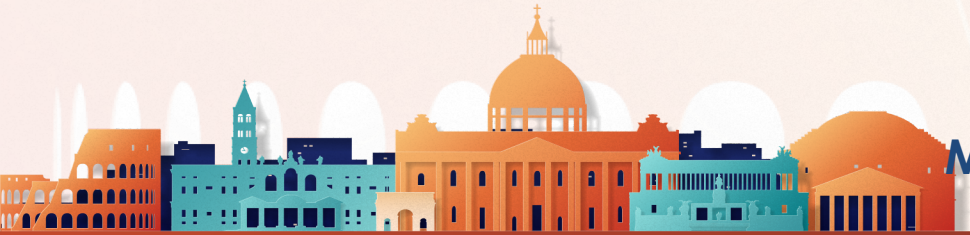
PLACE 2026
PLATFORM OF LABORATORIES FOR ADVANCES IN CARDIAC EXPERIENCE

CONGRESSO
ROMA, 25 - 27 Giugno 2026

CARDIOMIOPATIE RARE, CARDIOPATIE NEUROMUSCOLARI E MALATTIA DI ANDERSON-FABRY

La terapia nella malattia di AndersonFabry: dagli studi, ai registri, alla real-life

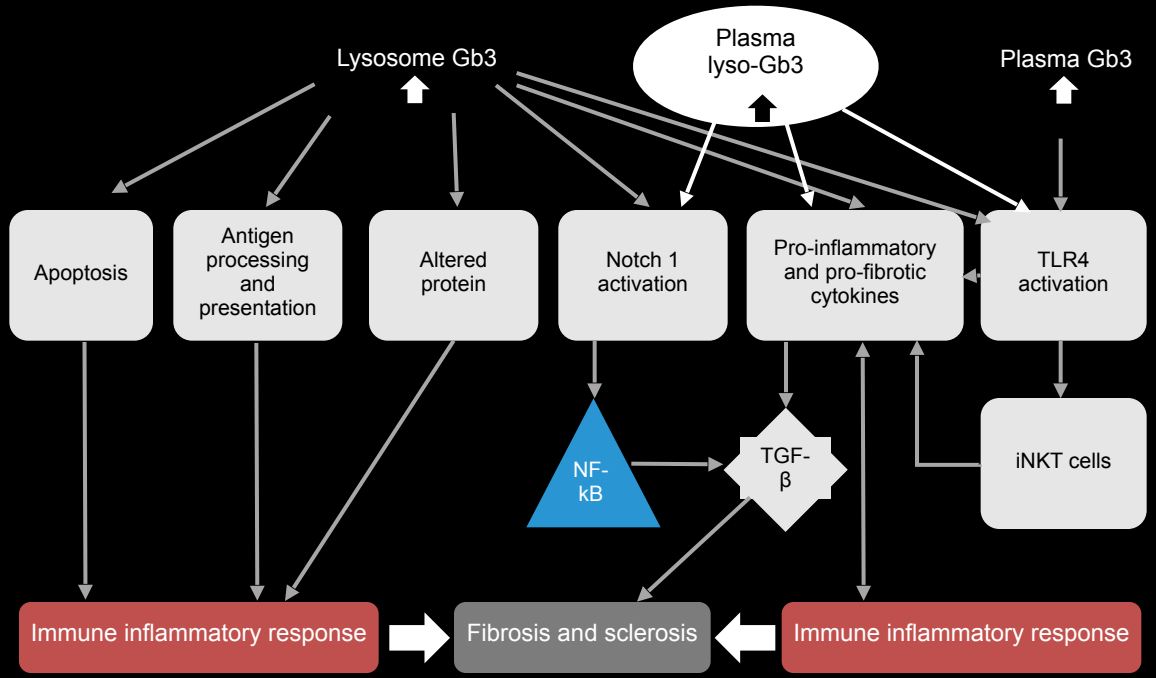


Sandro Feriozzi
Nefrologia
Università Campus Bio-Medico
Roma

DISCLOSURES

S. Feriozzi has received travel assistance and honoraria for lecturing and for participating in advisory boards from Sanofi/Takeda/Amicus/Medtronic/ Chiesi

PATHOGENETIC HYPOTHESIS OF TISSUE DAMAGE IN FABRY DISEASE



Adapted from Rozenfeld P, Feriozzi S. 2017

Gb3, globotriaosylceramide; iNKT, invariant natural killer T cell; lyso-Gb3, globotriaosylsphingosine; NF-κB, nuclear factor kappa B; TGF-β, transforming growth factor-beta; TLR4, toll-like receptor 4.
Rozenfeld P, Feriozzi S. *Mol Genet Metab*. 2017;122(3):19–27.

Rozenfeld & Feriozzi Mol Genet Metab 2017

Therapeutics

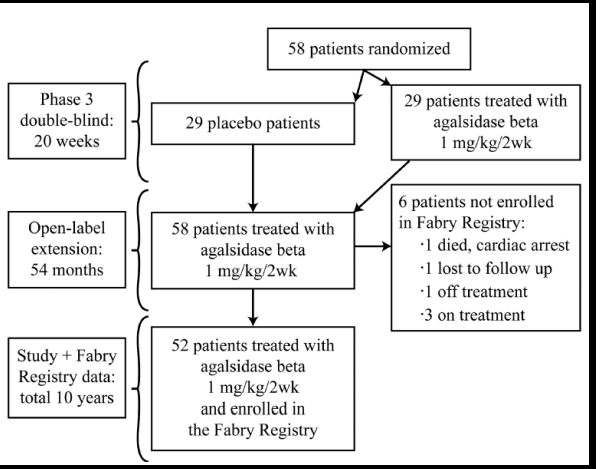
ORIGINAL ARTICLE

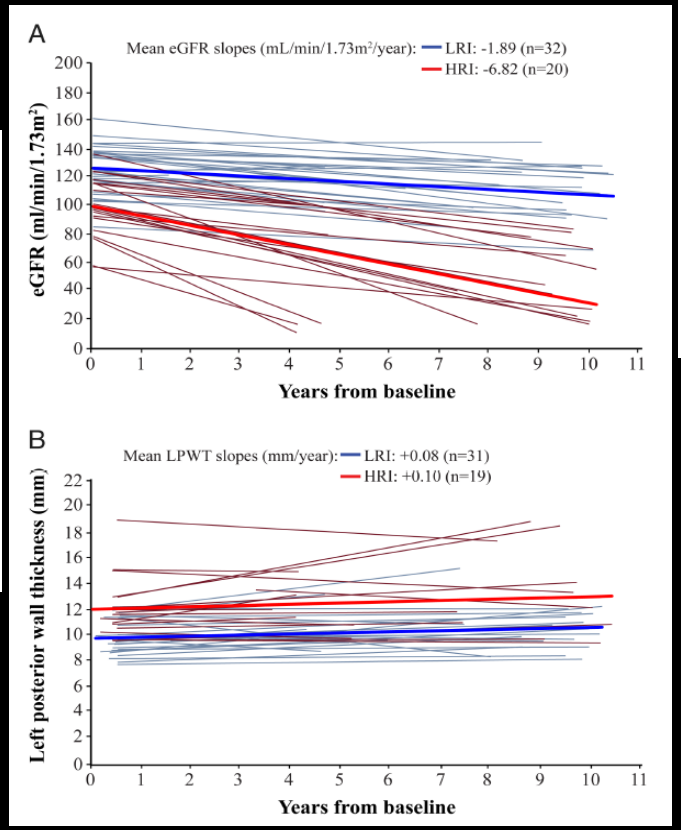
OPEN ACCESS

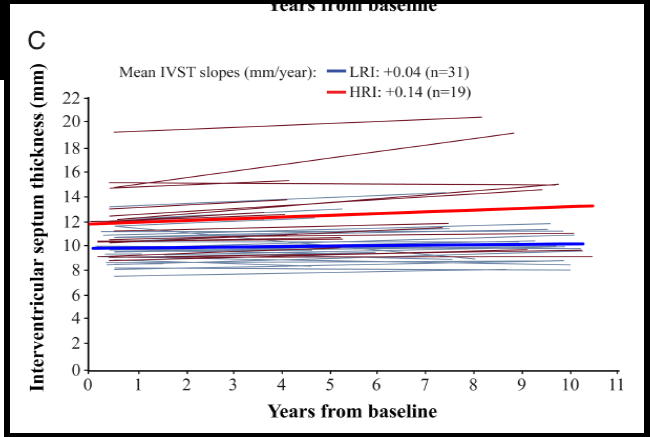
2015;52:353–358. doi:10.1136/jmedgenet-2014-102797

Ten-year outcome of enzy… with agalsidase beta in patients with Fabry disease

Dominique P Germain,[1] Joel Charrow,[2] Robert J Desnick,[3] Nathalie Guffon,[4] Judy Kempf,[5] Robin H Lachmann,[6] Roberta Lemay,[5] Gabor E Linthorst,[7] Seymour Packman,[8] C Ronald Scott,[9] Stephen Waldek,[10] David G Warnock,[11] Neal J Weinreb,[12] William R Wilcox[13]







Conclusions This 10-year study documents the effectiveness of agalsidase beta (1 mg/kg/2 weeks) in patients with Fabry disease. Most patients remained alive and event-free. Patients who initiated treatment at a younger age and with less kidney involvement benefited the most from therapy. Patients who initiated treatment at older ages and/or had advanced renal disease experienced disease progression.

