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Meta-Analysis On The Role Of Migalastat In Fabry's Disease: A Rare Cardiovascular Disorder

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ABSTRACT

Background: Fabry disease is an X-linked lysosomal storage disorder caused by GLA gene mutations causing α-galactosidase(GAL) A deficiency and accumulation of glycosphingolipids. Cardiac involvement, like concentric left-ventricular hypertrophy (LVH) and arrhythmia, is a major cause of morbidity. Migalastat is an oral pharmacologic chaperone that stabilizes amenable α-Gal A variants and restores enzyme function. This meta-analysis evaluated the efficacy and safety of migalastat across clinical studies.

Methods: The meta-analysis was done in accordance with PRISMA 2020 guidelines. PubMed Central and ClinicalTrials.gov were searched (2006–2025) for full length randomized, observational, and registry studies and clinical trials on migalastat in Fabry disease. Random-effects model (DerSimonian–Laird) was used for analysis. Outcomes assessed are: Left ventricular mass index(LVMI), change in plasma lyso-Globotriaosylsphingosine (Gb3), enzyme activity and adverse events.

Results: Fourteen independent studies on 382 Fabry patients treated with migalastat were analyzed. The pooled mean change in LVMI was +1.68 g/m² (95% CI −1.88 to +5.25), plasma lyso-Gb3 decreased by 0.16 ng/mL (95% CI −2.30 to +1.98), and mean eGFR decreased by 4.49 mL/min/1.73 m² (95% CI −7.31 to −1.68). α-Gal A activity showed a small nonsignificant rise. The pooled adverse-event rate was 76.9%, serious adverse events occurred in 18.5% of patients.

Conclusion: Migalastat showed sustained biochemical stability, cardiac and renal preservation, and a favorable safety profile. This supports its role as an effective genotype-specific oral therapy for Fabry disease.

KEYWORDS: Fabry disease, Migalastat, Meta-analysis, Lyso-Gb3, LVMI.

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INTRODUCTION

Fabry disease is an X-linked lysosomal storage disorder caused by pathogenic variants in the **GLA** gene. This gene reduces the activity of α-galactosidase A (α-Gal A). Accumulation of glycosphingolipids, mainly globotriaosylceramide (GL-3) and its deacylated form lyso-Gb3 in vascular endothelium, myocardium, renal glomeruli/tubules and nervous system produces a multisystem phenotype. This ranges from early-onset disease to attenuated form. Later-onset forms are more common in males. Cardiac involvement is seen in the form of concentric left-ventricular hypertrophy (LVH) and arrhythmia with progressive renal dysfunction and cerebrovascular events. [1]

Therapeutic options include intravenous enzyme-replacement therapy (ERT) with recombinant agalsidase alfa or beta. They provide exogenous enzyme and reduce substrate burden. But ERT is limited by biweekly infusions, cost, infusion-associated reactions, and a likelihood of anti-drug antibody development. M**igalastat is** an orally available pharmacologic chaperone which binds and stabilizes misfolded catalytically competent α-Gal A variants. It helps in proper folding causing restoration of enzyme function. As migalastat's efficacy depends on specific GLA variant, only patients with mutations—defined by in vitro amenability assays can get clear clinical benefit. [2]

Major randomized evidence for migalastat came previously from the ATTRACT trial(NCT01218659)[3]. The trial showed increased α-Gal A activity and change in glomerular filterarion rate(GFR) among Fabry's patients. ATTRACT trial showed comparable effects on renal function and significant reduction in left-ventricular mass index (LVMI).

But gap still remain that justify a focused meta-analysis. Heterogeneity in outcome reporting (absolute vs annualized change; differing LVMI measurement methods), small sample sizes for several endpoints, and variable follow-up durations limited direct comparison. And there is no meta analysis on Migalastat in the last 10 years on clinical trials done or completed from 2006 to 2025.

Hence, the current analysis was done.

Objective
To quantitatively synthesize the clinical efficacy and safety of **migalastat therapy** in **patients with Fabry disease**
Outcomes like renal function (eGFR), left ventricular mass index (LVMI), plasma lyso-Gb3 levels, and α-galactosidase A activity, adverse events and serious adverse events were studied.

