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Supplemental information

Defining the optimal dose and therapeutic window in SMA with respiratory distress type I model mice, FVB/NJ-*Ighmpb2* $^{nmd\text{-}2J}$

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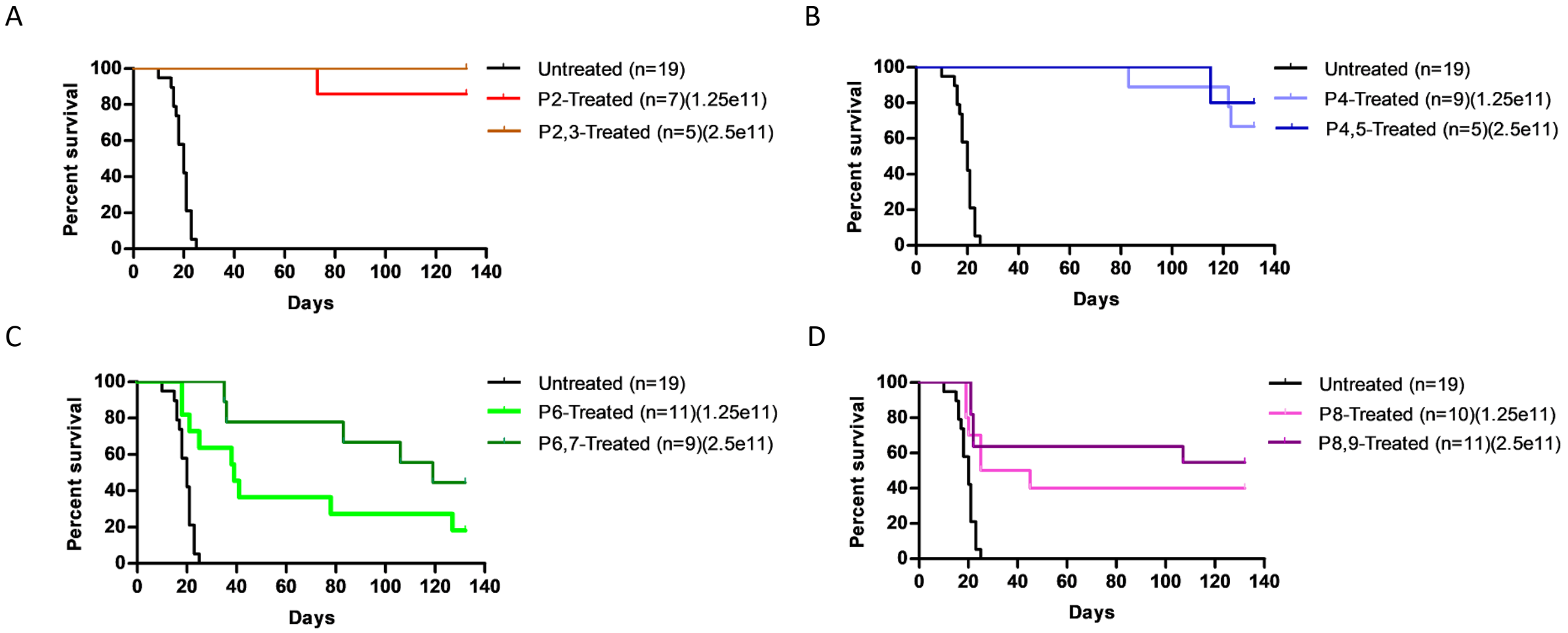

Figure S1: (**A, B, C, D**) Survival of low and high dose-treated at P2 (**A**), P4 (**B**), P6 (**C**) and P8 (**D**) was compared individually for each group. Each treated cohort lived significantly longer than the untreated animals, however, a higher dose did not result in a statistically significant increase in the survival of any treated group.