Molecular Genetics and Metabolism Reports 3 (2015) 21–27

Contents lists available at

Molecular Genetics and M

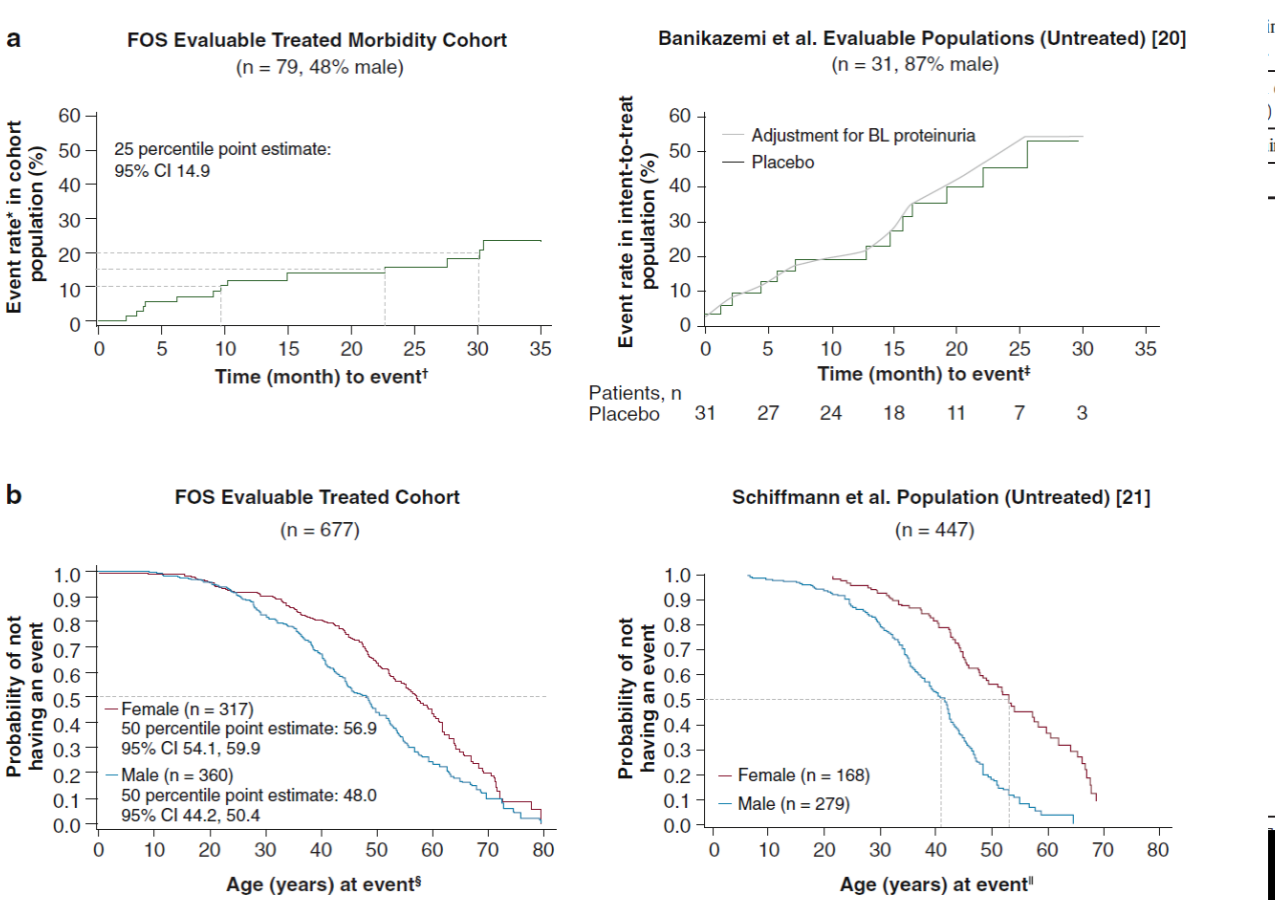
journal homepage: http://www.journals.els
metabolism-re

Long-term effectiveness of agalsidase alfa enzy disease: A Fabry Outcome Survey analysis

Michael Beck [a,*], Derralynn Hughes [b], Christoph Kampmann Guillem Pintos-Morell [d], Uma Ramaswami [b], Michael West the Fabry Outcome Survey Study Group

Table 5
Progression of cardiomyopathy, determined a and female patients stratified by the presenc

Baseline LVH status	n	FOS Evaluable Trea Cardiac cohort Mean annualized
Males		
Total	71	0.33 (0.1 [0.13, 0.5
LVH	29	0.19 (0.1 [−0.13, (
No LVH	42	0.47 (0.1 [0.22, 0.7
Females		
Total	93	0.48 (0.0 [0.30, 0.6
LVH	45	0.77 (0.1 [0.49, 1.0
No LVH	48	0.19 (0.1 [−0.03, (



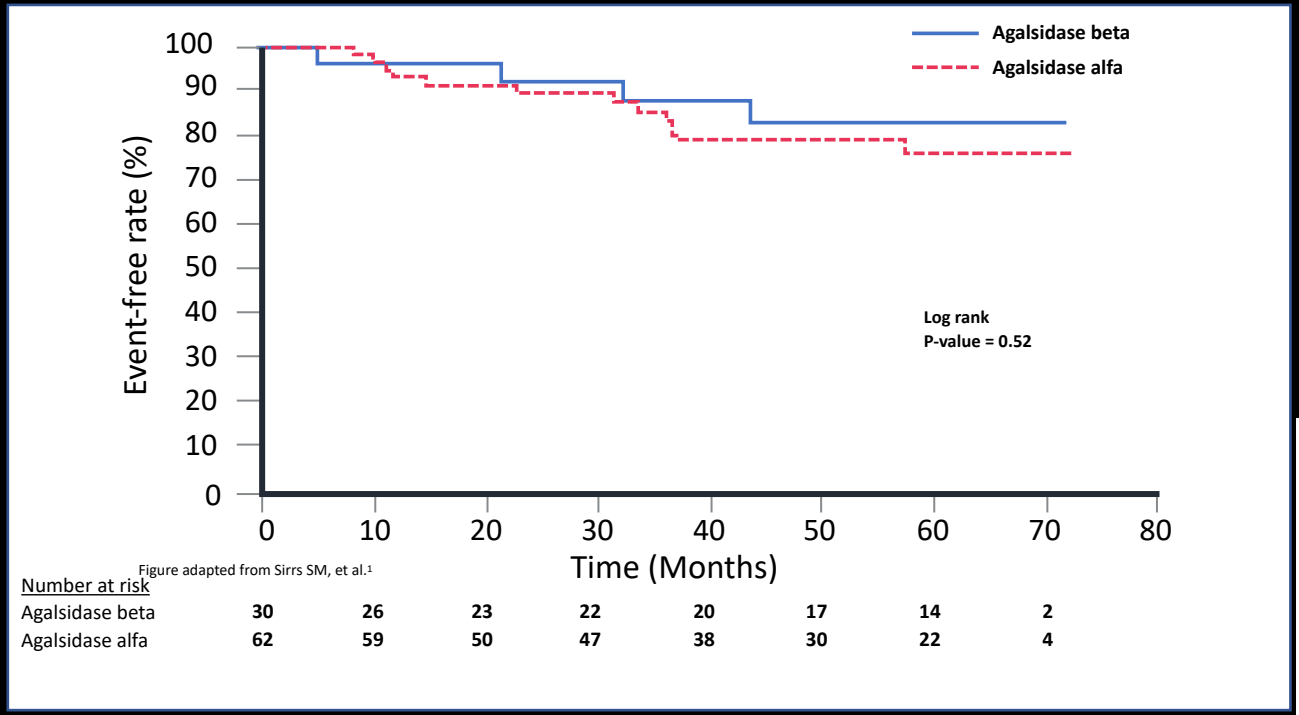
in eGFR in male

et al.
) [21]
in/1.73 m²/y

−3.0 (0.1)
−6.8 (1.5)
−0.9 (0.9)
−2.1 (1.6)
−6.9 (1.5)
−3.3 (1.8)
−1.6 (1.5)
−4.6 (2.3)
−2.2 (2.2)
−0.6 (2.6)

CANADIAN FABRY DISEASE INITIATIVE

Kaplan–Meier plot showing composite clinical endpoint for patients on algasidase alfa vs agalsidase beta (Cohort 1b)



For agalsidase alfa vs agalsidase beta

- Primary endpoint was met in 19.4% vs 13.3% of patients (*P*=0.57)
- Hazard ratio: 1.29 (*P*=0.67)

Cohort 1a Subjects receiving ERT prior to the start of the CFDI and are maintained on the ERT formulation (agalsidase alfa or agalsidase beta) at entry.

Cohort 1b Subjects naive to ERT who meet ERT criteria and undergo 1:1 randomization, stratified by gender, to either agalsidase alfa or agalsidase beta.

Cohort 1c Subjects naive to ERT who do not meet ERT criteria OR who do not consent to ERT. At any point, should a subject in Cohort 1c develop an indication for ERT, they are offered randomization into Cohort 1b.

ERT is given intravenously every other week using product monograph doses: agalsidase alfa 0.2 mg/kg and agalsidase beta 1 mg/kg.

Sirrs *et* al. *Mol Genet Metab* 2014;111:499–506

Clinical Kidney Journal

Reduction in kidney function decline and risk of severe clinical events in agalsidase beta-treated Fabry disease patients: a matched analysis from the Fabry Registry

Clinical trials of agalsidase beta may not be generalizable to all Fabry patients and do not address long-term outcomes. This analysis investigates long-term eGFR changes and risk of clinical events with agalsidase beta treatment in real-world populations.

