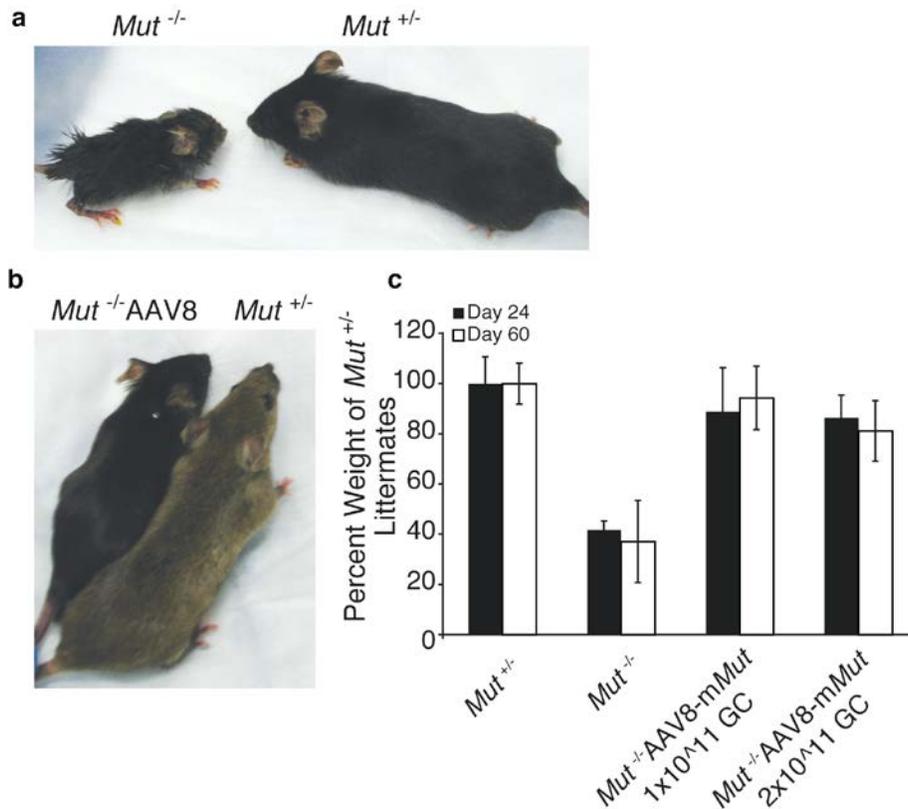


Growth and Phenotypic Correction after AAV8-mMut Gene Therapy



Growth and phenotypic correction after AAV8-mMut gene therapy, adapted from Chandler and Venditti (2010) Mol Ther 18: 11-16. (a) An untreated *Mut*^{-/-} mouse (left) compared to a *Mut*^{+/-} littermate at day of life 55 to illustrate the relative difference in size and appearance. The *Mut*^{-/-} mouse is severely growth retarded and has achieved only 40 percent of the *Mut*^{+/-} littermate weight. Shortly after this photo was taken the *Mut*^{-/-} mouse became lethargic and perished. (b) A *Mut*^{-/-} mouse (left, neck flexed) that received a single intrahepatic injection of AAV8-CBA-mMut 2x10¹¹ GC aside a *Mut*^{+/-} littermate (right, neck extended) at day of life 120. The treated *Mut*^{-/-} animal is similar in size to a control *Mut*^{+/-} littermate. (c) Growth correction between untreated *Mut*^{-/-} mice, intrahepatic AAV8-mMut treated *Mut*^{-/-} mice and untreated age, diet and gender matched *Mut*^{+/-} littermates. The graph depicts the percent weight at day of life 24 and 60 of *Mut*^{+/-} diet and gender matched littermates (n=25, 25) compared to untreated *Mut*^{-/-} mice (n=6,3) or *Mut*^{-/-} mice treated via an intrahepatic injection of AAV8-mMut 1x10¹¹ GC (n=10,10) or AAV8-mMut 2x10¹¹ GC (n=17,17) at birth. The AAV8-mMut treated *Mut*^{-/-} mice at both doses showed significant growth improvement compared to the untreated *Mut*^{-/-} mice (* $p < 10^{-6}$ for day 24, ** $p < 0.01$ for day 60). Error bars represent plus and minus one standard deviation.