

OMTM, Volume 18

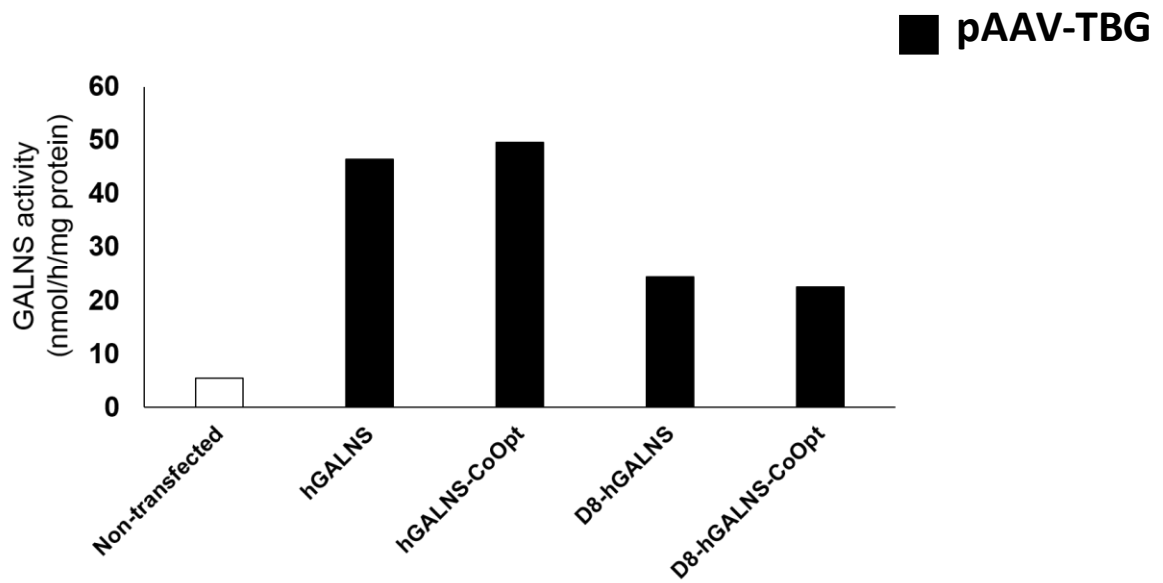
Supplemental Information

**Liver-Targeted AAV8 Gene Therapy Ameliorates
Skeletal and Cardiovascular Pathology
in a Mucopolysaccharidosis IVA Murine Model**

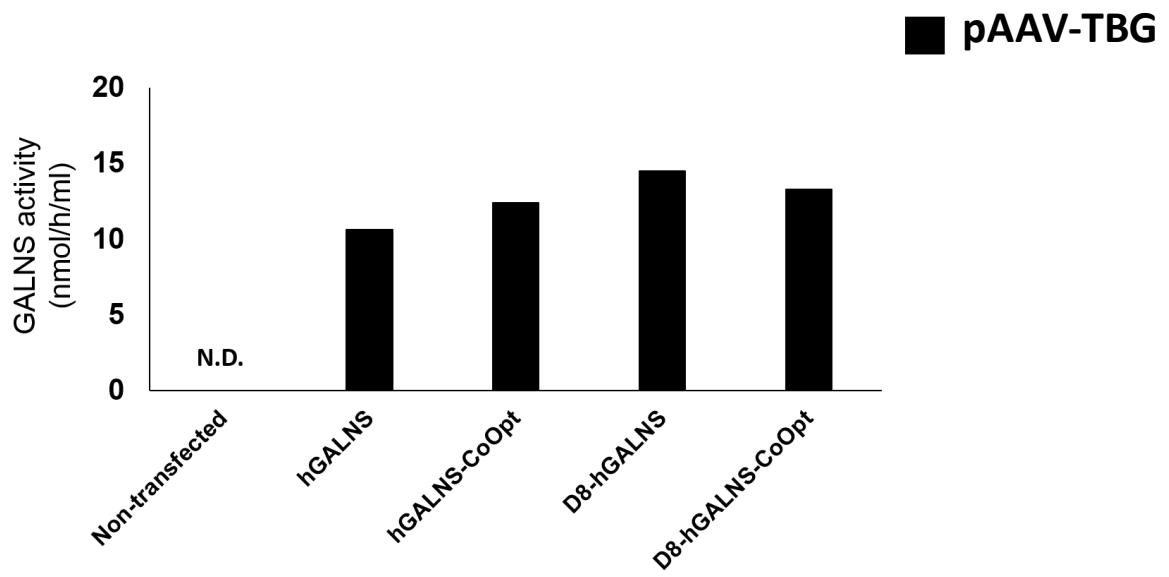
Kazuki Sawamoto, Subha Karumuthil-Melethil, Shaukat Khan, Molly Stapleton, Joseph T. Bruder, Olivier Danos, and Shunji Tomatsu