Methods

Patients:
Agalsidase beta-treated (Fabry Registry) and untreated patients (Natural History Study) aged ≥16 years

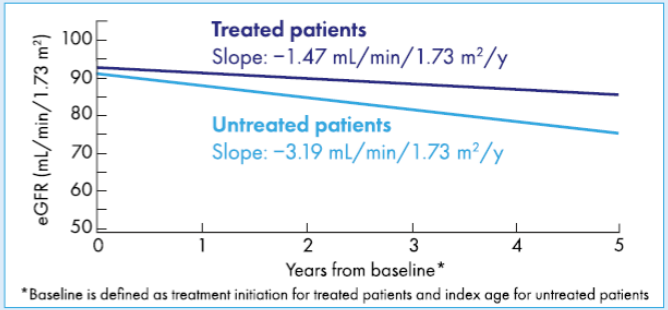
Outcomes:
- eGFR slope: change in eGFR over time
- Risk of composite clinical event: cardiovascular, cerebrovascular or renal event, or death

Analysis:
1:1 matching: untreated patients matched to similar treated patients to compare eGFR slopes and clinical events

Results

Estimated GFR slopes

53.9% reduction in rate of eGFR decline with agalsidase beta

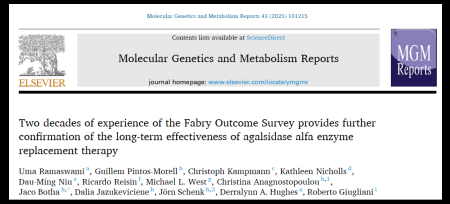


Composite clinical event

59% lower risk of composite clinical event with agalsidase beta

Conclusion: This real-world study provides evidence that long-term agalsidase beta treatment preserves kidney function and delays progression to severe clinical events among patients aged ≥ 16 years.

Batista, J. L.
Clinical Kidney Journal (2024)
Julie.Batista@sanofi.com
@CKJsocial



Molecular Genetics and Metabolism Reports 43 (2025) 101215

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Molecular Genetics and Metabolism Reports

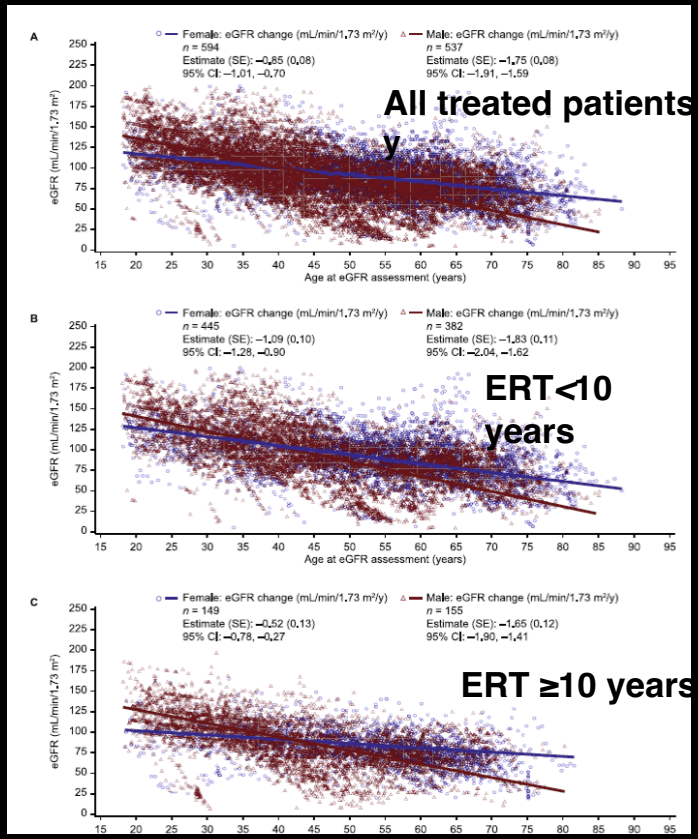
journal homepage: www.elsevier.com/locate/ymgmr

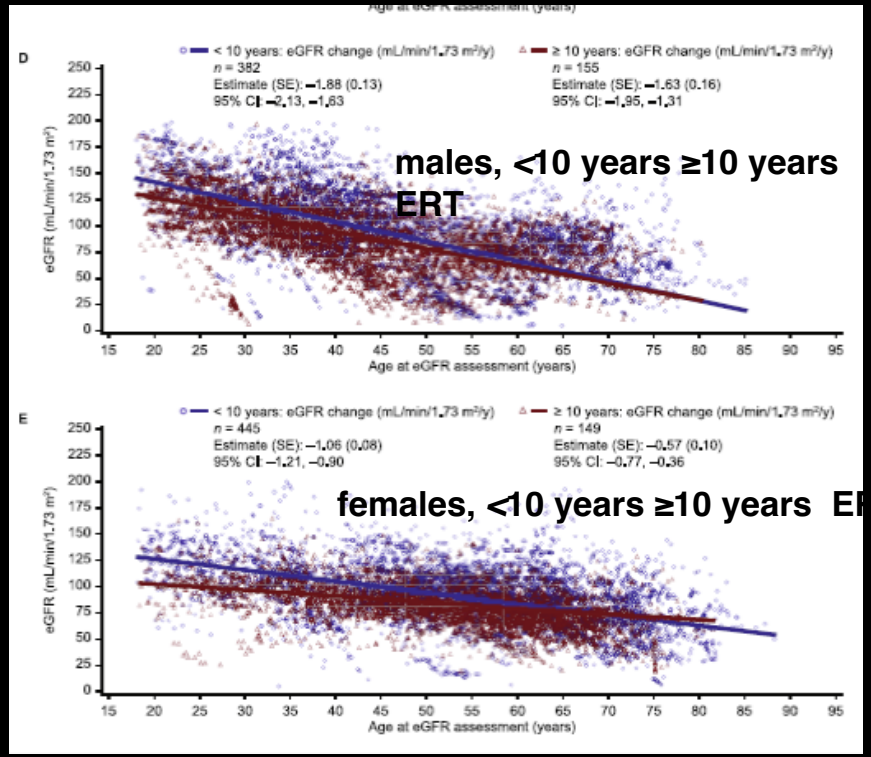
Two decades of experience of the Fabry Outcome Survey provides further confirmation of the long-term effectiveness of agalsidase alfa enzyme replacement therapy

Uma Ramaswami [a], Guillem Pintos-Morell [b], Christoph Kampmann [c], Kathleen Nicholls [d], Dau-Ming Niu [e], Ricardo Reisin [f], Michael L. West [g], Christina Anagnostopoulou [h,i], Jaco Botha [j,k], Dalia Jazukeviciene [l], Jörn Schenk [h,i], Dernlyn A. Hughes [m], Roberto Giugliani [i]

ANNUALISED eGFR CHANGE IN THE TREATED PATIENTS

Slope/y -0.85 f -1.76





Molecular Genetics and Metabolism Reports 43 (2025) 101235

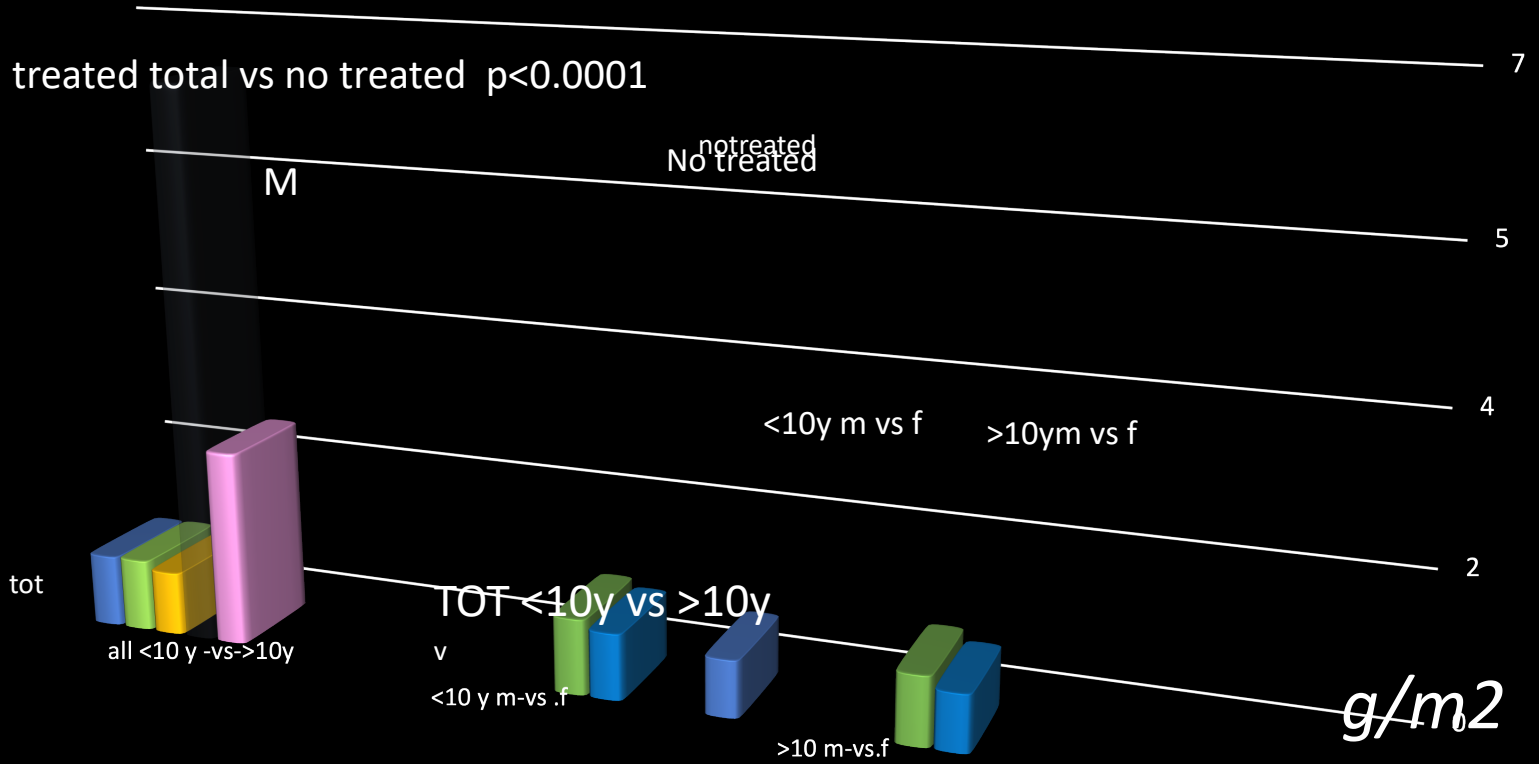
Contents lists available at ScienceDirect

