



Correspondence

Response to Gurevich and colleagues: The effect of enzyme replacement therapy on clinical outcomes in male patients with Fabry disease: a systematic literature review by a European panel of experts


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Dear Editor,

We are grateful to Gurevich and colleagues for their correspondence [1], highlighting our review of clinical outcomes in male patients with Fabry disease after enzyme replacement therapy (ERT) [2]. This is one of a series of papers published in *Molecular Genetics and Metabolism (MGM)* or *MGM Reports* that describe the results of a unique systematic review of the published literature on therapeutic outcomes in patients with Fabry disease, initiated and guided by established experts in this field with extensive experience in patients' clinical care [2–6].

We commend the authors for highlighting the importance of standardisation in the design, conduct and reporting of systematic reviews and concur with their comments on the necessity to report on bias in the published literature. The initial series, as submitted to MGM, included a manuscript with the detailed description of the methodology and bias evaluation for all papers. However, MGM's peer review as stand-alone publication resulted in conflicting feedback from reviewers, and this paper has now been published by the *European Journal of Medical Genetics* [7].

We confirm that the data search and extraction for this series was conducted in line with recommended practice and that our analysis included a grading of evidence using the levels of evidence classification published by the Oxford Centre for Evidence-Based Medicine [8]. All Grade 1–3 publications were assessed for risk of bias based on the Cochrane tool for assessing bias originally developed for randomised trials [9]. Importantly, pharmaceutical sponsorship of a study was also noted and when insufficient data were available to make a judgement on its impact, the item was noted as a potential risk.

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Declaration of Competing Interest

D.P. Germain is a consultant for Amicus Therapeutics, Sanofi Genzyme and Takeda, and has received speaker honoraria from Amicus Therapeutics, Sanofi Genzyme and Takeda.

B. Falissard has been a consultant for Actelion, Allergan, Ammirall, Astellas, AstraZeneca, Bayer, Biotronik, Boehringer Ingelheim, Bristol-Myers Squibb, Daiichi-Sankyo, Eli Lilly and Company, Gilead Sciences, GlaxoSmithKline, Grünenthal, HRA Pharma, Janssen, Lundbeck, MSD, Novartis, Otsuka, Pierre Fabre, Roche, Sanofi, Sanofi Genzyme, Servier, Stallergene, UCB Pharmaceuticals and ViiV Healthcare.

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M. Spada has received speaker and advisory board honoraria and travel support from Sanofi Genzyme and Takeda.

C. Wanner has received research support from Sanofi Genzyme, is a consultant for Actelion Pharmaceuticals, Protalix, Boehringer Ingelheim and Sanofi Genzyme, and is a member of the European Advisory Board of the Fabry Registry.

P.M. Elliott has received speaker honoraria from Takeda, and has received consultancy and speaker honoraria from Gilead Sciences, MyoKardia, Pfizer and Sanofi Genzyme.

References

- [1] A. Gurevich, J. Schenk, H. Wellhoefer, V. Kalampoki, A response to Germain, et al., The effect of enzyme replacement therapy on clinical outcomes in male patients with Fabry disease: a systematic literature review by a European panel of experts, *Mol. Genet. Metab. Rep.* 19 (2019) 100471, <https://doi.org/10.1016/j.ymgmr.2019.100471> (Feb 6 2019).
- [2] D.P. Germain, P.M. Elliott, B. Falissard, V.V. Fomin, M.J. Hilz, A. Jovanovic, I. Kantola, A. Linhart, R. Mignani, M. Namdar, A. Nowak, J.P. Oliveira, M. Pieroni, M. Viana-Baptista, C. Wanner, M. Spada, The effect of enzyme replacement therapy on clinical outcomes in male patients with Fabry disease: a systematic literature review by a European panel of experts, *Mol. Genet. Metab. Rep.* 19 (2019) 100454, <https://doi.org/10.1016/j.ymgmr.2019.100454>.
- [3] M. Spada, R. Baron, P.M. Elliott, B. Falissard, M.J. Hilz, L. Monserrat, C. Tøndel, A. Tyłki-Szymańska, C. Wanner, D.P. Germain, The effect of enzyme replacement therapy on clinical outcomes in paediatric patients with Fabry disease - a systematic literature review by a European panel of experts, *Mol. Genet. Metab.* 126 (2019) 212–223, <https://doi.org/10.1016/j.ymgme.2018.04.007>.

- [4] D.P. Germain, M. Arad, A. Burlina, P.M. Elliott, B. Falissard, U. Feldt-Rasmussen, M.J. Hilz, D.A. Hughes, A. Ortiz, C. Wanner, F. Weidemann, M. Spada, The effect of enzyme replacement therapy on clinical outcomes in female patients with Fabry disease - a systematic literature review by a European panel of experts, *Mol. Genet. Metab.* 126 (2019) 224–235, <https://doi.org/10.1016/j.ymgme.2018.09.007>.
- [5] C. Wanner, M. Arad, R. Baron, A. Burlina, P.M. Elliott, U. Feldt-Rasmussen, V.V. Fomin, D.P. Germain, D.A. Hughes, A. Jovanovic, I. Kantola, A. Linhart, R. Mignani, L. Monserrat, M. Namdar, A. Nowak, J.P. Oliveira, A. Ortiz, M. Pieroni, M. Spada, A. Tylki-Szymańska, C. Tøndel, M. Viana-Baptista, F. Weidemann, M.J. Hilz, European expert consensus statement on therapeutic goals in Fabry disease, *Mol. Genet. Metab.* 124 (2018) 189–203, <https://doi.org/10.1016/j.ymgme.2018.06.004>.
- [6] C. Wanner, D.P. Germain, M.J. Hilz, M. Spada, B. Falissard, P.M. Elliott, Therapeutic goals in Fabry disease: recommendations of a European expert panel, based on current clinical evidence with enzyme replacement therapy, *Mol. Genet. Metab.* 126 (2019) 210–211, <https://doi.org/10.1016/j.ymgme.2018.04.004>.
- [7] P.M. Elliott, D.P. Germain, M.J. Hilz, M. Spada, C. Wanner, B. Falissard, Why systematic literature reviews in Fabry disease should include all published evidence, *Eur. J. Med. Genet.* (2019) 103702Epub ahead of print <https://doi.org/10.1016/j.ejmg.2019.103702>.
- [8] CEBM, Oxford Centre for Evidence-based Medicine – Levels of Evidence, <https://www.cebm.net/oxford-centre-evidence-based-medicine-levels-evidence-march-2009>, (March 2009) (accessed 22.06.2019).
- [9] J.P. Higgins, D.G. Altman, P.C. Gøtzsche, P. Jüni, D. Moher, A.D. Oxman, J. Savovic, K.F. Schulz, L. Weeks, J.A. Sterne, Cochrane Bias Methods Group, Cochrane

Statistical Methods Group, The Cochrane Collaboration's tool for assessing risk of bias in randomised trials, *BMJ* 343 (2011) d5928, <https://doi.org/10.1136/bmj.d5928>.

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