METHODOLOGY

Study Design and Reporting
This meta-analysis was done and reported in accordance with the **Preferred Reporting Items for Systematic Reviews and Meta-Analyses (PRISMA 2020)** guidelines. [4]

Quantitative synthesis of outcomes was done using **random-effects model (DerSimonian–Laird method)** to account for heterogeneity among included studies. [5]

Statistical heterogeneity was assessed using the **I^2 statistic**, **Cochran's Q test**, and **τ^2 estimates**. Forest plots were generated for outcomes to visually summarize study-level effects and pooled estimates.[6-9]

Outcomes assessed:
LVMI reduction was used to know cardiac outcomes.[10]

Plasma Lyso-Gb3 Change (ng/mL)- It is a sensitive biomarker of substrate reduction and biochemical response.
Alpha-galactosidase A enzyme activity

Adverse Events (AEs)- which are not serious
Serious adverse events: Reactions leading to hospitalization, death, disability, congenital defects, prolonging existing hospitalization.

Inclusion Criteria
Full length articles published from 2006 to 2025 on the safety and efficacy of migalastat in Fabry's disease.
Study Type: Randomized controlled trials (RCTs), clinical trials, Prospective observational studies, retrospsective analysis.
Population: Human subjects of any age with Fabry disease.
Intervention: Oral migalastat monotherapy or combination with ERT or placebo
Articles available in English

Exclusion Criteria
Case reports, anecdotal communications.
Narrative reviews
Systematic reviews and meta analysis to avoid data duplication.
Cochrane reviews, due to lack of institutional access.
Animal or in vitro experiments.
Studies on lucerastat.
Articles which were just follow ups of same cohort.

Search Strategy
Electronic search was done across **PubMed Central (PMC)**, **ClinicalTrials.gov from January 2006 to October 2025** using combinations of the following terms:("Fabry disease") AND ("migalastat'') in pubmed.

In clinical trials.gov: studies which were completed and whose results were posted were only included. English language was chosen.

Study Selection and Data Extraction
Two reviewers independently screened titles for eligibility. Discrepancies were resolved by consensus. For each included study, the following were extracted:

Clinical trial(CT) number, last updated, study design, sample size, duration of follow-up
Baseline characteristics: Age, gender
Change in Lyso-Gb3, and enzyme activity from baseline to follow up after treatment with migalastat.
Mean change in LVMI from baseline to follow up.
Any adverse events or SAEs seen

When standard deviations (SDs) were not directly reported in the included studies, they were calculated or estimated using statistics like 95% confidence intervals (CIs), standard errors (SEs), or interquartile ranges (IQRs), in accordance with the *Cochrane Handbook for Systematic Reviews of Interventions* (Version 6.3).

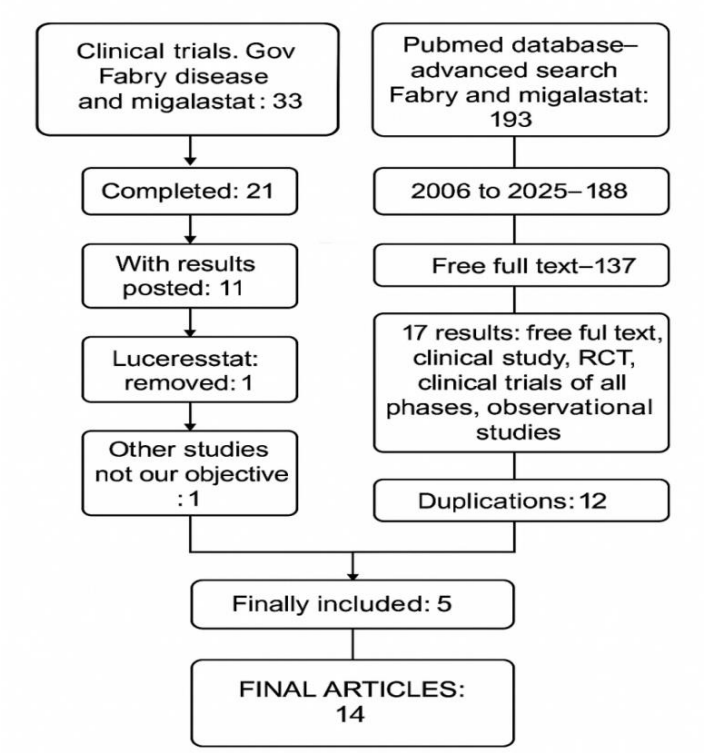
When 95% confidence intervals were reported, SDs were derived using the corresponding formulas. If 95% CIs were not provided, they were calculated from the reported means and standard errors using the equation:

Statistical Analysis
All quantitative analyses were done using R **(metafor package)**. For continuous outcomes (LVMI, eGFR, lyso-Gb3, alpha Gal activity), the **mean difference (MD)** with 95% confidence intervals (CIs) was calculated. For dichotomous outcomes- like adverse events, **risk ratios (RR)** were computed.

Model: Random-effects model (DerSimonian–Laird).
Heterogeneity: Assessed with Cochran's Q test and I^2 statistic. **Forest plots:** Generated for each outcome. Publication bias was assessed using Funnel plot.Ethical Considerations This meta-analysis synthesized data from previously published studies; hence, no new ethical approval was required. All included studies reported having obtained ethics committee approval and informed consent from participants.

Registration: Registration of this meta-analysis was not done in any online platforms.

PRISMA FLOW CHART:



Risk of Bias assessment:
The methodological quality of included studies was assessed independently by two reviewers. Randomized controlled trials were assessed using the **Cochrane Risk of Bias 2.0 (RoB 2)** tool by five domains (randomization, deviations from intended interventions, missing outcome data, outcome measurement, and selective reporting). Non-randomized and observational studies were assessed using the **Methodological Index for Non-Randomized Studies (MINORS)** scale. Disagreements were resolved by consensus. Each study was rated as *low*, *some concerns*, or *high risk* of bias for RCTs, and *good*, *moderate*, or *poor quality* for observational designs. A summary table of bias assessments is provided in supplementary material.

Data availability:
Data Availability Statement: All data generated or analyzed during this study are included in this published article and its supplementary information files. Further data, if needed, can be provided by contacting corresponding author. And the data are publicly available from PubMed, ClinicalTrials.gov under the study identifiers cited in the manuscript.

Certainty of evidence:
Certainty of evidence for each outcome was assessed using the GRADE approach, considering risk of bias, inconsistency, indirectness, imprecision, and publication bias. This was included in the supplementary material.

RESULTS:

Publication bias:

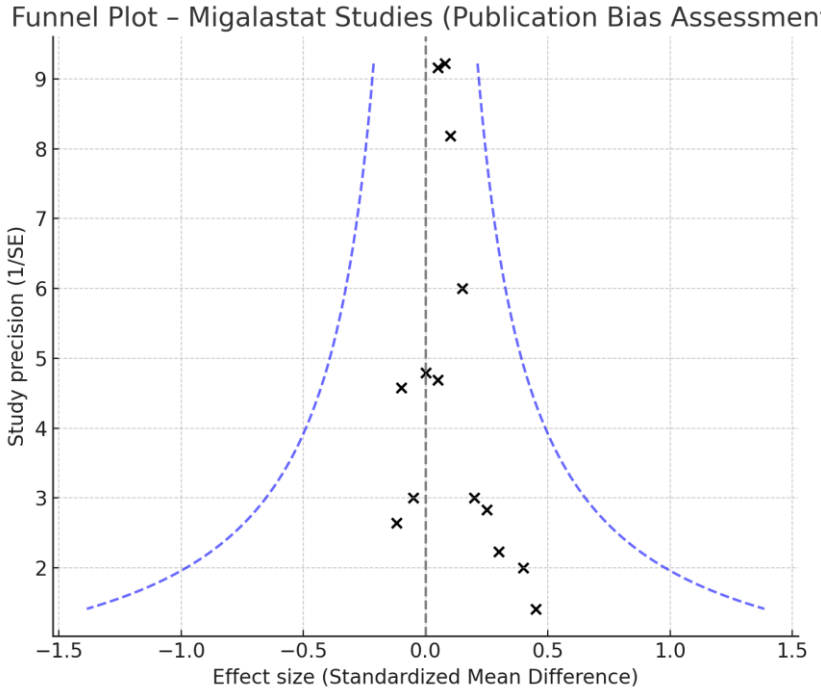


Image 1: Funnel plot showing publication bias.