Molecular Genetics and Metabolism Reports

journal homepage: www.elsevier.com/locate/ymgmr

Two decades of experience of the Fabry Outcome Survey provides further confirmation of the long-term effectiveness of agalsidase alfa enzyme replacement therapy

Uma Ramaswami [1], Guillem Pintos-Morell [2], Christoph Kampmann [3], Kathleen Nicholls [4], Dau-Ming Niu [5], Ricardo Beiún [6], Michael L. West [7], Christina Anagnostopoulou [8,9], Jaco Botha [8,9], Dalia Jazukeviciene [10], Jörn Schenk [11], Deenslynn A. Hughes [12], Roberto Giugliani [13]



Contents lists available at ScienceDirect

Molecular Genetics and Metabolism Reports

journal homepage: www.elsevier.com/locate/ymgmr

Effectiveness and tolerability of migalastat in adult Fabry disease: A single regional centre experience

Eamon P. McCarron [a,b,c], Rajkumar Chinnadurai [d,e], Karolina M. Stepien [a,e], John Bassett [a], Jonathan Meyer [a], Reena Sharma [a], Peter Woolfson [f], Ana Jovanovic [a,b,c]

Conclusion: Migalastat was well tolerated and maintained stable renal and cardiac parameters with plasma lyso-Gb3 improvement. However, a subset of patients showed progression or intolerance, underscoring that variant amenability alone may not predict clinical benefit.

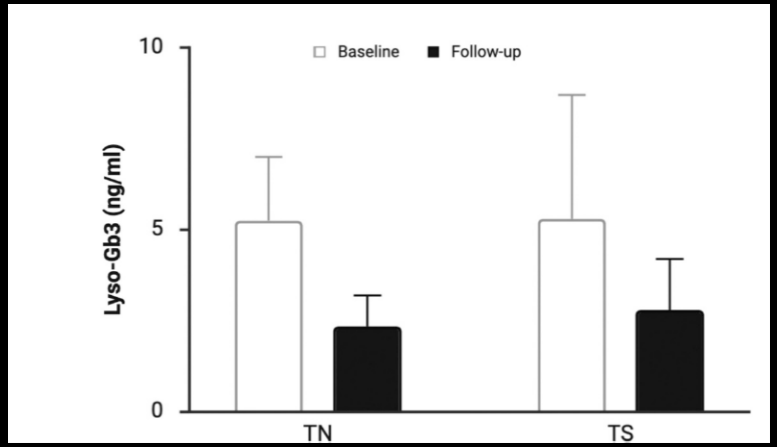


Table 3
Renal and cardiovascular parameters at baseline and follow-up in treatment-naïve and treatment-switch patients receiving migalastat.

	Treatment naïve ($N = 48$)			Treatment switch ($N = 39$)		
Variables	Baseline	Follow-up	p-Value	Baseline	Follow-up	p-Value
Biochemical						
eGFR >90, ml/min/1.73m^2	24/48 (50%)	14/48 (29%)	**0.037**	33/39 (84.6%)	12/39 (30.8%)	**<0.001**
eGFR >60, ml/min/1.73m^2	40/48 (83.3%)	34/48 (70.8%)	0.145	39/39 (100%)	35/39 (89.7%)	0.157
uPCR, mg/mmol	12 (7–21)	13 (8–30)	1.000	9 (7–16)	11 (5–19)	0.175
uACR, mg/mmol	3.7 (1.3–13.5)	4.55 (1.29–12.4)	0.343	1.8 (1–4.3)	2.15 (0.6–16)	0.564
Cardiovascular						
PR interval (ms)	158 (146–176)	169 (146–192)	0.067	156 (133–176)	163 (142.5–180.5)	0.063
QTc interval (ms)	435 (408–454.5)	444 (411–466.5)	0.319	418 (397.5–434)	442.5 (422–469)	**<0.001**
Systolic BP, mm Hg	133 (120–148)	132 (119–152)	0.257	133 (126–145)	129 (119–140)	0.060
Diastolic BP, mm Hg	80 (74–89)	82 (71–90)	0.301	80 (74–87)	82 (76–90)	0.340
Intraventricular septum at end-diastole (IVSd) (cm)	1.3 (1–1.7)	1.3 (1.2–1.65)	0.308	1.3 (0.96–1.6)	1.4 (1.2–1.7)	0.214
LVM (g)	224.6 (176.6–328.6)	237.1 (173.45–299.4)	0.739	233.5 (175.8–303.4)	219.1 (176.5–257.6)	0.110
LVMI (g/m^2)	110.9 (93.1–154.6)	113.8 (93–161.8)	0.736	121.7 (96.1–138.7)	105.3 (90.8–136.3)	0.088

Holida et al. Orphanet Journal of Rare Diseases (2026) 21:185
https://doi.org/10.1186/s13023-026-04303-8

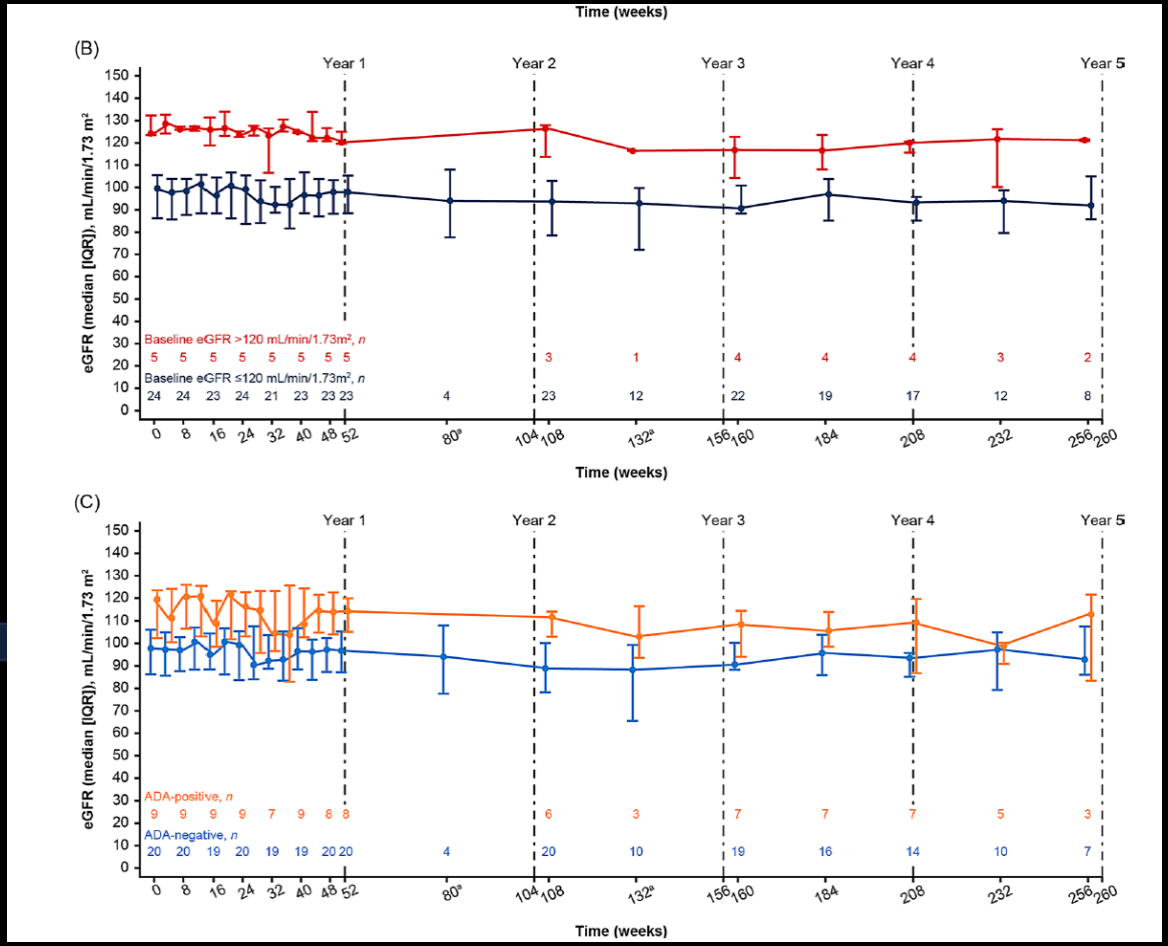
Orphanet Journal of Rare Diseases

RESEARCH Open Access

Long-term efficacy and safety of pegunigalsidase alfa administered every 4 weeks in adults with Fabry disease: results from up to 5 years of the BRIGHT F51 phase III, open-label extension study

Myrl Holida[1], Aleš Linhart[2], Nicola Longo[3], Eric Wallace[4], Camilla Tøndel[5], Derralynn Hughes[6], David G. Warnock[4], Antonio Pisani[7], François Eyskens[8], Patrick Deegan[9], Ulla Feldt-Rasmussen[10], Özlem Goker-Alpan[11], Ankit Mehta[12], Giovanni Piotti[13], Vito Fichera[13], Meng Wang[13], Raul Chertkoff[14], Stephen Waldek[15], William R. Wilcox[16] and John A. Bernat[1*]

Long-term treatment with pegunigalsidase alfa 2 mg/kg E4W was well-tolerated and Maintained disease stability, especially in females and ADA-negative males; more data are needed to better understand outcomesin ADA-positive males.