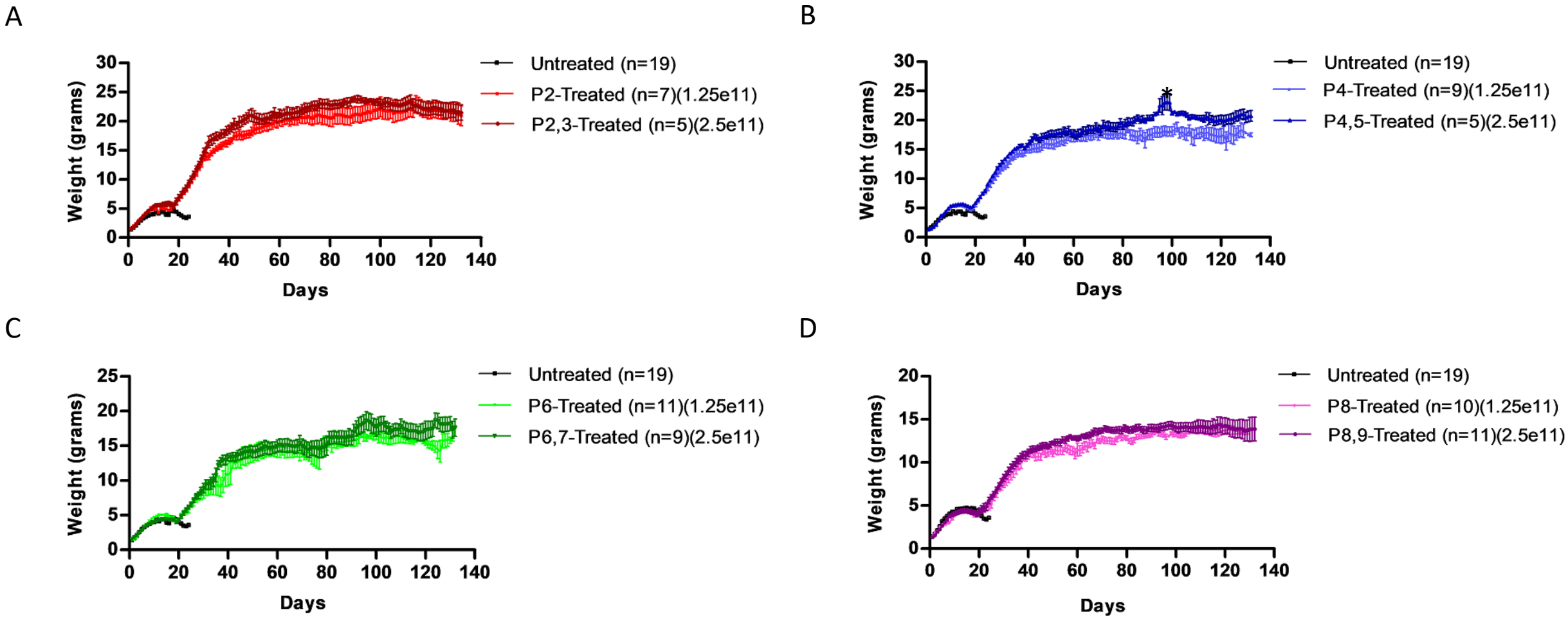

Figure S2: (**A, B, C, D**) The impact of low and high dose on weight gain at P2 (**A**), P4 (**B**), P6 (**C**) and P8 (**D**) treatment compared individually for each group. The increase in dose did not statistically affect the weight in any group except the P4-treated in which the high-dose treated weighed heavier compared to low dose-treated during 85-115 days of age (one-way ANOVA, P=0.04). Every treated group weighed significantly less than the "HET" littermates (one-way ANOVA, P<0.001).

