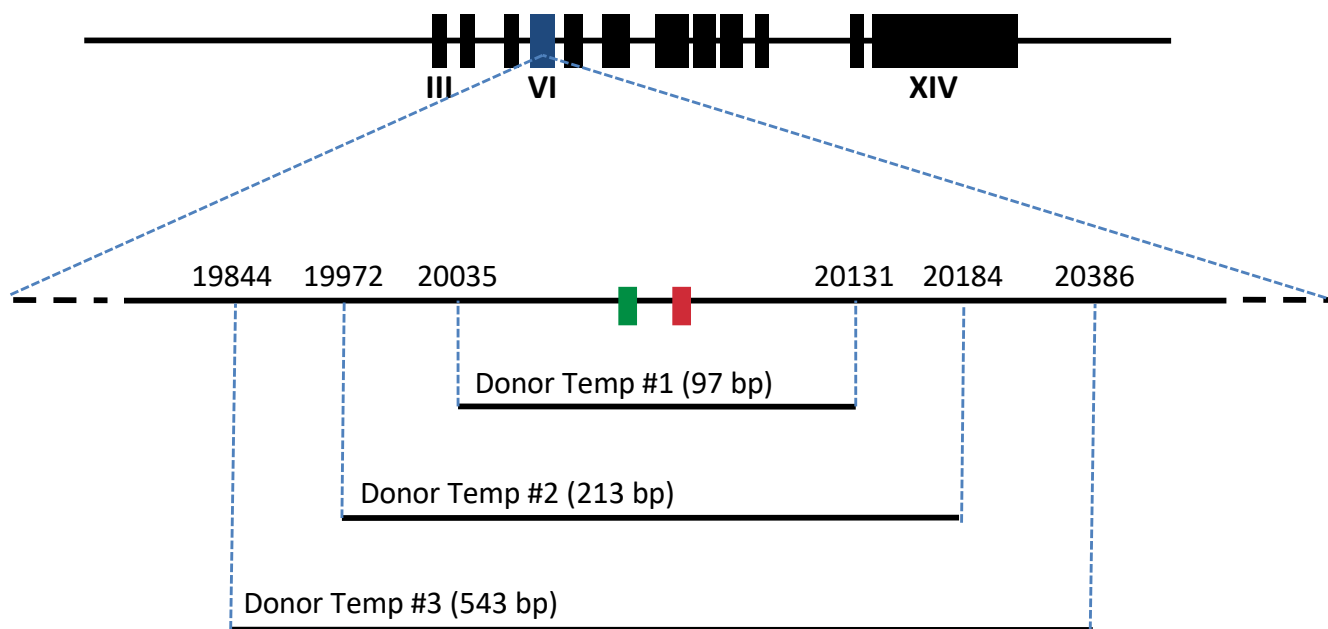


Supplementary Figure 1

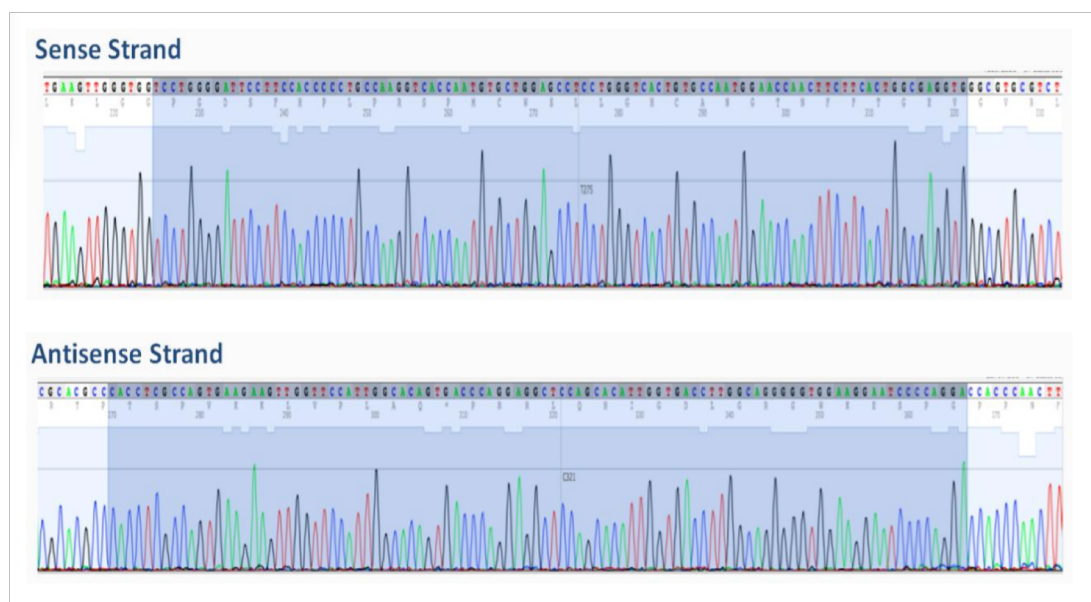
A

Mus musculus strain C57BL/6J chromosome 5
Idua gene (Ch5. 108,660,331~108,685,446)



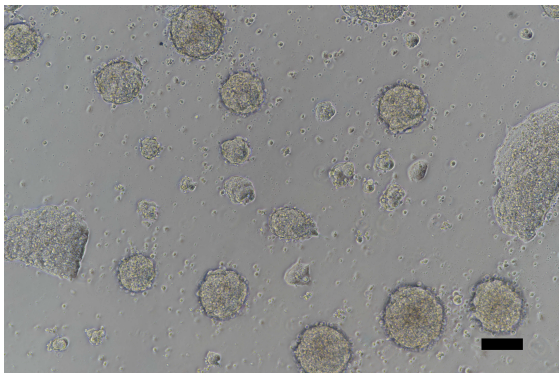
B

Post gene editing validation sequencing results

