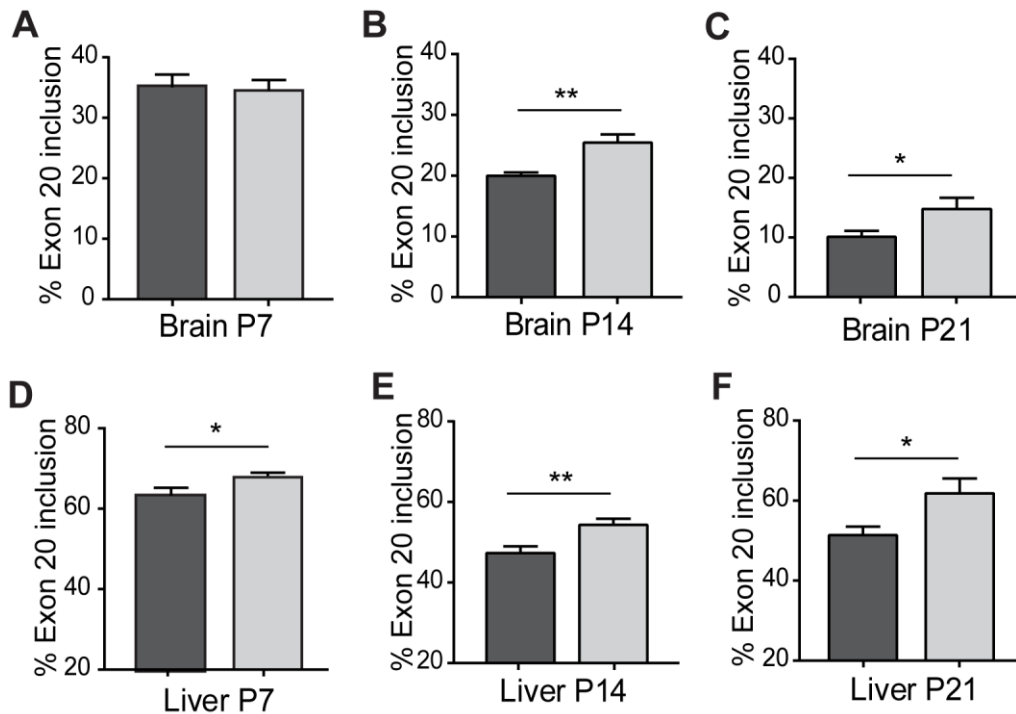


**Supplemental Data**

***ELP1* Splicing Correction Reverses**

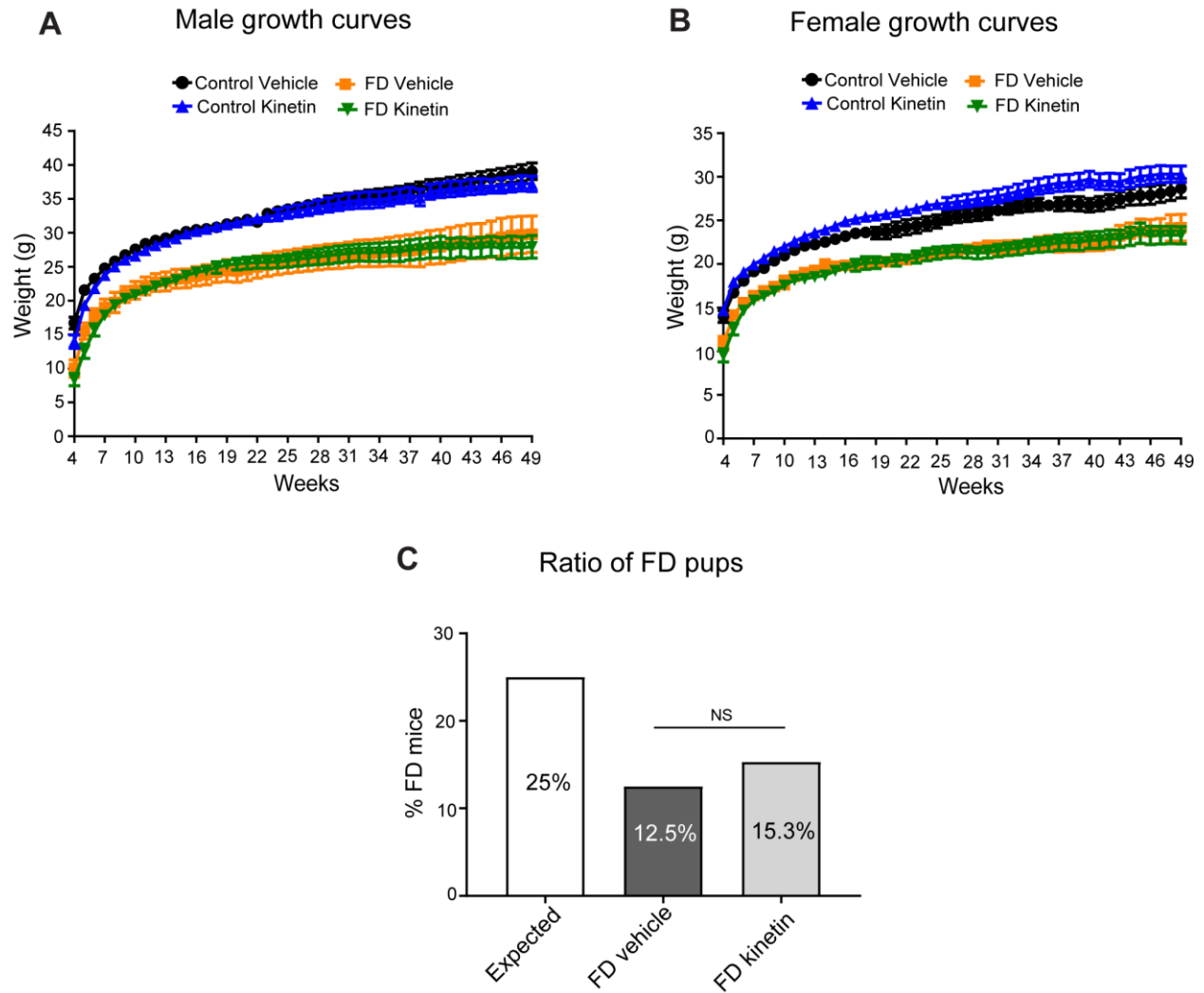
**Proprioceptive Sensory Loss in Familial Dysautonomia**

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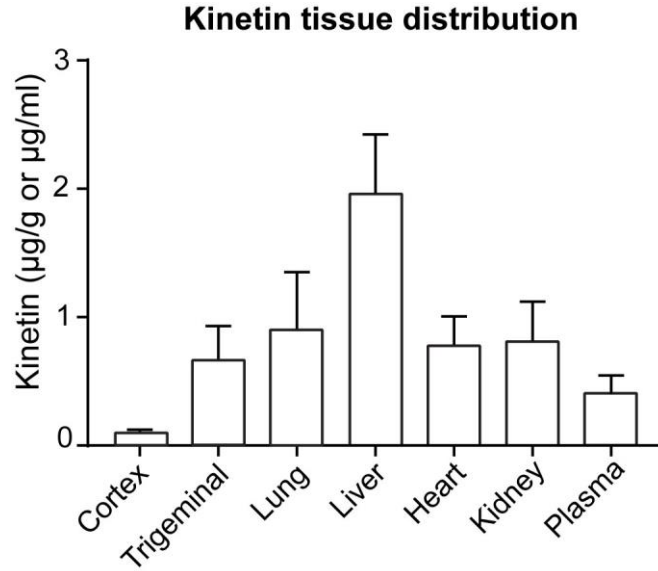
**Figure S1. Kinetin treatment improves *ELPI* splicing in nursing pups.** At the day of delivery the dams were randomly assigned to vehicle diet or kinetin diet, and, continued to be fed these diets until the time of weaning. *ELPI* splicing was analyzed in nursing pups carrying the human transgene with the major FD splicing mutation, *TgFD9*. (A-F) Percent of exon 20 inclusion in vehicle-assigned (dark grey) and kinetin-assigned (light grey) *TgFD9* pups. (A) In brain at P7 no significant differences were detected in percent of exon 20 inclusion ( $P = 0.73$ ) between vehicle-assigned ( $n=5$ ) and kinetin-assigned ( $n=7$ ) *TgFD9* pups, two-tailed unpaired Student's *t*-test. (B) In brain at P14 a significant difference of  $**P < 0.01$  was detected in percent of exon 20 inclusion between vehicle-assigned ( $n=5$ ) and kinetin-assigned ( $n=8$ ) *TgFD9* pups, two-tailed unpaired Student's *t*-test. (C) In brain at P21, weaning time, a significant difference of  $*P < 0.05$  was detected in percent of exon 20 inclusion between vehicle-assigned ( $n=5$ ) and kinetin-assigned ( $n=6$ ) *TgFD9* pups, two-tailed unpaired Student's *t*-test. (D) In liver at P7 a significant