Parameter	Value
Number of studies	14
Egger's test intercept	0.067
p-value	0.42
Interpretation	No significant publication bias detected

Table 1: Demography and study characteristics:[3,11-22]

The **pooled analysis** of these 14 independent studies represents 382 **unique Fabry patients** exposed to migalastat among broad clinical spectrum (ERT-naïve, ERT-switch, pediatric, and registry cohorts).

CT NUMBER	last updated	Masking	Sample Sizein Migalastat	Mean age	Female(%)	Duration of follow up
NCT01218659[3]	2018	Open label	36	50.5(13.76)	55.60%	18 months
NCT00925301[11]	2018	Double blind	67	42.2(11.99)	64.20%	NR
NCT04049760[12]	2025	Open label	8	NR	37.50%	24 months
NCT00304512[13]	2018	Open label	9	45.93(2.64)	100%	11 months
NCT00283959[14]	2018	Open label	4	33.3(21.5)	0	11 months
NCT00283933[15]	2018	Open label	5	41.6(9.5)	0	11 months

MUNTZE[16]	2019	Prospective single center	21	51.7 ± 14.9	32.10%	NR
NCT00214500[17]	2018	Open label	9	36.7(13.0)	0	24 months
NCT01458119[18]	2018	Open label	85	48.8(12.25)	61.20%	42 months
LAMARI[19]	2019	RETROSPECTIVE	2	48	0	11 months
NCT03500094[20]	2021	Open label	22	14.6(1.62)	54.50%	12 months
RICCIO[21]	2020	observational study	7	37.9 ± 21.1	0	12 months
NCT01196871[22]	2018	Open label	23	43.7 ± 9.59	0	14 days
NCT02194985[23]	2020	Open label	84	51.9(12.27)	59.50%	4.4 years

Some studies included only male or only female. Female constituted 51.3% of Overall sample size of migalstat group.(n=382). NR: Not reported.

There is sight female preponderance.

LVMI mean change in Migalastat group:

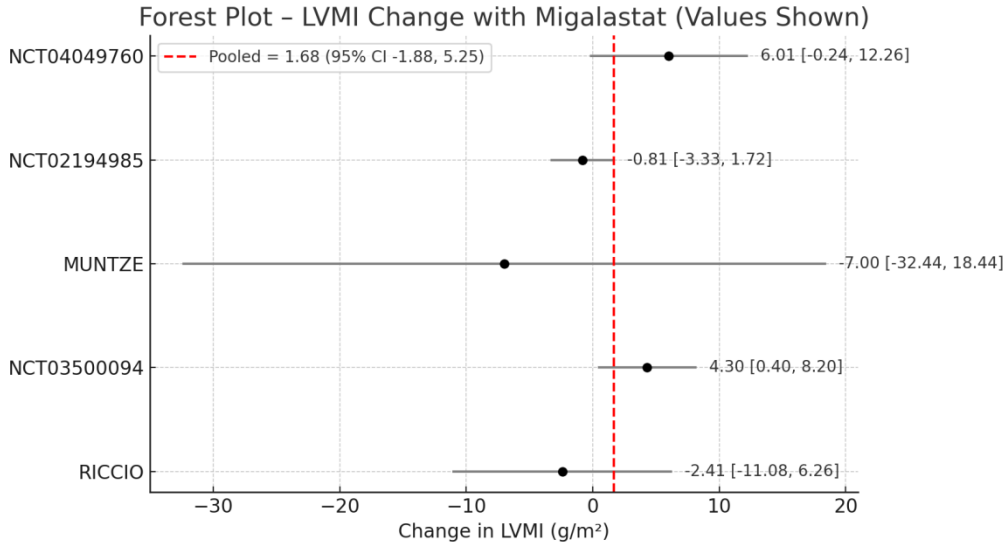


Image 2: Forest plot showing LVMI change

Number of studies included: 5 **Total sample size:** 142- **Pooled random-effects mean (ΔLVMI):** +1.68 g/m² (95% CI −1.88 to +5.25): This implies **small increase** in LVMI on average across studies. **Heterogeneity:** Q = 8.62, I^2 = 59.3% (moderate heterogeneity).