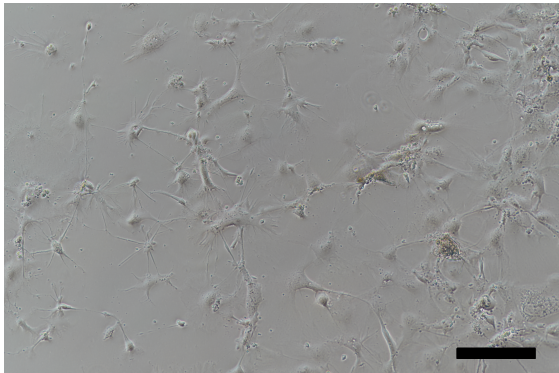


Supplementary Figure 2

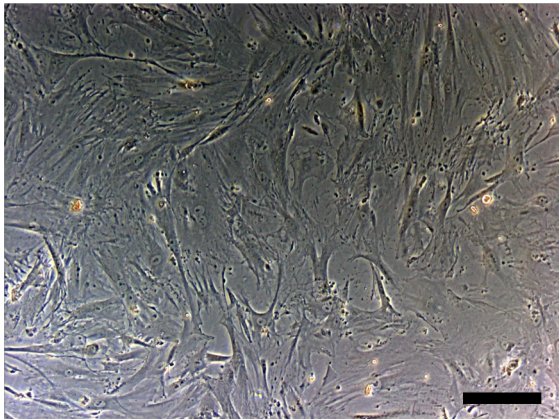
A



B



C



Supplementary Figure Legend

Figure S1. (A) Schematic representation of donor template designing strategy. (B) Computer-generated sequencing electropherogram of *Idua* gene corrected iPSC.

Figure S2.