Drug Design, Development and Therapy

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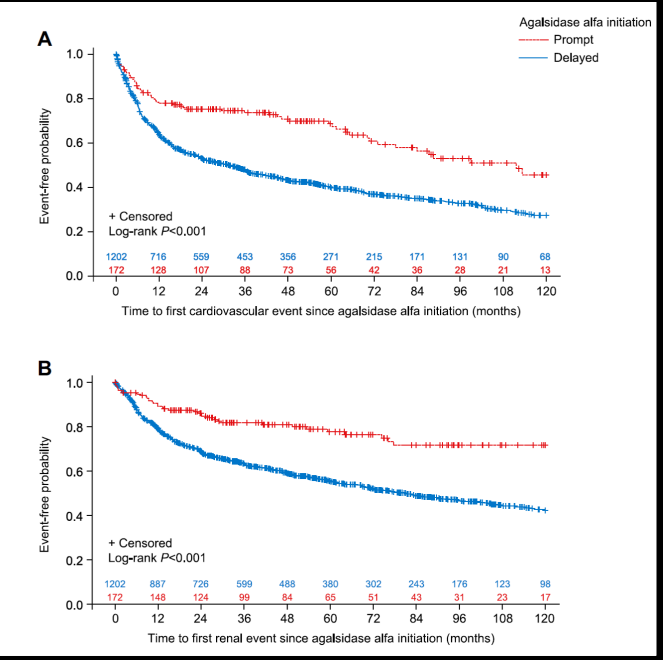
ORIGINAL RESEARCH

Prompt Agalsidase Alfa Therapy Initiation is Associated with Improved Renal and Cardiovascular Outcomes in a Fabry Outcome Survey Analysis

Drug Design, Development and Therapy 2021:15 3561–3572

Derralynn Hughes[1]
Aleš Linhart[2]
Andrey Gurevich[3]
Vasiliki Kalampoki[3]
Dalia Jazukeviciene[3]
Sandro Feriozzi[4]

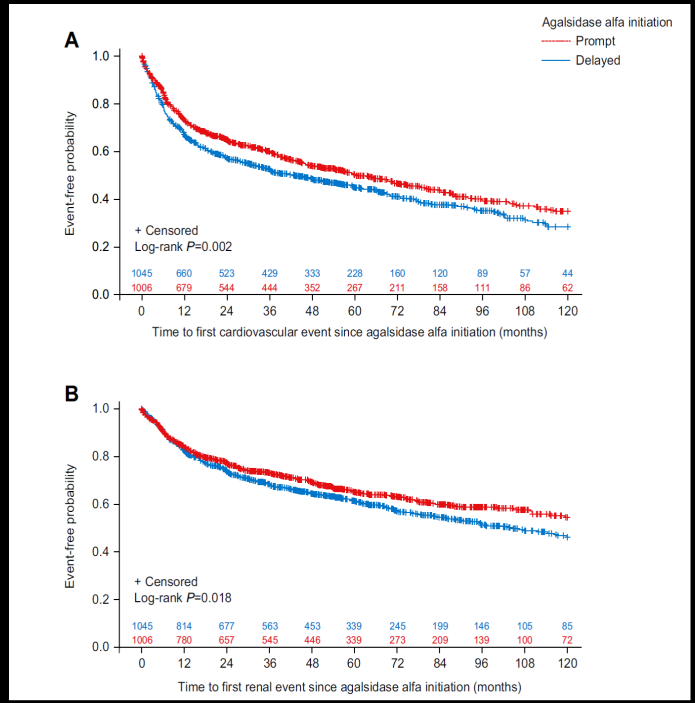
On behalf of the FOS Study Group



Analysis A: Prompt and Delayed Treatment Since Symptom Onset

"This study suggests that prompt treatment initiation with agalsidase alfa provided better renal and cardiovascular outcomes than delayed treatment in patients with Fabry disease."

Analysis B: Prompt and Delayed Treatment Since Diagnosis

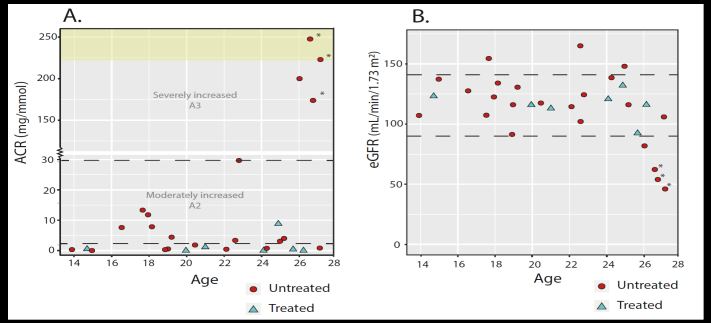


CLINICAL AND LAB HISTORY OF AN ITALIAN-GREEK BOY

Male date of birth 30/3/2008; in 2017 he received a diagnosis of Fabry disease; his mother and sister bearing classical pathogenetic mutation C1871T>C (Namazanova-Buroma 2016 Mol Genet Metab 120:S101) AGAL 0 (nv >2.3) umol/L/h

Organ	2017 / 9 yo	2018	2019	2022	2026	
heart	Echo normal range		Normal range	Norml range	Normal range	
Brain/eye	Cornea vertcillata	Angio MR neg		Angio MR neg		
Kidney Scr eGFR Microalb	O,45 142 Neg	0,42 155 Neg	0,43 157 Neg	0,43 167 Neg	0,6 144	mg/dl ml/min/1.73/ m2
Peripheral nervous system	hypoidrosis	hypoidrosis	No	no		
Lyso Gb3		166	**ERT Agalsidase A**	36,8 **ERT Agalsidase A**	25ng **ERT Agalsidase A**	ng/ml

EARLY START OF ENZYME REPLACEMENT THERAPY IN PEDIATRIC MALE PATIENTS WITH CLASSICAL FABRY DISEASE IS ASSOCIATED WITH ATTENUATED DISEASE PROGRESSION



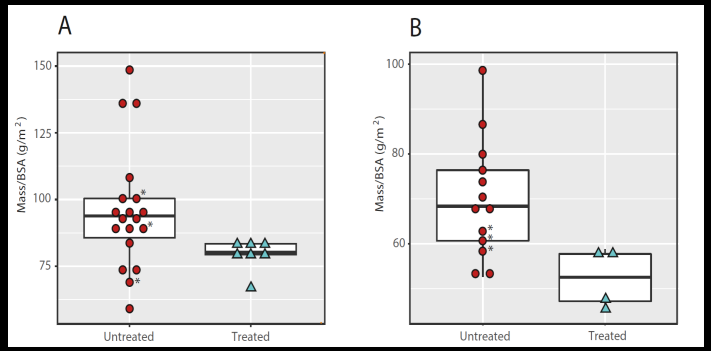


Table 1

Patient characteristics. Categorical variables are depicted as number (percentage) and continuous variables as median (range). Missing values (if any): presence of cornea verticillata ($n = 2$), reason unknown.

	Treated	Untreated	*P*-value
No of patients	7	23	
Age at evaluation (years)	24 (14–26)	22 (13–27)	0.7
Treatment duration (years)	10.4 (9.5–10.7)	–	
Mutation type			
Nonsense/Frameshift	4/7 (57%)	10/23 (43%)	1.0
Missense	3/7 (43%)	11/23 (48%)	
Other	0/7 (0%)	2/23 (9%)	
Reason for diagnosis			
Family screening	5/7 (71%)	10/23 (43%)	0.5
Acroparesthesia	2/7 (29%)	10/23 (43%)	
Renal insufficiency or albuminuria[a]	0/7 (0%)	3/23 (13%)	
Untreated plasma lysoGb3 (nmol/l)	118 (89–215)[b]	102 (65–137)	0.07
Clinical features of classical FD (at diagnosis)			
Acroparesthesia	7/7 (100%)	22/23 (96%)	1.0
Angiokeratomas	4/7 (57%)	17/23 (74%)	0.6
Cornea verticillata	6/7 (86%)	17/21 (81%)	1.0
Hypertension	0/7 (0%)	1/23 (4%)[c]	1.0
Use of ACEi or ARB	0/7 (0%)	4/23 (17%)	0.5

Reproduced from van der Veen SJ, et al. 2022

"...to provide clinical evidence that treatment of male cFD patients before the age of 16 has a beneficial impact on progression…"

Figures reproduced from van der Veen SJ, et al. 2022

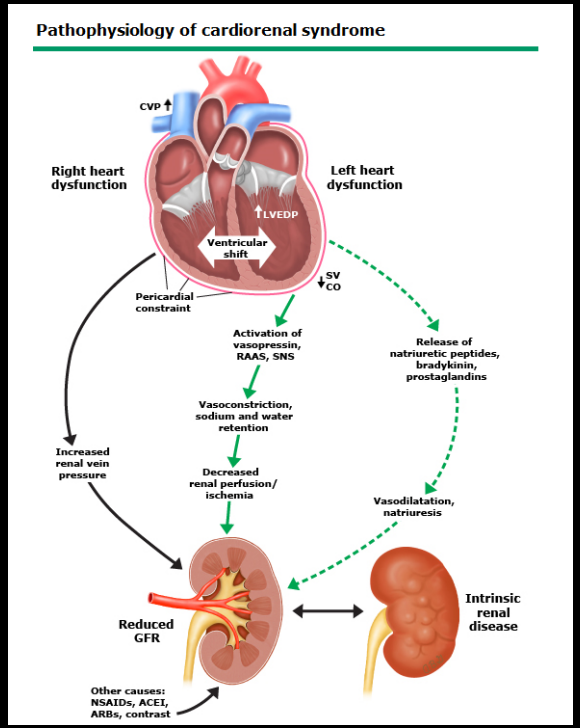
ACEi, angiotensin-converting enzyme inhibitor; ACR, albumin-to-creatinine ratio; ARB, angiotensin receptor blocker; BSA, body surface area; cFD, classical disease phenotype of Fabry disease; eGFR, estimated glomerular filtration rate; FD, Fabry disease; lyso-Gb3, globotriaosylsphingosine.

van der Veen, SJ, et al. *Mol Genet Metab*. 2022;135(2):163–169.

van der Veen, Mol Genet Metab. 2022.