difference of  $*P < 0.05$  was detected in percent of exon 20 inclusion between vehicle-assigned (n=5) and kinetin-assigned (n= 7) *TgFD9* pups, two-tailed unpaired Student's *t*-test. (E) In liver at P14 a significant difference of  $**P < 0.01$  was detected in percent of exon 20 inclusion between vehicle-assigned (n=5) and kinetin-assigned (n= 8) *TgFD9* pups, two-tailed unpaired Student's *t*-test. (F) In liver at P21, weaning time, a significant difference of  $*P < 0.05$  was detected in percent of exon 20 inclusion between vehicle-assigned (n=5) and kinetin-assigned (n= 6) *TgFD9* pups, two-tailed unpaired Student's *t*-test. Means and s.e.m. are shown.



**Figure S2. Kinetic treatment did not affect growth or ratio of the FD mice.** (A) Postnatal growth curves for vehicle-treated (n=12, black), kinetin-treated (n=16, blue) control male mice and vehicle-treated (n=7, orange), kinetin-treated (n=11, green) FD male mice. (B) Postnatal growth curves for vehicle-treated (n=13, black), kinetin-treated (n=18, blue) control female mice and vehicle-treated (n=12, orange), kinetin-treated (n=15, green) FD female mice. Means and s.e.m. are shown. (C) The expected Mendelian ratio of *TgFD9; Ikbkap<sup>Δ20/flox</sup>* mice obtained by crossing *TgFD9; Ikbkap<sup>flox/flox</sup>* mice × *Ikbkap<sup>Δ20/+</sup>* mice is 25% (white bar). The observed ratio was 12.5% (38/305) for the vehicle-treated *TgFD9; Ikbkap<sup>Δ20/flox</sup>* mice (dark grey bar) and 15.3% (52/340) for the kinetin-treated *TgFD9; Ikbkap<sup>Δ20/flox</sup>* mice (light grey bar). No significance

differences were observed in the actual ratio between vehicle-treated and kinetin-treated *TgFD9*; *Ikkap<sup>Δ20/flox</sup>* mice, (P=0.1539)  $\chi^2$ -Test.



**Figure S3. Kinetin distribution in different tissues.** The levels of compound were measured in cortex, trigeminal ganglia, lung, liver, heart, kidney and plasma from kinetin-treated FD mice (n = 13) using mass spectrometry. Means and s.e.m. are shown.

**Table S1. Fibroblast cell lines from FD patients used in the RNAseq experiment to assess kinetin specificity.**

<b>Coriell #</b>	<b>Genotype</b>	<b>Sex</b>	<b>Age</b>	<b>Race</b>
0850	Homozygous for <i>ELP1</i> T>C splice mutation	Male	26	White
2341	Homozygous for <i>ELP1</i> T>C splice mutation	Male	17	White
4589	Homozygous for <i>ELP1</i> T>C splice mutation	Male	16	White
2343	Homozygous for <i>ELP1</i> T>C splice mutation	Female	24	White
4663	Homozygous for <i>ELP1</i> T>C splice mutation	Female	2	White
4899	Homozygous for <i>ELP1</i> T>C splice mutation	Female	12	White