**Supplemental Table 2.** Genotype, sample size, and gender of mice used in study

|  |  |  |
| --- | --- | --- |
| Study group | Age at sacrifice | Sample size |
| *Npc1m1N/m1N* saline | 9 weeks / End stage disease | 4M, 1F / 2M, 6F |
| *Npc1m1N/m1N* AAV9-*NPC1* | 9 weeks / End stage disease | 3M, 4F / 5M, 4F |
| *Npc1m1N/m1N* AAV-PHP.B-*NPC1* | 9 weeks / End stage disease | 2M, 2F / 4M, 5F |
| *Npc1+/+* AAV-PHP.B-*NPC1* | 9 weeks | 3M, 4F |
| *Npc1+/+* no treatment | 9 weeks / End of study | 2M, 3F / 3M, 5F |
| *Npc1m1N/m1N* AAV9-GFP | 9 weeks | 2M, 3F |
| *Npc1m1N/m1N* AAV-PHP.B-GFP | 9 weeks | 2M, 2F |
| *Npc1+/+* AAV9-GFP | 9 weeks | 2M, 3F |
| *Npc1+/+* AAV-PHP.B-GFP | 9 weeks | 2M, 4F |