Supplemental Figure 1.

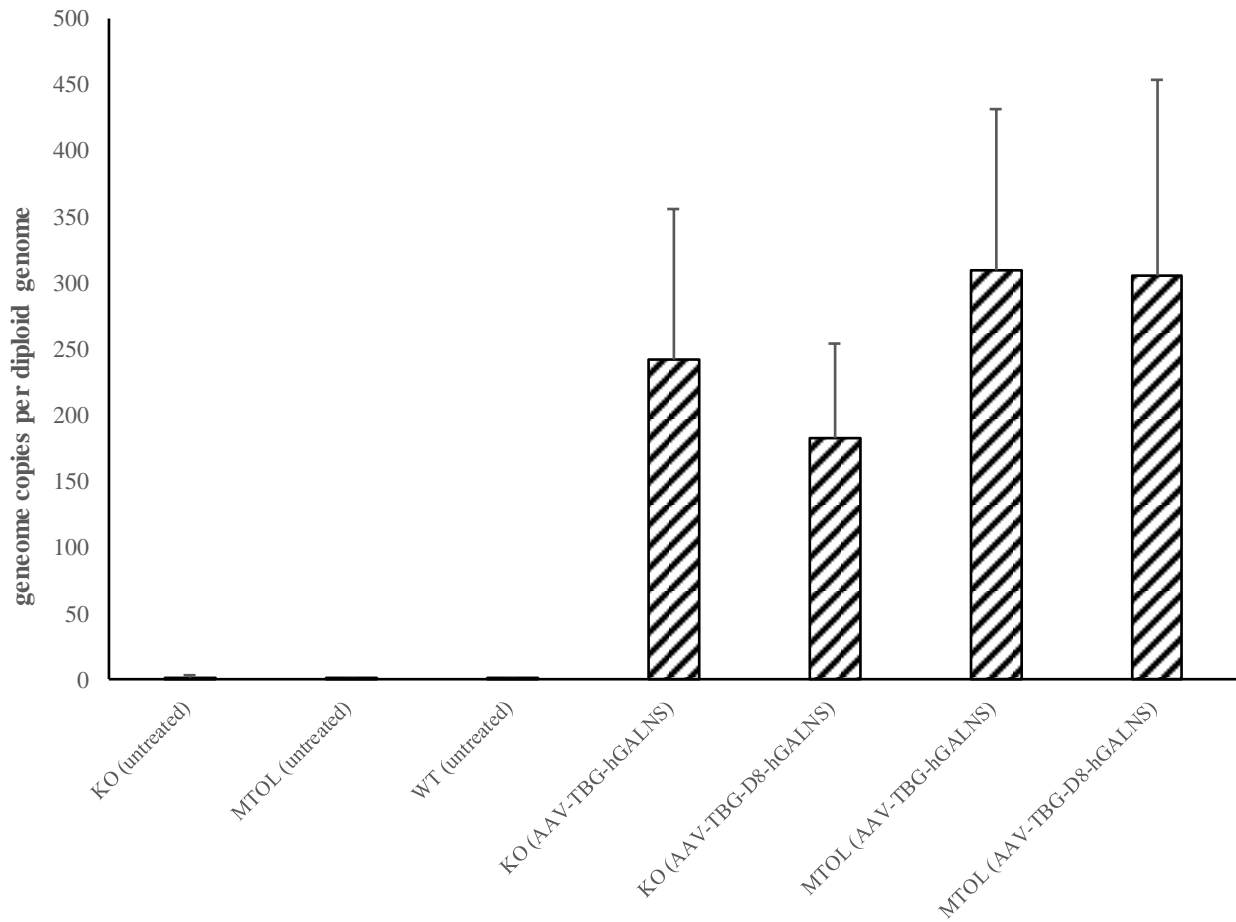
A



