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Correspondence

ERT impact on left ventricular mass in Fabry disease

To the Editor,

We read with interest the systematic review on clinical outcomes in Fabry males [1]. However, two statements did not fully represent the results of the original publication cited: “One of these noted a statistically significant decrease from baseline in LVM with ERT in patients without fibrosis, but no such effect in patients with mild or severe fibrosis [174]” and “the importance of administering ERT early was demonstrated in patients without fibrosis at baseline. They experienced a statistically significant decline from baseline in LVM and a statistically significant improvement in exercise capacity with ERT, while patients in whom treatment was started when mild or severe fibrosis was already present experienced no effect of ERT [174].” Both refer to table 3 of Weidemann et al., which is not easy to interpret since the meaning of the *p* values differs for the different lines of the table [2]. Thus, in the no fibrosis group, LV mass decreased from 238 ± 42 to 202 ± 46 g and this mean decrease of 36 g was statistically significant ($p < .01$), as correctly reported by Germain et al. However, in mild fibrosis, the LV mass decreased from 275 ± 62 to 244 ± 65 and this 31 g decrease did not differ from the decrease observed in the no fibrosis group (p 0.31 vs changes observed in the no fibrosis group), similar to the observation in the severe fibrosis group (LV decreased from 303 ± 84 to 247 ± 45 g, a mean decrease of 56 g that did not differ from the decrease observed in the no fibrosis group (p 0.24 vs changes observed in the no fibrosis group). Thus, a significant decrease in LV mass was observed in patients with no fibrosis and the decrease in LV mass observed in patients with fibrosis did not significantly differ from the decrease observed in patients with no fibrosis.

Declaration of Competing Interest

Consultant for Sanofi-Genzyme, lecture or consultancy fees from Sanofi-Genzyme Freeline, Shire, Amicus.

References

[1] D.P. Germain, P.M. Elliott, B. Falissard, 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) Feb 6. (100454).

[2] F. Weidemann, M. Niemann, F. Breunig, et al., Long-term effects of enzyme replacement therapy on Fabry cardiomyopathy: evidence for a better outcome with early treatment, Circulation. 119 (4) (2009) 524–529 Feb 3.

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