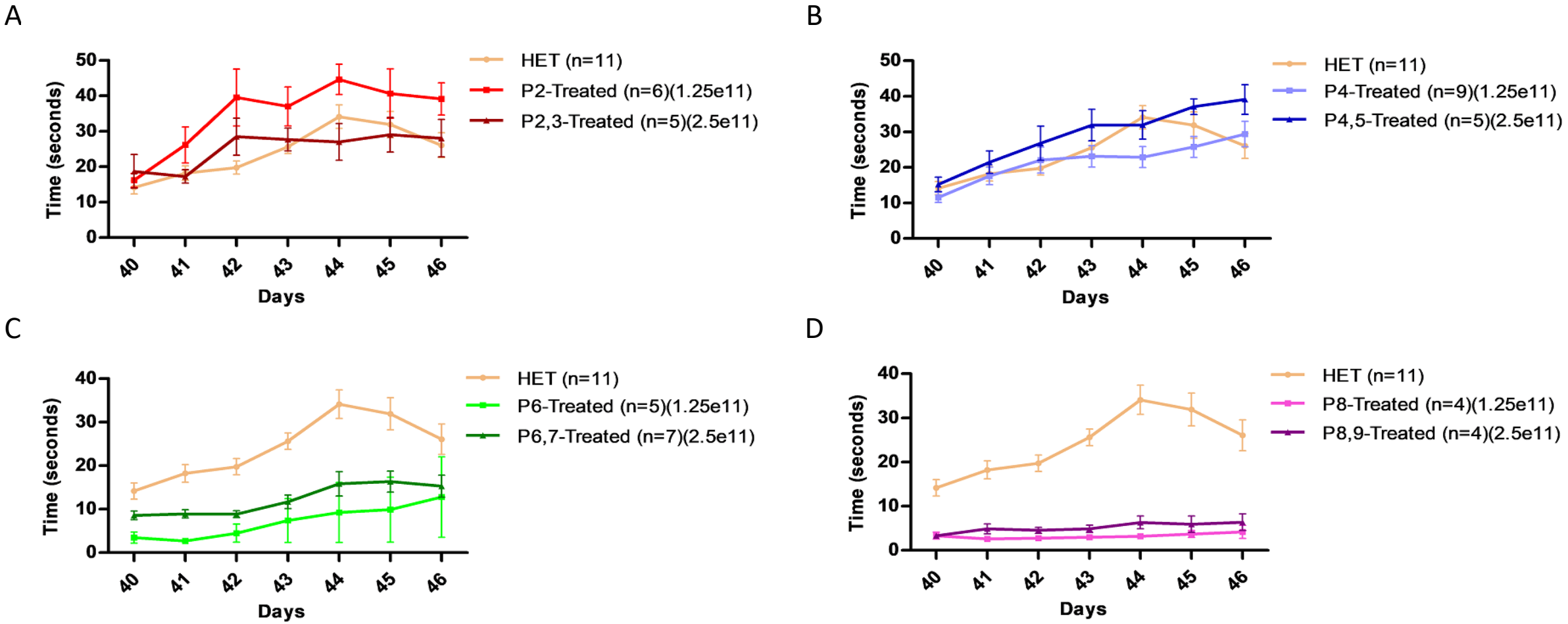

Figure S3: (**A, B, C, D**) The impact of low and high dose on rotarod performance at P2 (**C**), P4 (**D**), P6 (**E**) and P8 (**F**) treatment compared individually for each group. The increase in dose did not statistically affect the rotarod performance in any of the treated groups. P6 and P8-treated animals were drastically weaker than "HET" in either low or high dose group ($P<0.001$). Measurements were taken for 7 consecutive days starting from P40 through P46. Error bars represent ± SEM.

