Supplementary Information for:

CRISPR/Cas9 mediated generation of an ovine model for infantile neuronal ceroid lipofuscinosis (CLN1 disease)

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Supplementary Figure 1: Sequence of the HDR repair template provided as an ssODN
The red letters indicate sequence changes introduced into the WT sequence. The TAA in both ssODNs introduces a coding change, the R151* mutation described in patients. The G in ssODN2 is a silent blocking mutation required to prevent the re-cutting of the repaired allele by sgRNA 2.
Supplementary Figure 2: PPT1 sheep show exhibit clinical signs similar to patients with CLN1 disease at humanely defined endpoint

A. Photograph demonstrating assessment of the menace response. B. Scatter plot demonstrates a significant loss in the menace response of the homozygote PPT1 sheep (P<0.0001). C. Picture demonstrates hoof placement test. D. Scatter plot demonstrates a significant increase in loss of hoof placement in homozygote sheep (P=0.0011). E. Photograph demonstrates knuckling test in rear limbs. F. Scatter plot demonstrates a significant loss of conscious proprioception in the homozygote sheep as indicated using the knuckling test on the hind limbs (P=0.0226). Statistical analyses utilized unpaired two-tailed Student’s t-test. Error bars represent SEM.