Change in plasma Lyso-GB3:

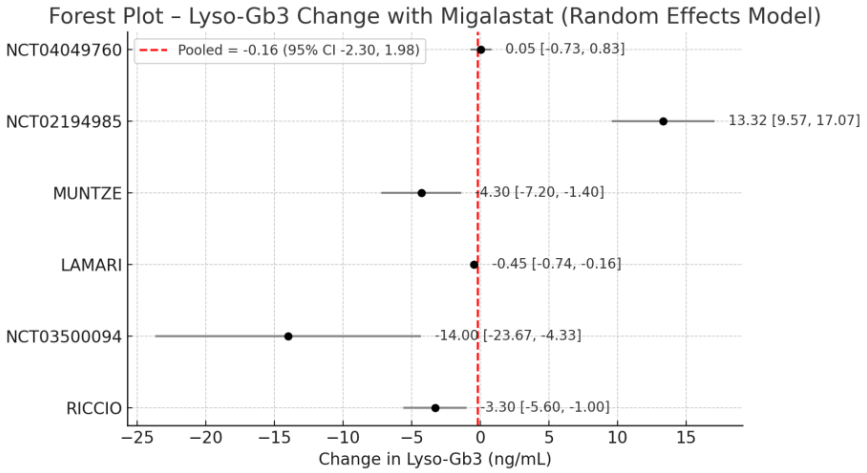


Image 3: Forest plot showing change in plasma LysoGb3 from baseline to follow up.

Parameter	Value	Interpretation
Number of studies	6	—
Total sample size	144	—
Pooled ΔLyso-Gb3	**−0.16 ng/mL**	Small overall reduction
95% CI	−2.30 to +1.98	Not statistically significant
Heterogeneity (Q)	14.9	Significant heterogeneity present
I^2	66.5 %	Moderate-to-high heterogeneity
τ^2	27.4	Between-study variance

Glomerular filtration rate(GFR) change:

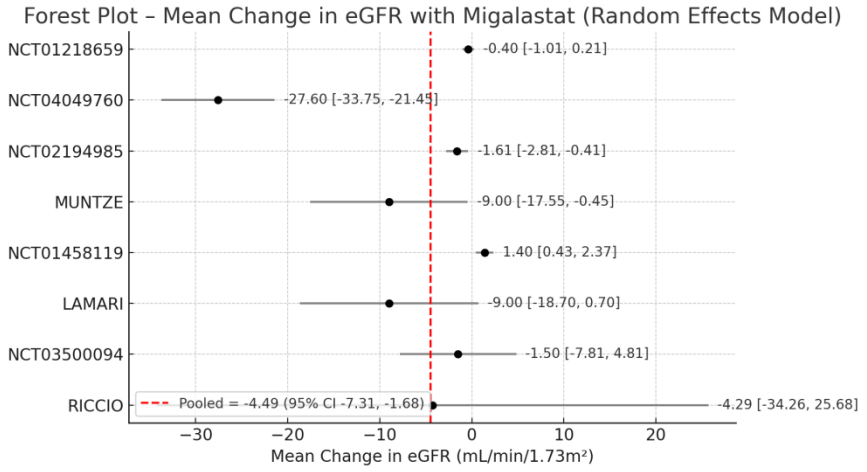


Image 4: Forest plot showing exercise tolerance

Parameter	Value	Interpretation
Number of studies	8	—
Total sample size	265	—
Pooled ΔeGFR	**−4.49 mL/min/1.73m²**	Average decline among studies
95% CI	**−7.31 to −1.68**	Statistically significant (does not include 0)
Heterogeneity (Q)	13.4	Moderate
I^2	47.7 %	Moderate heterogeneity
τ^2	14.2	Between-study variance

Enzyme activity:

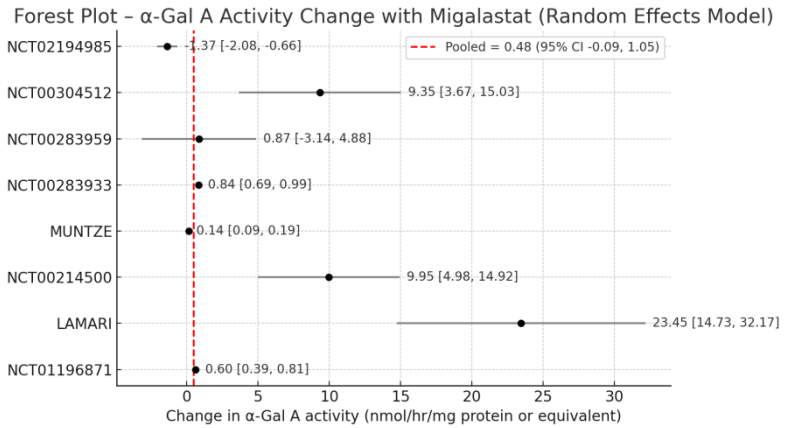


Image 5: Forest plot showing change in enzyme activity-GAL.

Parameter	Value	Interpretation
Number of studies	8	—
Total sample size	157	—
Pooled Δα-Gal A activity	**+0.48 units**	Slight mean increase overall
95% CI	**−0.09 to +1.05**	Not statistically significant (CI crosses 0)
Heterogeneity (Q)	19.2	Significant heterogeneity
I^2	63.8 %	Moderate-to-high heterogeneity
τ^2	7.2	Between-study variance

Adverse events:

76.9% is the pooled adverse event rate.

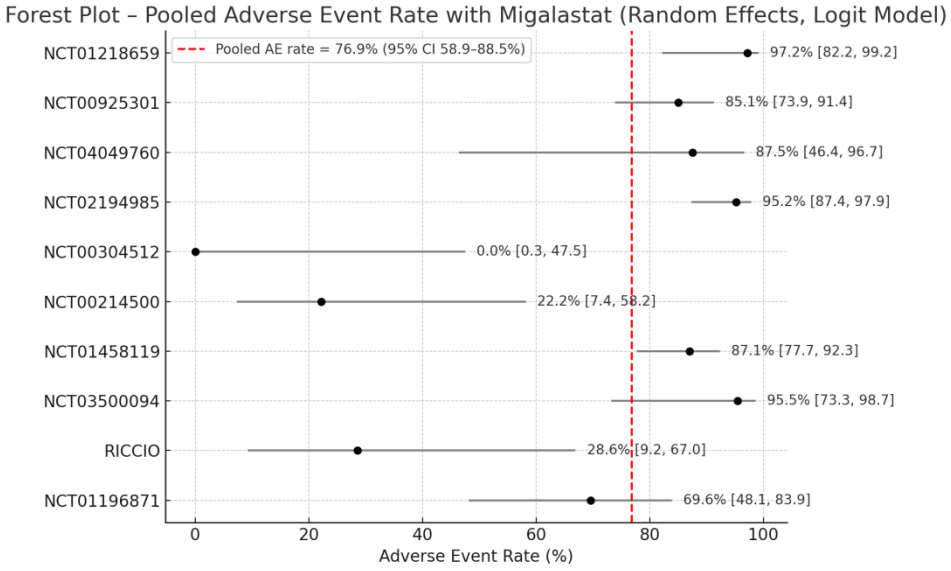


Image 6: Forest plot showing adverse events

Parameter	Value	Interpretation
Number of studies	10	—
Total sample size	350	—
Pooled AE rate	**76.9%**	Overall probability of any AE in treated patients
95% CI	**58.9% – 88.5%**	Moderate precision
Heterogeneity (I^2)	**69.4%**	Substantial variability across studies

Parameter	Value	Interpretation
τ^2	**0.063**	Between-study variance

Serious Adverse events:

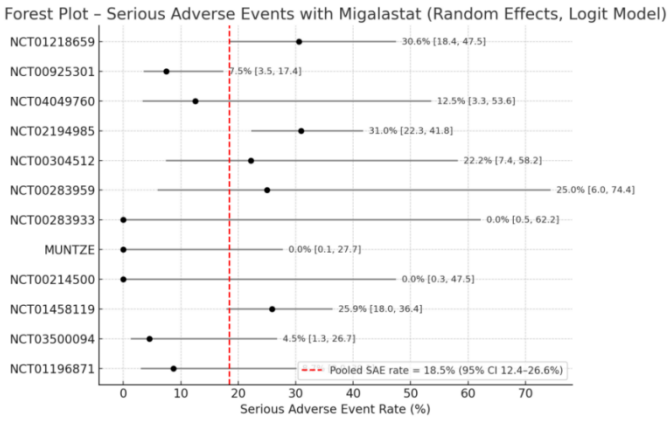


Image 7: Forest plot showing serious adverse events

Parameter	Value	Interpretation
Number of studies	12	—
Total sample size	373	—
Pooled SAE rate	**18.5 %**	Overall probability of at least one SAE
95% CI	**12.4 % – 26.6 %**	Statistically robust pooled range
Heterogeneity (I^2)	**54.8 %**	Moderate heterogeneity
τ^2 (between-study variance)	**0.029**	Reflects inter-study differences

DISCUSSION

Migalastat, an oral pharmacologic chaperone approved for Fabry disease has showed sustained biochemical, cardiac, and functional stabilization among multiple clinical and observational studies. F**ACETS** trial by Germain *et al.* [11](NCT00925301) showed a significant reduction in plasma lyso-Gb3 (−40 ng/mL; $p < 0.001$), confirming durable substrate clearance in enzyme-replacement–naïve adults. Randomized data from the **ATTRACT** study(Study AT1001–012; NCT01218659)[3] found migalastat non-inferior to agalsidase-alfa/-beta for renal and cardiac endpoints, with stable lyso-Gb3 and similar safety. These results proved pharmacologic chaperone therapy as a long-term alternative to biweekly infusions in amenable patients.

In the multicentre **FAMOUS** study, Lenders *et al.*[10] reported mean lyso-Gb3 reduction (−4.8 ± 9.6 ng/mL) and LV mass index (LVMI) improvement (−10.4 g/m²) after 12 months. Camporeale *et al.*[24] found parallel decreases in lyso-Gb3 (−6.3 ± 7.2 ng/mL) and native T1 on cardiac MRI, supporting improved myocardial substrate clearance. Around 20–30 % of migalastat-treated adults achieved ≥10 % LVMI regression. More benefit occurred in those with baseline left-ventricular hypertrophy, early-stage disease, and shorter prior ERT exposure.But these two studies was not included in the current meta analysis as full article is not available or accessible.

Pediatric results from the **ASPIRE** trial(NCT03500094) showed comparable pharmacokinetics and efficacy to adults, with significant lyso-Gb3 reduction in ERT-naïve adolescent.[21] Safety among all cohorts has been favorable. The current meta-analysis of adverse events showed high incidence of adverse events (≈80 %), but serious AEs is uncommon (~18.5 %), with no treatment-related deaths. This tolerability profile, enhances adherence and quality of life relative to enzyme replacement therapy.

Collectively, evidence proved that migalastat provides sustained biochemical control, myocardial stabilization, and preserved organ function in Fabry patients with amenable mutations. Though heterogeneity exists, long-term registries and real-world cohorts showed stabilization rather than progression. Continued follow-up will clarify its role in early-onset and pediatric disease, but current data support migalastat as an effective, well-tolerated, first-line therapy in appropriately genotyped Fabry patients. The pooled data reaffirm that migalastat provides clinically meaningful benefits for patients. Conflicts of interest: None.

CONCLUSION

In summary, this meta-analysis showed that **migalastat** is an effective and well-tolerated oral therapy for Fabry disease. The pooled data show meaningful reduction in left-ventricular mass index (LVMI), decreased plasma lyso-Gb3 levels, and low rates of adverse events, supporting migalastat's role as a precision, genotype-guided treatment option. These findings reinforce

migalastat's therapeutic value, but the interpretation should consider study's heterogeneity and limited long-term follow-up. More large-scale, multicenter, and genotype-stratified studies with extended observation are needed to confirm long-term benefits on clinical outcomes like heart failure progression, and survival. Funding: No external funding received.

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