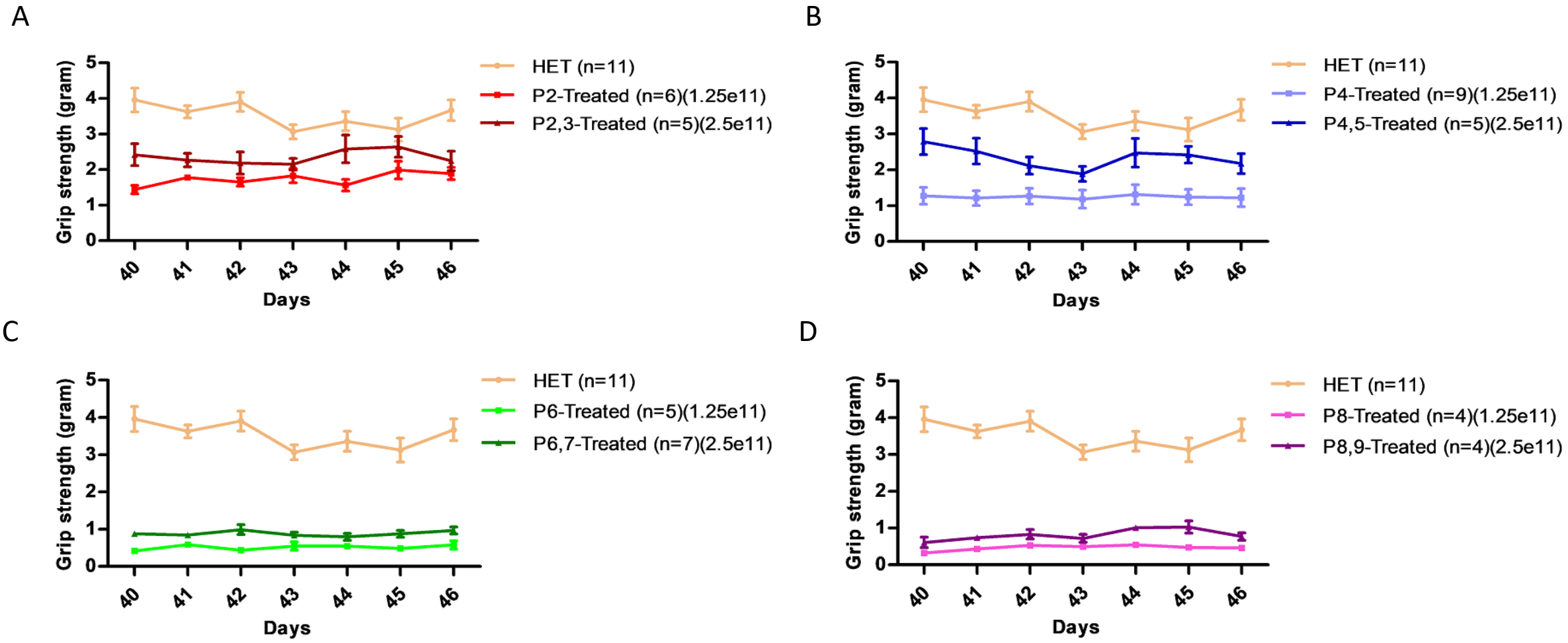

Figure S4: (A, B, C, D) The effect of low and high dose on grip strength at P2 (**A**), P4 (**B**), P6 (**C**) and P8 (**D**) treatment compared individually for each group. High dose significantly impacted the grip strength of treated groups at each time point (one-way ANOVA, P2,3 vs. P2 $P<0.001$; P4,5 vs. P4 $P<0.001$; P6,7 vs. P6 $P<0.01$; P8,9 vs. P8 $P<0.05$). Measurements were taken for 7 consecutive days starting from P40 through P46. Error bars represent $\pm$ SEM.

