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Rescue of a lysosomal storage disorder caused by *Grn* loss of function with a brain penetrant progranulin biologic

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In the originally published version of this article, we reported tissue and fluid concentrations of PTV:PGRN and Fc:PGRN following IV administration in a subset of panels from Figures 5 and S5. Following publication, further characterization of PTV:PGRN and assay optimization for drug pharmacokinetic profiling was conducted as part of the ongoing clinical investigation of PTV:PGRN. During these efforts, we discovered that the tissue drug concentrations reported in this manuscript did not account for a uniform, 10-fold dilution factor introduced during tissue homogenization. As a result, the y-axis values in Figures 5C, S5B, and S5D should be one order of magnitude higher than originally reported. To validate this finding, the study was repeated with the original assay format and drug doses, and the dilution factor error was confirmed. This calculation error does not alter the relative relationship of drug uptake in the brain between doses and molecule architecture, nor does it impact the conclusions drawn based on the original data, but it does suggest that brain uptake of drug is 10-fold higher than previously reported. No changes were made in the text of the manuscript, only in the figures. The corrected Figures 5C, S5B, and S5D are shown here. We apologize for any confusion this error may have caused.

Supplementary Material

Refer to Web version on PubMed Central for supplementary material.

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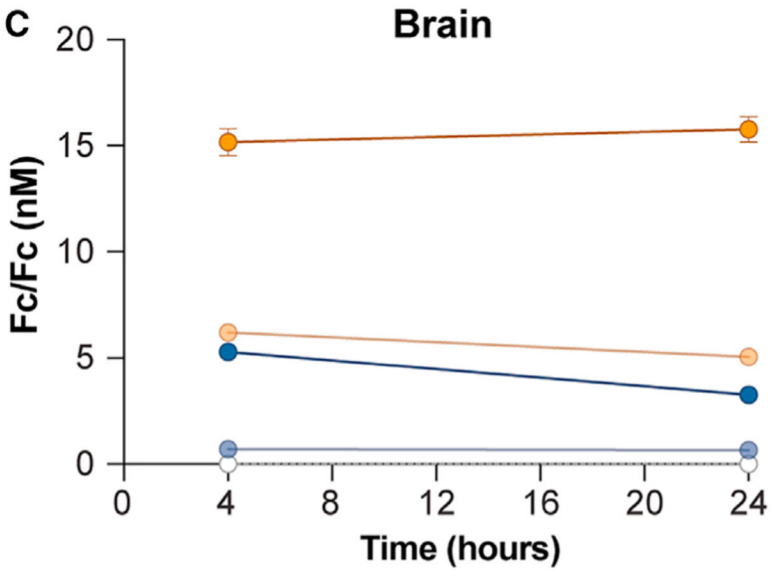

Figure 5C.
PTV:PGRN increases brain uptake and lysosomal lipid rescue in *Grn*$^{-/-}$; TfR$^{mu/hu}$ mice relative to Fc:PGRN