



Shaping Tomorrow's Nephrology: Insight-Driven Kidney Care

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Real-World Effectiveness of Migalastat Versus Enzyme Replacement Therapy in Previously Treatment-Naïve Patients With Fabry Disease: Analyses of Matched Populations From the Global followME Fabry Pathfinders Registry

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Objectives : The ongoing followME Fabry Pathfinders registry (EUPAS20599) evaluates outcomes in patients with Fabry disease. We compared effectiveness of the oral small-molecule chaperone migalastat with enzyme replacement therapy (ERT) in previously treatment-naïve patients aged ≥ 16 years at registry enrollment.

Methods : The predefined focus was incidence of Fabry-associated clinical events (FACEs); analyses also included FACE rates (overall and cardiac/renal/cerebrovascular) and annualized rate of change in estimated glomerular filtration rate calculated using the Chronic Kidney Disease Epidemiology Collaboration equation ($eGFR_{CKD-EPI}$ slope). Patients initiated migalastat or ERT at "index date"; migalastat-treated patients had amenable GLA variants. Patients included in $eGFR_{CKD-EPI}$ slope analyses also had ≥ 3 $eGFR$ assessments. Propensity score matching (1:1; nearest neighbor with replacement) used age, sex and $eGFR_{CKD-EPI}$ at index date; matching was evaluated using standardized mean differences (SMDs). Poisson regression and mixed models with repeated measures compared FACEs and $eGFR_{CKD-EPI}$ slope, respectively.

Results : Overall, 290/319 migalastat-treated patients were matched with 106/140 ERT-treated; mean post-matching exposures were 3.68 years (standard deviation 1.70) and 3.49 (1.54), respectively. Corresponding values for $eGFR_{CKD-EPI}$ slope analyses were 222/268, 80/119, 3.98 (1.52) and 3.96 (1.52) years. Matched variables were well-balanced (SMDs <0.1). Overall, patients experienced 55.3 FACEs/1000 patient-years (95% confidence interval [95%CI] 40.7, 75.3) with migalastat and 100.7 (53.7, 188.9) with ERT; event-rate ratio was 0.55 (95%CI 0.27, 1.11; $P=0.093$; Figure). Cardiac/renal FACEs had similar trends. Event-rate ratio for cerebrovascular FACEs was 0.24 (95%CI 0.06, 0.88; $P=0.031$), representing 4.7 FACEs/1000 patient-years (2.0, 11.2) with migalastat and 19.8 (7.5, 52.4) with ERT. $eGFR_{CKD-EPI}$ slope was -1.47 mL/min/ 1.73 m²/year (95%CI -2.17 , -0.77) with migalastat and -2.50 (-3.53 , -1.46) with ERT; difference $\Delta 1.03$ (95%CI -0.23 , 2.28; $P=0.109$).

Conclusions : All comparisons numerically favored migalastat over ERT, with the comparison for cerebrovascular FACEs being statistically significant. These analyses provide evidence supporting beneficial treatment effects of chaperone therapy on FACEs and rate of $eGFR$ decline in Fabry disease.



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