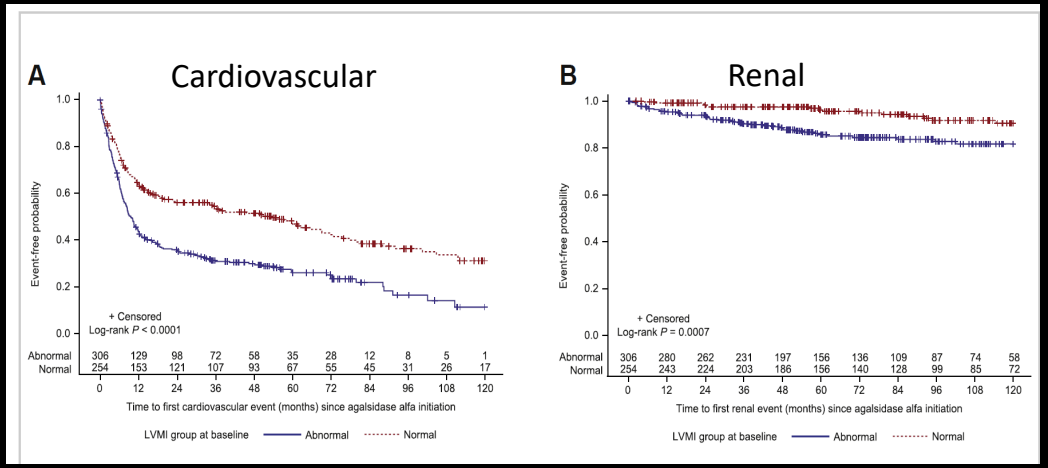
CARDIORENAL SYNDROME

- *Type 1 (acute*) – Acute HF results in AKI
- *Type 2 – Chronic cardiac dysfunction*) causes chronic kidney disease (CKD)).
- *Type 3 – Abrupt and primary worsening of kidney function* Ex.renal ischemia or glomerulonephritis with acute cardiac dysfunction
- *Type 4 – Primary CKD with* contributes to cardiac dysfunction
- *Type 5 (secondary) – Acute or chronic systemic disorders (*eg, sepsis or diabetes mellitus) that cause both cardiac and renal dysfunction.



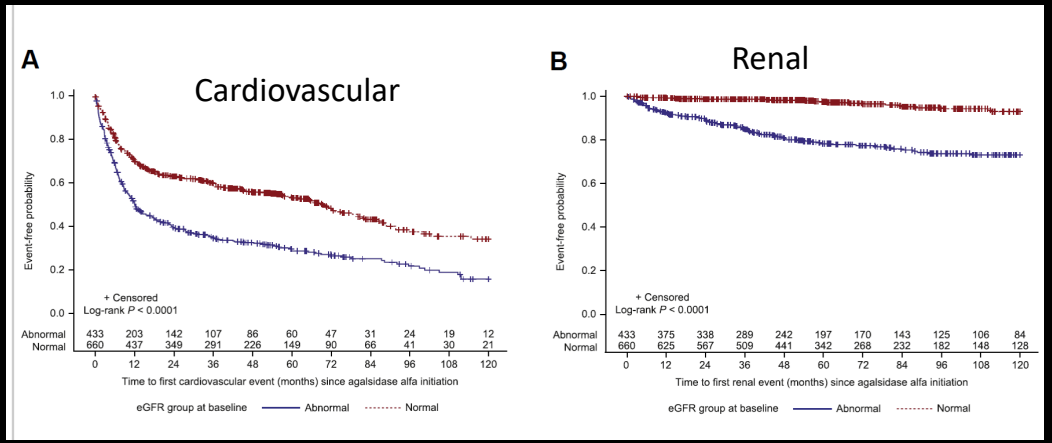
UpToDate 2020

CARDIOVASCULAR AND RENAL EVENTS



Occurrence of events in patients with or without LVH

The findings suggest that cardiovascular and renal pathologies are closely inter-related



Occurrence of events in patients with low or normal GFR

ADDITIONAL EVIDENCE IN LITERATURE

Heart 2015;**101**:287–293.

Special populations

ORIGINAL ARTICLE

Cardiovascular outcomes in Fabry disease are linked to severity of chronic kidney disease

Andrew S Talbot,[1] Nigel T Lewis,[2] Kathy M Nicholls[1,3]

RESEARCH ARTICLE

Retrospective study of long-term outcomes of enzyme replacement therapy in Fabry disease: Analysis of prognostic factors

Maarten Arends[1]*, Marieke Biegstraaten[1], Derralynn A. Hughes[2], Atul Mehta[2], Perry M. Elliott[3], Daniel Oder[4], Oliver T. Watkinson[3], Frédéric M. Vaz[5], André B. P. van Kuilenburg[5], Christoph Wanner[4], Carla E. M. Hollak[1]

PLOS ONE | https://doi.org/10.1371/journal.pone.0182379 August 1, 2017

Table 5. Multivariate analysis.

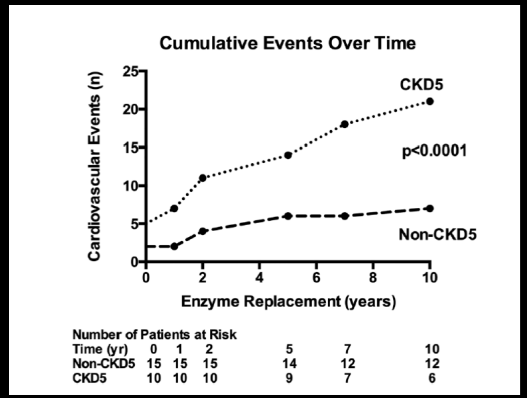
	HR	95% CI
eGFR (per -10 ml/min/1.73m^2)	1.12††	1.03–1.22
LVMI (per 10 gram/m$^{2.7}$)	1.16	0.99–1.36
Event(s) before ERT	1.45	0.85–2.53

Cox regression analysis on the clinical event rate, adjusted for age, sex and phenotype. The hazard ratios (HR) of eGFR, LVMI and events before ERT on the clinical event rate were calculated in a multivariate model. Number of patients included in the analysis: 233. eGFR: estimated glomerular filtration rate, LVMI: left ventricular mass index measured by echocardiography adjusted for height$^{2.7}$, ERT: enzyme replacement therapy.

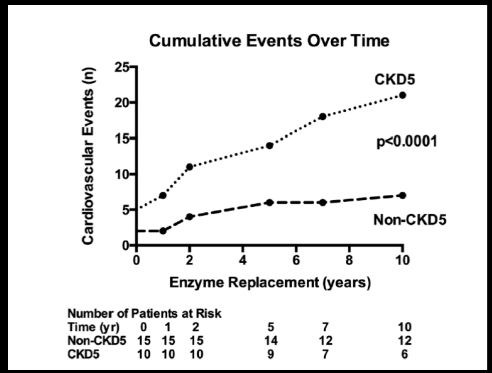
††$p < 0.01$;

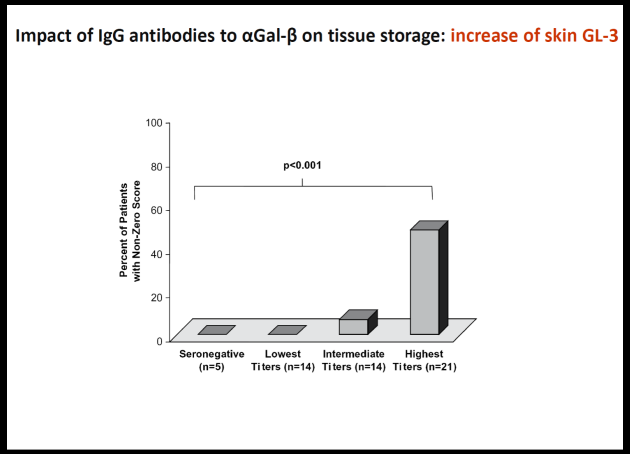
«the multivariate analysis….

