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Supplemental Information

Overexpression of Mutant FKRP Restores Functional Glycosylation and Improves Dystrophic Phenotype in FKRP Mutant Mice

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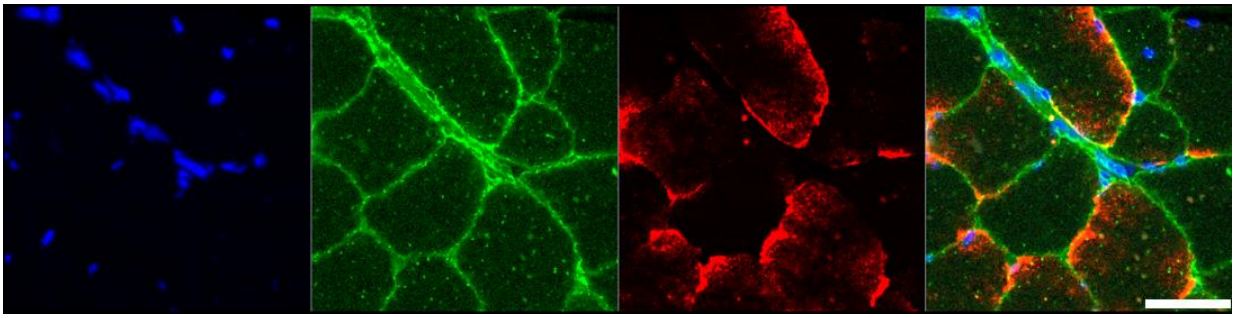
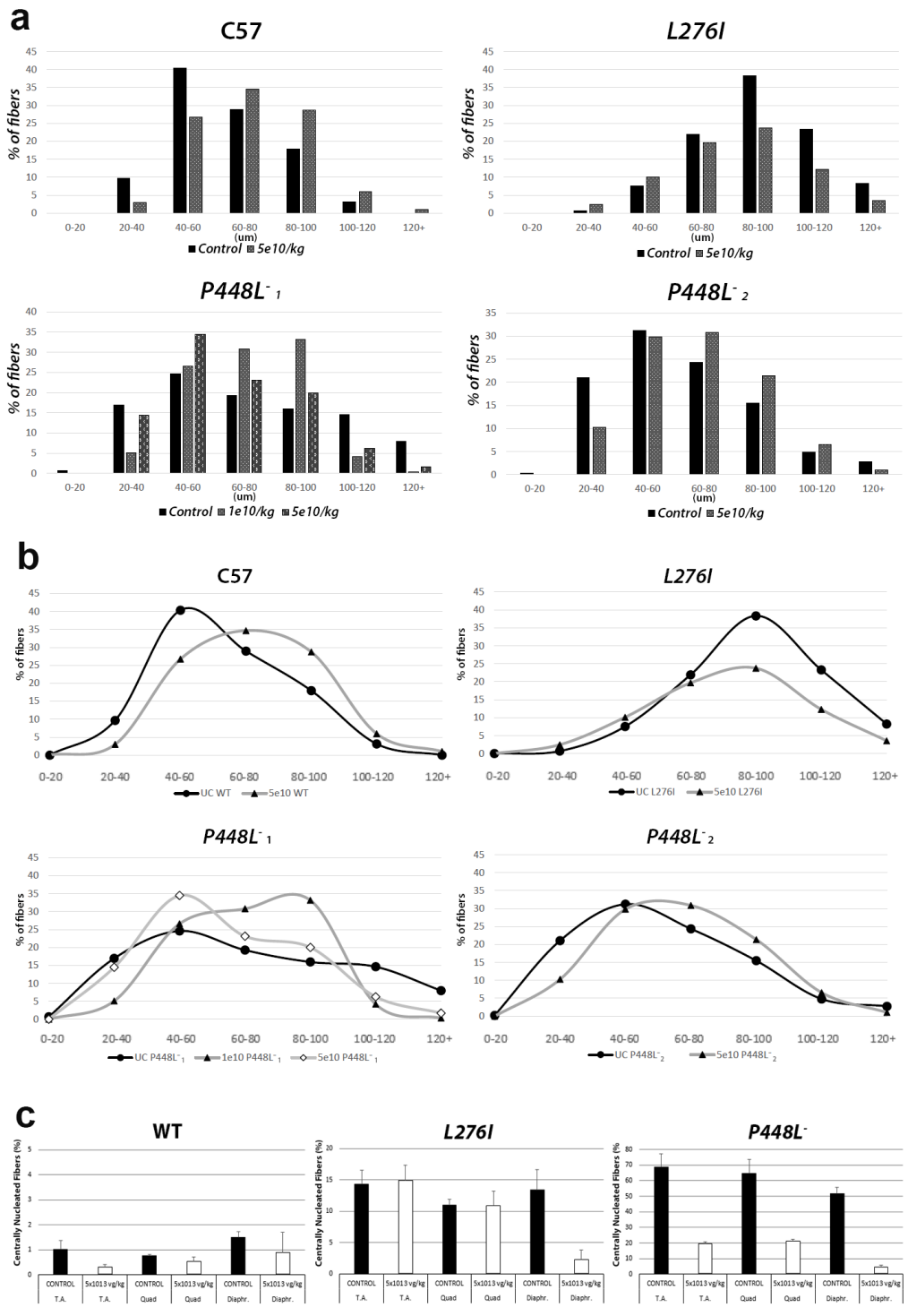
SUPPLEMENTAL INFORMATION