B

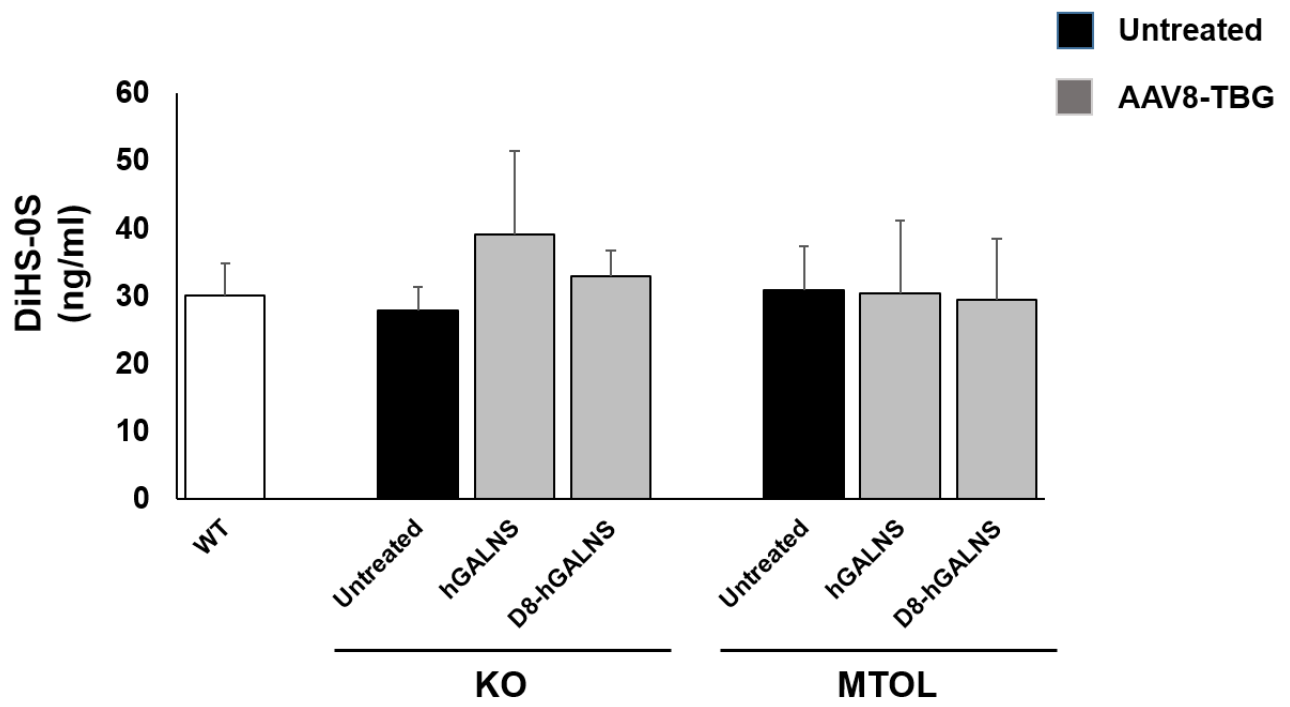


Supplemental Figure 2.

