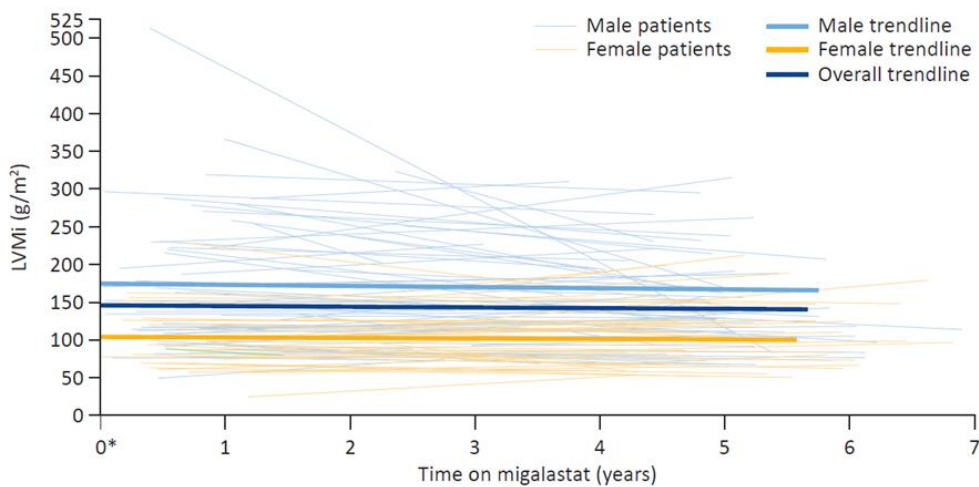
Conclusions : In this real-world observational study, migalastat preserved cardiac size and renal function over median 5.7 years with no new safety findings reported.

Figure 1.png



*Time 0 represents start of migalastat treatment. The ARC line of one patient was not shown due to a data collection anomaly.

Figure 1.png



*Time 0 represents start of migalastat treatment.