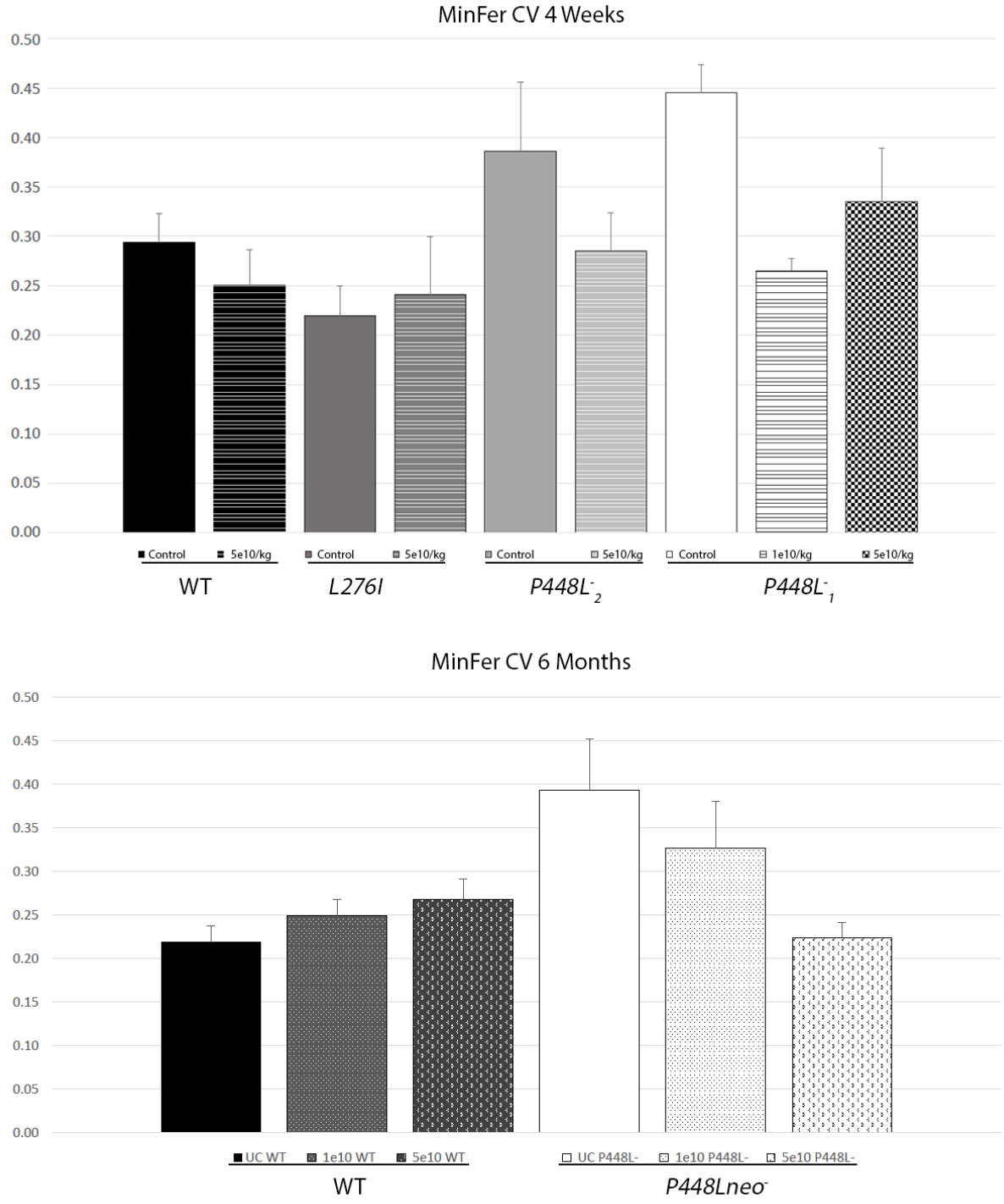
Overexpression of Mutant FKRP Associated with CMD Restores Functional Glycosylation and Improves Dystrophic Phenotype in Skeletal and Cardiac Muscle of FKRP mutant Mice



SI Figure 1. Localization of GM130 (green as a Golgi marker presenting as points within the cytoplasm and along fiber membrane) and mhFKRP (red) in *P448Lneo*$^{-}$ TA muscles 1 month after AAV9-*mhFKRP* 5e13 vg/kg treatment by confocal microscopy. No consistent co-localization of the two signals was observed. Scale Bars = 50 µm



SI Figure 2. (A) Morphometric distribution of skeletal muscle fiber diameters (μm) in treated and control *C57BL/6*, *L276I* and *FKRP* mutant mice one month after AAV9-*mhFKRP* administration. **(B)** Line representation of the same data, more clearly demonstrating shifts in distribution patterns. **(C)** Centrally nucleated fibers of the TA, quadriceps, and diaphragm of control and treated *C57BL/6*, *P448L*$^{-}$ and *L276I* mutant mice after one-month treatment with AAV9-*mhFKRP*. Error bars represent the standard error of the mean (SEM).



SI Figure 3. Coefficient of variance (CV) calculated from morphometric measures 'MinFer' (ImageJ) in *C57BL/6* (WT) and *FKRP* mutant mice one month after AAV9-*mhFKRP* treatment. Reduction in the CV, indicating a more homogenous muscle fiber composition can be observed as early as one month after AAV9-*mhFKRP* administration in all treated groups, with the exception of the WT and *L276I* groups which were relatively unchanged. At six months, both 1e13 vg/kg and 5e13 vg/kg treated *P448Lneo*$^{-}$ cohorts demonstrated reduced CV in fiber sizes with variance more closely resembling WT.