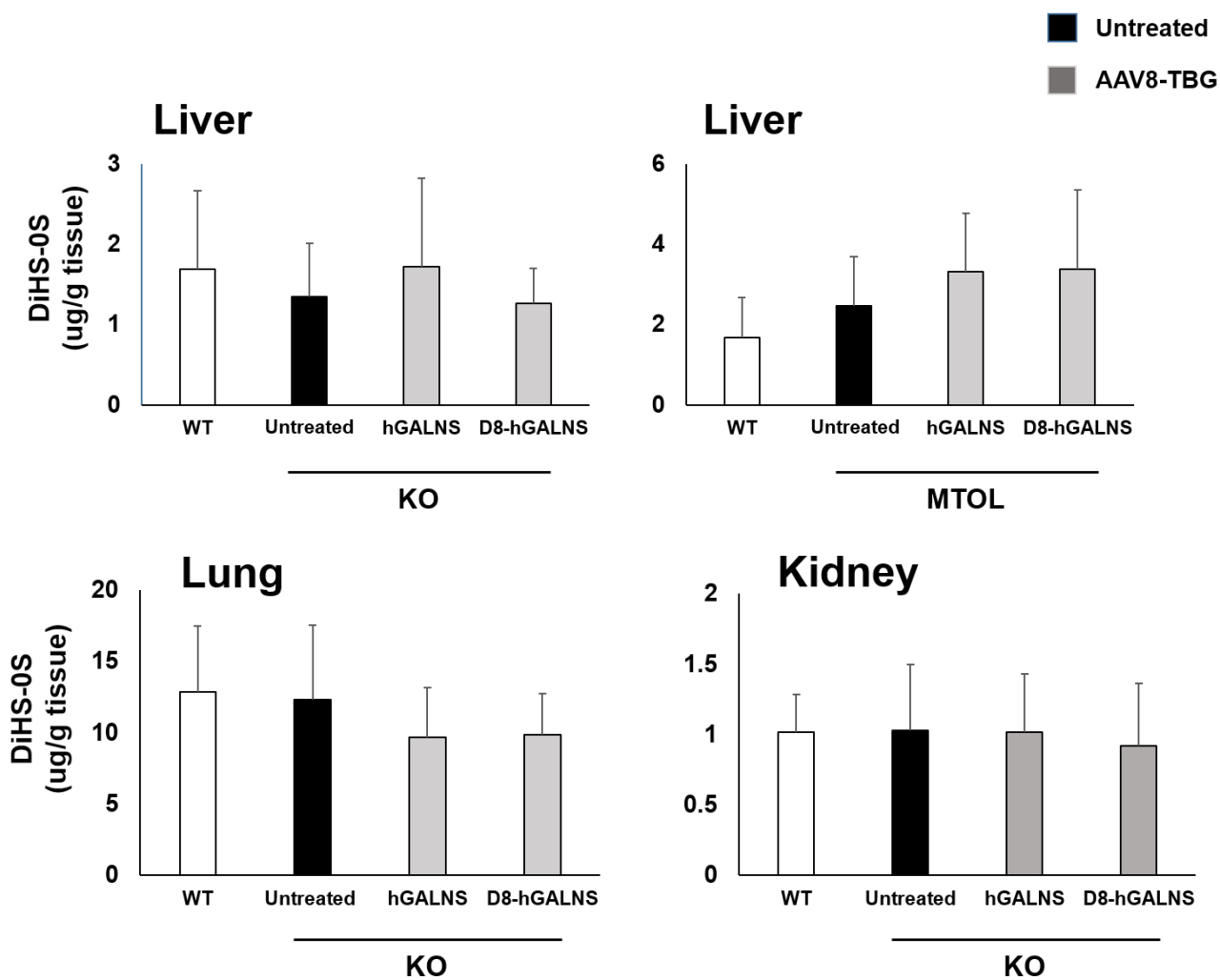


| Group | AAV vector genome copies per diploid genome |
|---------------------------------|--|
| KO (untreated) | 1.57 ± 1.21 (n = 6) |
| MTOL (untreated) | 0.083 ± 0.12 (n = 4) |
| WT (untreated) | 0.063 ± 0.11 (n = 6) |
| KO (AAV-TBG-hGALNS) | 242.23 ± 114.08 (n = 7) |
| KO (AAV-TBG-D8-hGALNS) | 182.36 ± 71.85 (n = 6) |
| MTOL (AAV-TBG-hGALNS) | 309.51 ± 112.14 (n = 5) |
| MTOL (AAV-TBG-D8-hGALNS) | 305.36 ± 147.82 (n = 4) |

Supplemental Figure 3.