- *eGFR indipendently resulted in an increased clinical vent rate. …a trend towards an inncresed event rate with higher LVMI»*



- *«ESRD was the strongest indicator of cardiovascolar progression*
- *ERT was associated with stability in cardiac an renal disease*





ORIGINAL ARTICLE

Agalsidase alfa versus agalsidase beta for the treatment of Fabry disease: an international cohort study

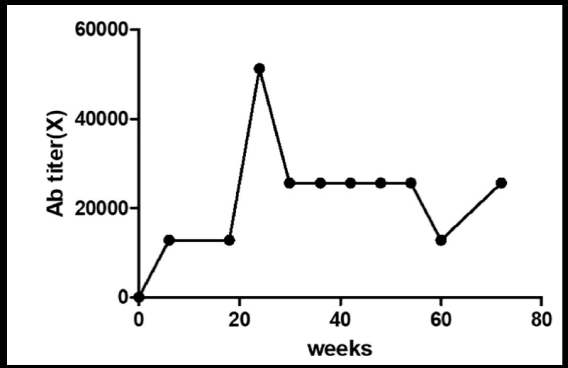
Maarten Arends,[1] Marieke Biegstraaten,[1] Christoph Wanner,[2] Sandra Sirrs,[3] Atul Mehta,[4] Perry M Elliott,[5,6] Daniel Oder,[2] Oliver T Watkinson,[5,6] Daniel G Bichet,[7] Aneal Khan,[8] Mark Iwanochko,[9] Frédéric M Vaz,[10] André B P van Kuilenburg,[10] Michael L West,[11] Derralynn A Hughes,[4] Carla E M Hollak[1]

J Med Genet 2018;**55**:351–358. doi:10.1136/jmedgenet-2017-104863

Table 1 Patient characteristics at start of ERT

	Agalsidase alfa (0.2 mg/kg)	Agalsidase beta (1.0 mg/kg)	P value
Patients	248	139	
Men, classical*	69 (28%)	71 (51%)	<0.001
Men, non-classical	47 (19%)	7 (5%)	0.22
Women, classical	95 (38%)	43 (30%)	0.14
Women, non-classical	37 (15%)	18 (13%)	0.86
ERT start <18 years of age	15 (6%)	3 (2%)	0.13
Follow-up time (years)	5.2 (0.8–14.4)	3.8 (0.8–12.1)	<0.001
Events before initiation of ERT			
Dialysis/renal transplant	8 (3%)	12 (9%)	0.007
PM/ICD	21 (8%)	9 (7%)	0.87
Stroke	22 (9%)	17 (12%)	0.09
Any of the above†	46 (19%)	31 (22%)	0.08
LysoGb3 (nmol/L)	10 (0.7–146)	80 (2.0–178)	<0.001
eGFR (mL/min/1.73 m^2)	89 (10–159)	86 (10–140)	0.009
CKD category A3	44/195 (23%)	42/113 (37%)	0.008
LVMI (g/m$^{2.7}$)	49 (15–117)	52 (20–148)	0.14
Use of ACEi/ARBs	89/248 (36%)	52/139 (37%)	0.83
Hypertension	109/236 (39%)	62/137 (45%)	0.23
BMI (kg/m^2)	26 (±4.9)	25 (±5.6)	0.30
HDL cholesterol (mmol/L)	1.5 (±0.4)	1.5 (±0.4)	0.92
LDL cholesterol (mmol/L)	2.7 (±0.9)	2.7 (±0.8)	0.76
Total cholesterol (mmol/L)	4.8 (±1.1)	4.7 (±1.0)	0.51
Triglycerides (mmol/L)	1.2 (0.2–5.9)	1.2 (0.3–3.6)	0.18

ADA



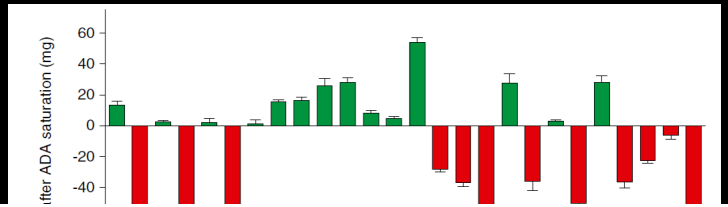
Konto Mol Gen Met 2020

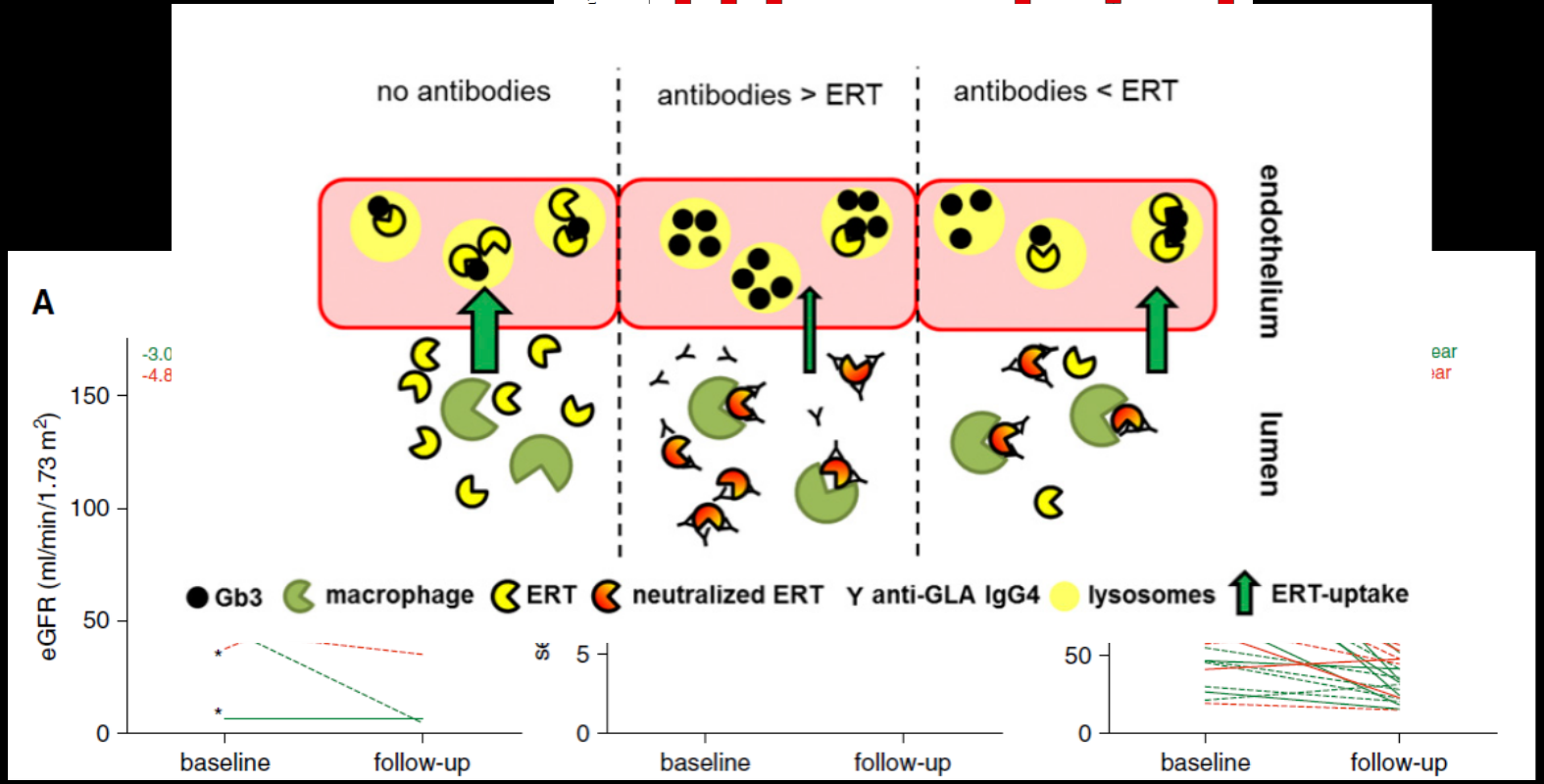
***Male classical phenotype #81**:*
11 of 39 agalsidase-alfa- (28%)
22 of 42 (52%) were positive for

CLINICAL RESEARCH www.jasn.org

Dose-Dependent Effect of Enzyme Replacement Therapy on Neutralizing Antidrug Antibody Titers and Clinical Outcome in Patients with Fabry Disease

Malte Lenders,[1] Leon Paul Neußer,[1] Michael Rudnicki,[2] Peter Nordbeck,[3] Sima Canaan-Kühl,[4] Albina Nowak,[5] Markus Cybulla,[6] Boris Schmitz,[7] Jan Lukas,[8] Christoph Wanner,[3] Stefan-Martin Brand,[7] and Eva Brand[1]