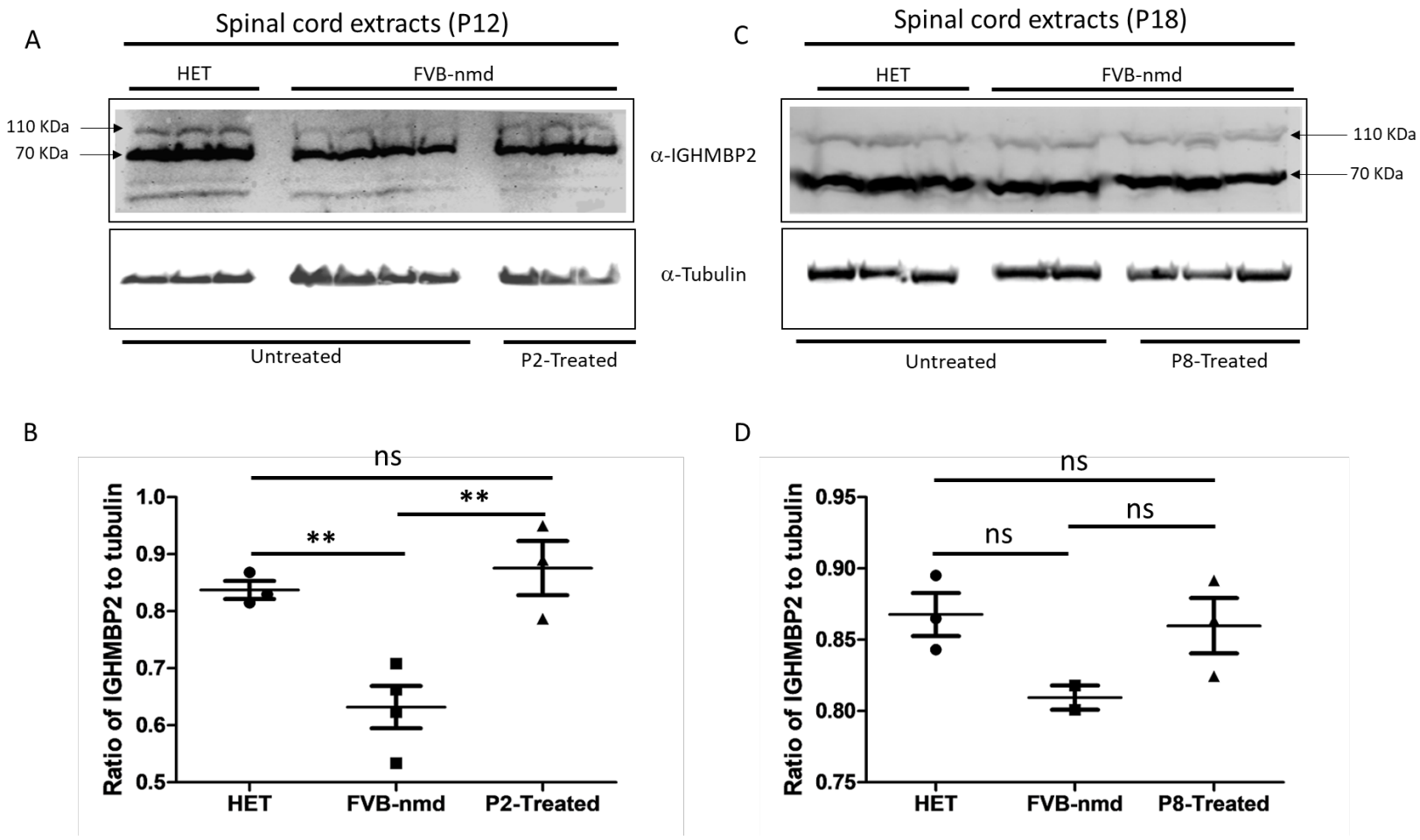

Figure S5: (**A, B, C, D**) IGHMBP2 protein expression in spinal cord extract from P2 and P8 cohorts. Spinal cord extract was generated from heterozygous ("HET") animals, or FVB-nmd mice that were untreated or treated (P2 or P8) with ssAAV9-IGHMBP2. Tissues were harvested 10 days after treatment: P2-treated collected on P12 (**A,B**); P8-treated collected on P18 (**C,D**). Tubulin was used as an internal loading control and used in the graphical depiction of the quantification of the relative band intensity (**B,D**).