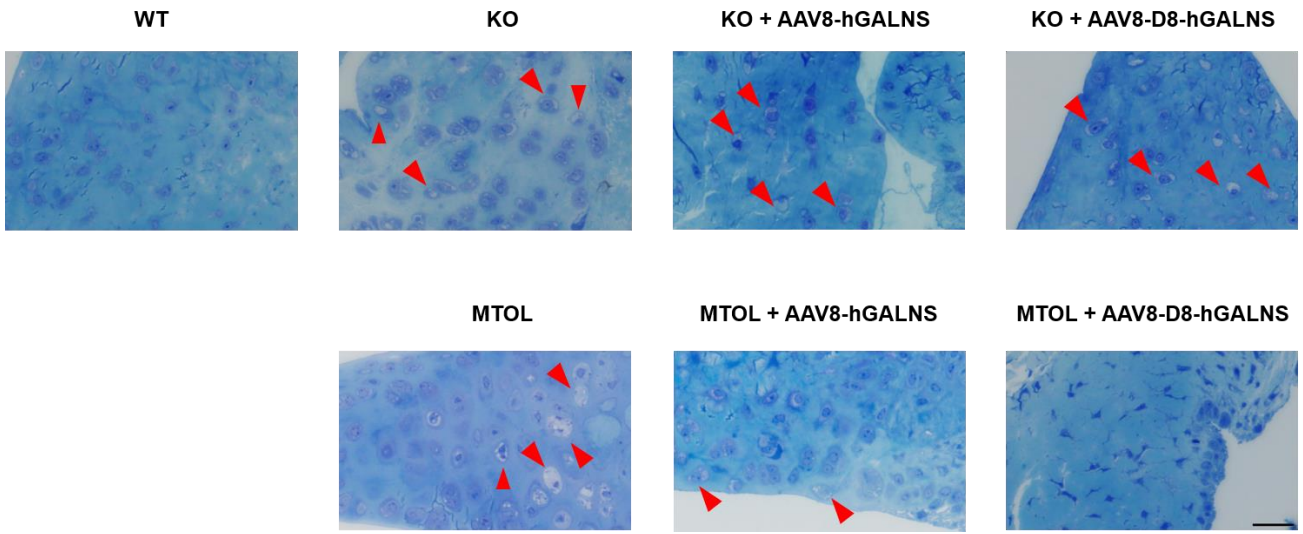


Supplemental Figure 4.

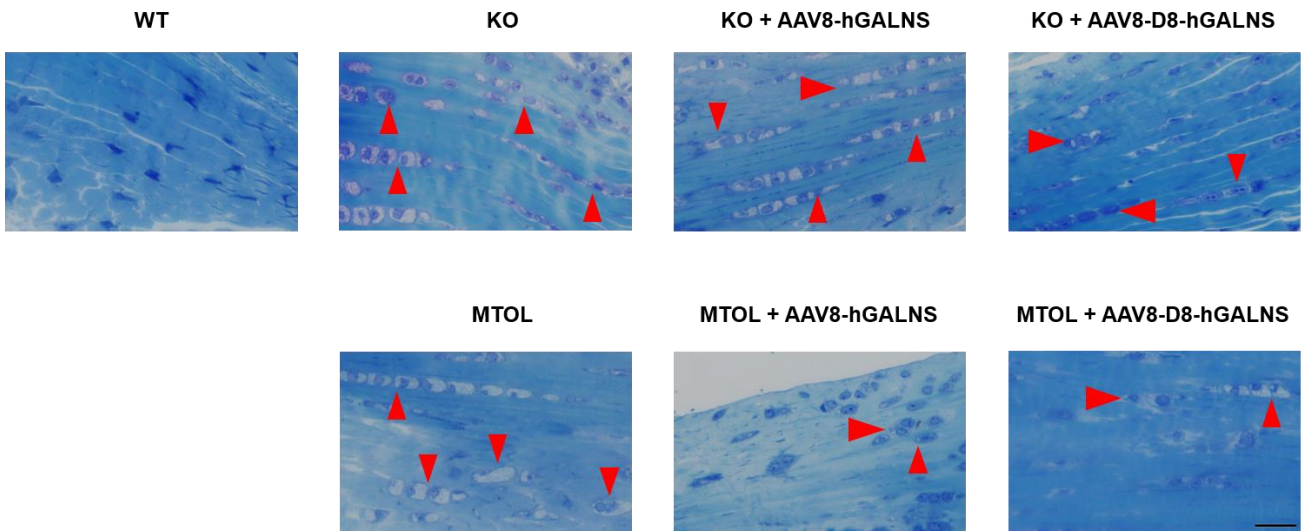


Supplemental Figure 5.

A



B



Supplemental Figure 6.