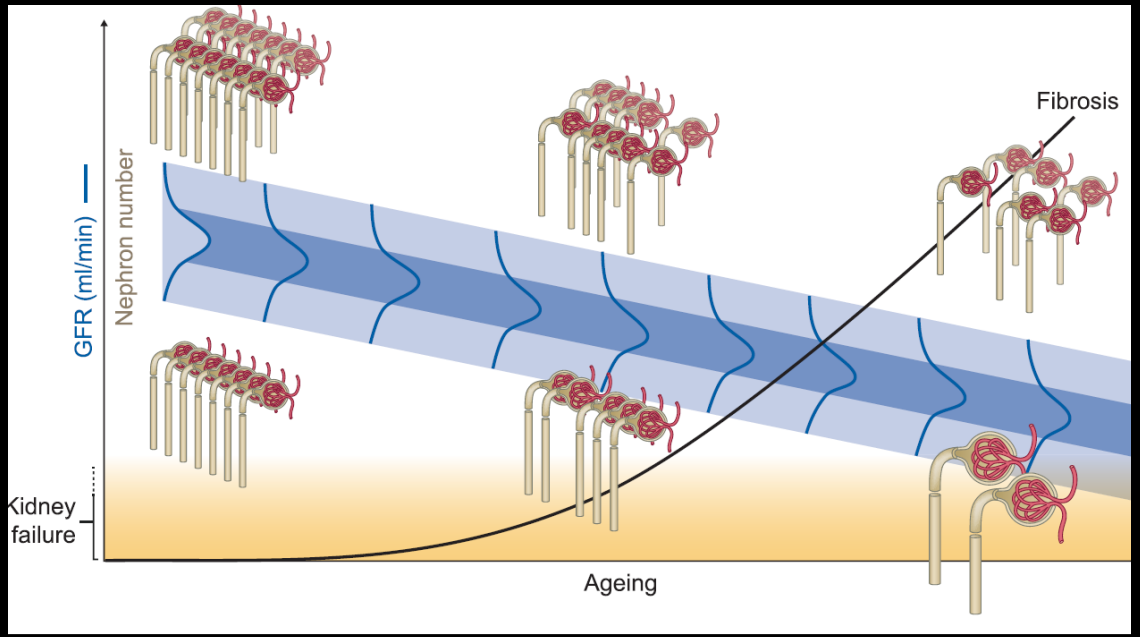


Nephrol Dial Transplant (2020) 1–7
doi: 10.1093/ndt/gfaa329

ndt
Nephrology Dialysis Transplantation

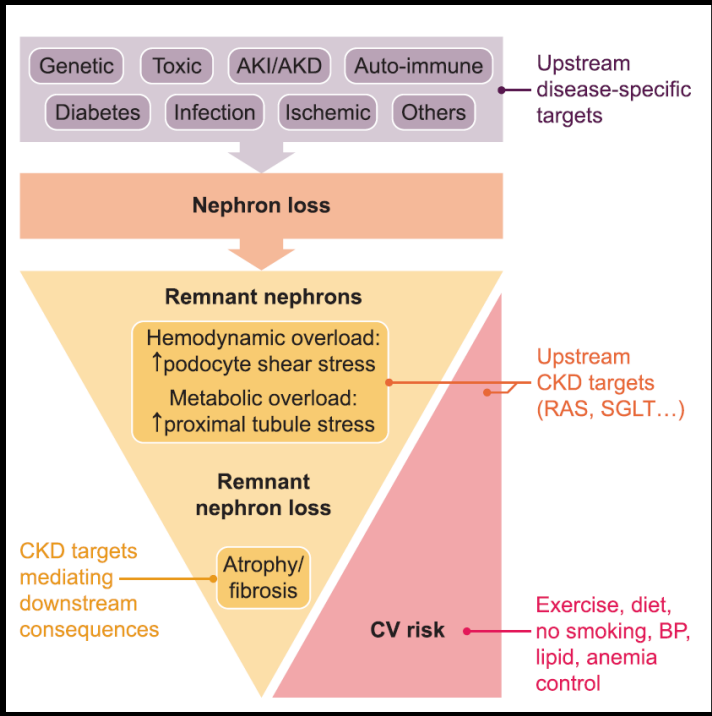
SGLT2 inhibition requires reconsideration of fundamental paradigms in chronic kidney disease, 'diabetic nephropathy', IgA nephropathy and podocytopathies with FSGS lesions

Hans-Joachim Anders[1], Anna Julie Peired[2,3] and Paola Romagnani[3,4]



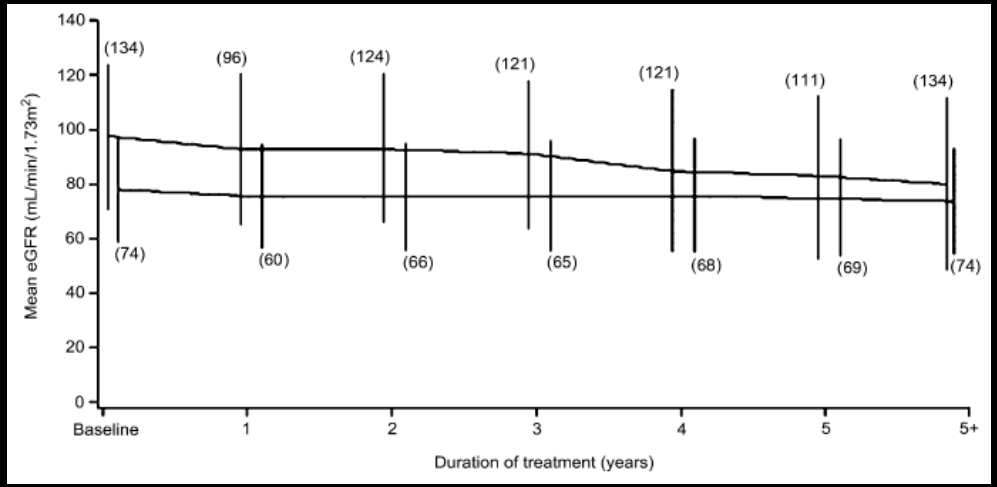
Healthy ageing:
Declining nephron number/GFR + fibrosis still match body needs = ***normal*** nephron dimensions

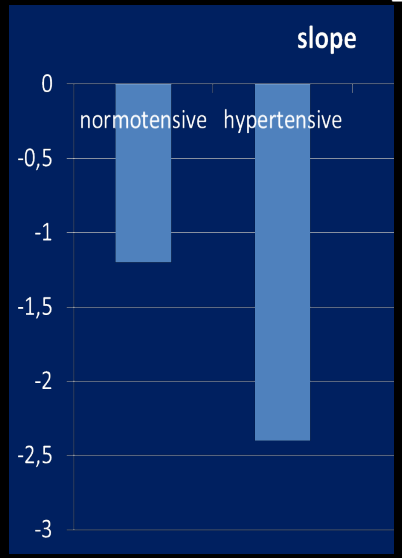
Chronic kidney disease:
Nephron number/GFR + fibrosis ***no longer*** match body needs = functional overload of remnant nephrons, which triggers structural adaptations that drive further nephron loss = CKD progression

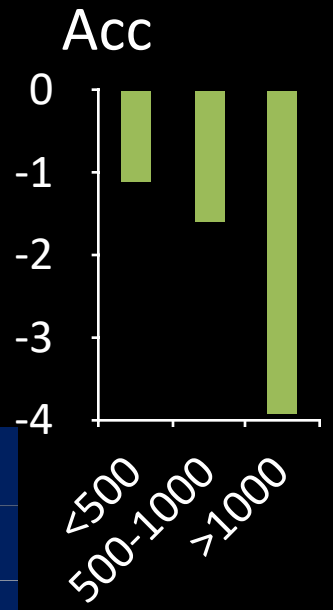


The Effectiveness of Long-Term Agalsidase Alfa Therapy in the Treatment of Fabry Nephropathy

Sandro Feriozzi, Joan Torras,† Markus Cybulla,‡ Kathy Nicholls,§ Gere Sunder-Plassmann,‖ and Michael West,¶ on behalf of the FOS Investigators*







ANTI- PROTEINURIC THERAPIES

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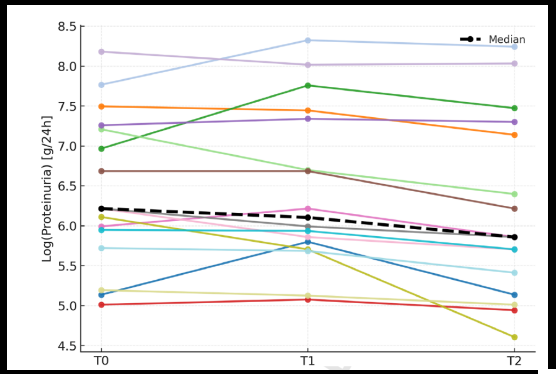
CLINICAL RESEARCH

Dapagliflozin in Patients With CKD With Fabry Disease

Yuri Battaglia[1,2,13], Nicola Vitturi[3,13], Giacomo Marchi[4], Federica Baciga[1,2], Federica Caccia[1], Giorgia Gugelmo[2], Loris Martinetti[5], Lucia Federica Stefanelli[5], Sara Torresani[1], Antonio Pisani[6], Eleonora Riccio[6], Pasquale Esposito[2], Francesca Viazzi[7], Cristina Chimenti[8], Francesca Katiana Martino[9], Livia Lenzini[6], Annalisa Sechi[10], Luca Zanoli[11,12], Domenico Girelli[4], Gian Paolo Fadini[3], Federico Nalesso[5,13] and Gianni Carraro[5]



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https://doi.org/10.1093/ndt/gfaf088
Advance access publication date: 8 May 2025

Semaglutide in non-diabetic patients with Fabry disease

Eleonora Riccio, Ivana Capuano and Antonio Pisani

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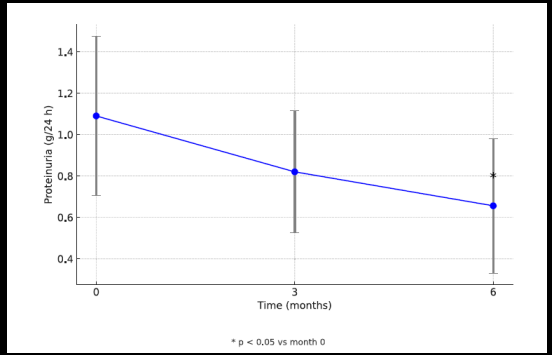
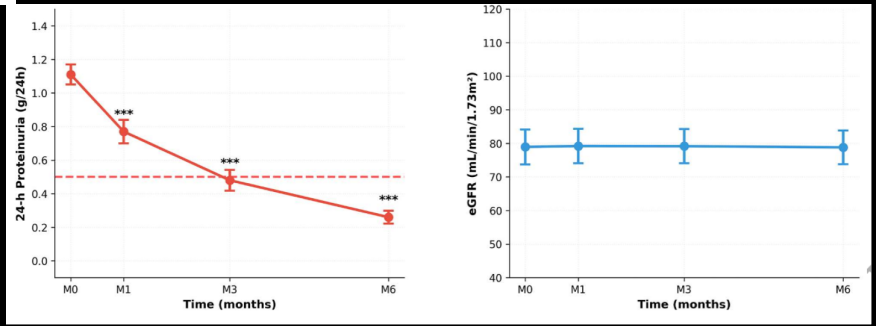


› Nephrol Dial Transplant. 2026 May 20:gfag115. doi: 10.1093/ndt/gfag115. Online ahead of print.

DUAL GIP/GLP-1Ra reduces residual proteinuria in non-diabetic fabry disease

Eleonora Riccio [1], Alessandra Cuomo [2], Ivana Capuano [1], Letizia Spinelli [2], Guido Iaccarino [2], Oriana De Marco [3], Antonio Pisani [1]



CONCLUSION

- The studies and the registries provided robust basic evidence for Fabry disease thera

- Real life and the researchers' improving of direct expertise led to a focus on some critical points.

- At present, the availability of different therapeutic options is challenging to target to individual patients.

- New medications for the supportive therapy are now available to prevent chronic organ damage.