Phase contrast images of gene-corrected miPSCs; (A) before inducing differentiations. (B) 21 days after spontaneous differentiation induction. (C) 21 days after fibroblast differentiation induction. Scale bars: 100 μ m

Supplementary Information

1. Target sequences for Guide RNAs:

- 1) ATTCCTTCCACCCCCTGCCAAGG (20043-20065)
- 2) CTCCTGGGTCACTGTGCCAATGG (20084-20106)
- 3) AGCCCCACTTTGAAGTTGGGTGG (20012-20034)
- 4) TGGCGAGGTGGGCGTGCGTCTGG (20122-2144)
- 5) GCGTTTTTCCAGACCCTGTTGGG (19884-19906)
- 6) TACCCCTATTTACAATGACGAGG (20324-20346)

2. Donor Templates:

Template #1: 97 bp

5'-TCCTGGGGATTCTTCCACCCCCTGCCAAG^ATCACCAATGTGCTGGAGC
CTCCTGGGTCACTGTGCCAATGGAACCAACTTCTTCACTGGCGAGGTG-3' (20035-20131)

Template #2 213 bp

5'-GAATTACTATGATGCCTGCTCTGAGGGGCTGCGCATTGCCAGCCCCACTTTGAAGTT
GGGTGGTCCTGGGGATTCTTCCACCCCCTGCCAAGGTCACCAATGTGCTGGAGCCT
CCTGGGTCACTGTGCCAATGGAACCAACTTCTTCACTGGCGAGGTGGGCGTGCGTCT
GGATTACATCTCCCTGCACAAGAAGGTGTGTGGTGCTCCAC-3' (19972-20184)

Template #3: 543 bp

5'-ATGACTTTGACAACGTGTCCATGACCACACAAGGTAGGCAGCGTTTTTCCAGACCCT
GTTGGGTGGGCTGTATGAGAGGGAAGCAGGACAATCCCAGACCCTCAAGACCCTCC
TGCCCTAGGCTTCCTGAATTACTATGATGCCTGCTCTGAGGGGCTGCGCATTGCCAG
CCCCACTTTGAAGTTGGGTGGTCCTGGGGATTCTTCCACCCCCTGCCAAGGTCACC
AATGTGCTGGAGCCTCCTGGGTCACTGTGCCAATGGAACCAACTTCTTCACTGGCGA
GGTGGGCGTGCGTCTGGATTACATCTCCCTGCACAAGAAGGTGTGTGGTGCTCCAC
CCCTCCCTGCCTCCTTTGAGCTGAGCCTGTACTGAGTGAGGTATGTGCTGGCTCAGG
GTGCAGGTAGCTCCATCGCCATCCTGGAGCAGGAGATGGCAGTTGTGGAGCAGGTC
CAGCAGCTCTTCCCTGAGTTCAAGGATACCCCTATTTACAATGACGAGGCAGACCCT
CTGGTGGGCTGGTCCCTGCCACAACCTTGGAG-3' (19844-20386)

3. Validation qPCR primers:

Neo^r primers:

Forward 5'-ATGGCTTTCTTGCCGCCA-3'

Reverse 5'-GAACCTGCGTGCAATCCATC-3'

IDUA primers:

Forward 5'-AGGTCACCAATGTGCTGGA-3'

Reverse 5'-CCTTCTTGTGCAGGGAGATG-3'

Sequencing primers:

Forward 5'-TAGGTATGGGCTGACACACG-3'

Reverse 5'-AAGGACCCACCTTCACCAC-3'

4. Mouse Idua gene target sequence:

TGCGCATTGCCAGCCCCACTTTGAAGTTGGGTGGTCCTGGGGATTCCTTCCACCCCCTGC
CAAGGTCACCAATGTGCTGGAGCCTCCTGGGTCACTGTGCCAATGGAACCAACTTCTTCAC
TGGCGAGGTGGGCGTGCGTCTGGATTACA

Yellow and green highlighted sequences are targets of gRNA#1 and #2, respectively. The underlined sequence is the donor template sequence #1. The “G” in red font is in the native mouse Idua gene sequence.