EMBO Molecular Medicine

Disease modeling of a mutation in α-actinin 2 guides clinical therapy in hypertrophic cardiomyopathy

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Referee #2

I read through the letter. I agree the second site does not significantly change the findings. The phenomenon of losing one allele through premature truncation/NMD and having the second WT allele produce the normal amount of protein is well-described for many sarcomere genes/proteins. So the authors might want to add that this is another approach to fixing the underlying defect- knocking down the mutant allele.