

# Neul Cas9-KO Strategy

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## **Project Overview**



Project Name Neu1

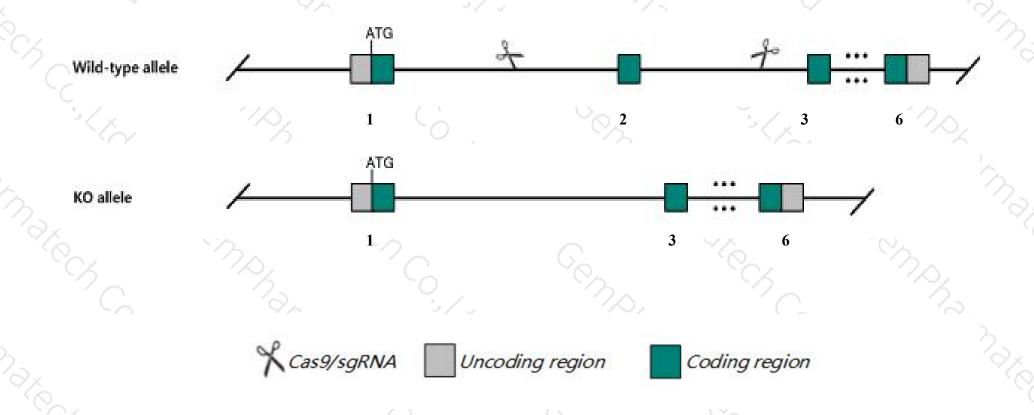
Project type Cas9-KO

Strain background C57BL/6J

## **Knockout strategy**



This model will use CRISPR/Cas9 technology to edit the Neul gene. The schematic diagram is as follows:



## **Technical routes**



- ➤ The *Neu1* gene has 3 transcripts. According to the structure of *Neu1* gene, exon2 of *Neu1-201*(ENSMUST0000007253.5) transcript is recommended as the knockout region. The region contains 193bp coding sequence.

  Knock out the region will result in disruption of protein function.
- ➤ In this project we use CRISPR/Cas9 technology to modify *Neu1* gene. The brief process is as follows: sgRNA was transcribed in vitro.Cas9 and sgRNA were microinjected into the fertilized eggs of C57BL/6J mice. Fertilized eggs were transplanted to obtain positive F0 mice which were confirmed by PCR and sequencing. A stable F1 generation mouse model was obtained by mating positive F0 generation mice with C57BL/6J mice.

### **Notice**



- ➤ According to the existing MGI data, Nullizygous mice develop features of early-onset lysosomal storage disease (sialidosis), including severe nephropathy, edema, splenomegaly, kyphosis and oligosacchariduria, and display myoclonus, lordosis, extramedullary hematopoiesis, dyspnea, weight loss, gait defects, tremors and premature death.
- ➤ The *Neu1* gene is located on the Chr17. If the knockout mice are crossed with other mice strains to obtain double gene positive homozygous mouse offspring, please avoid the two genes on the same chromosome.
- ➤ This Strategy is designed based on genetic information in existing databases. Due to the complexity of biological processes, all risk of the gene knockout on gene transcription, RNA splicing and protein translation cannot be predicted at the existing technology level.

## Gene information (NCBI)



#### Neu1 neuraminidase 1 [Mus musculus (house mouse)]

Gene ID: 18010, updated on 9-Apr-2019

#### Summary

☆ ?

Official Symbol Neu1 provided by MGI

Official Full Name neuraminidase 1 provided by MGI

Primary source MGI:MGI:97305

See related Ensembl:ENSMUSG00000007038

Gene type protein coding
RefSeq status VALIDATED
Organism Mus musculus

Lineage Eukaryota; Metazoa; Chordata; Craniata; Vertebrata; Euteleostomi; Mammalia; Eutheria; Euarchontoglires; Glires; Rodentia; Myomorpha;

Muroidea; Muridae; Murinae; Mus; Mus

Also known as AA407268, AA407316, Aglp, Apl, Bat-7, Bat7, G9, Map-2, Neu, Neu-1

Expression Broad expression in kidney adult (RPKM 114.3), colon adult (RPKM 62.8) and 20 other tissuesSee more

Orthologs <u>human all</u>

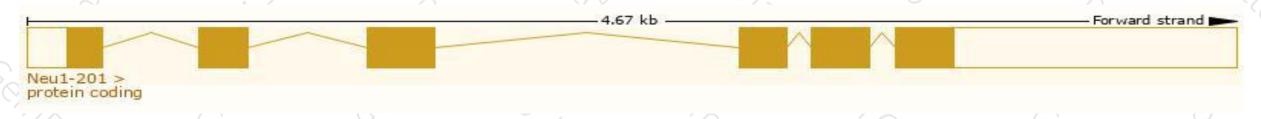
## Transcript information (Ensembl)



The gene has 3 transcripts, all transcripts are shown below:

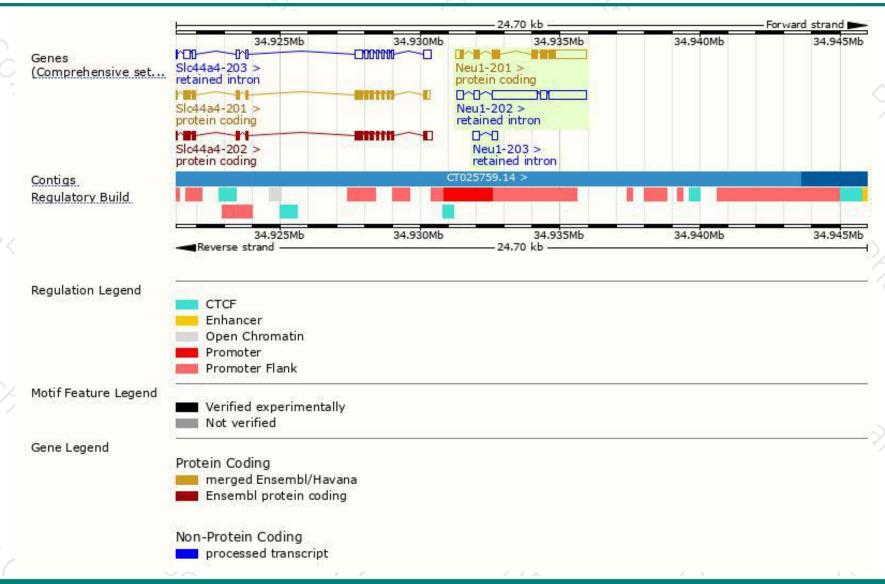
Name	Transcript ID	bp	Protein	Biotype	CCDS	UniProt	Flags
Neu1-201	ENSMUST00000007253.5	2474	409aa	Protein coding	CCDS28668	O35657 Q3UL64	TSL:1 GENCODE basic APPRIS P1
Neu1-202	ENSMUST00000173269.1	3619	No protein	Retained intron	2-	5	TSL:1
Neu1-203	ENSMUST00000174715.1	446	No protein	Retained intron	-	2	TSL:2

The strategy is based on the design of Neu1-201 transcript, The transcription is shown below



### Genomic location distribution





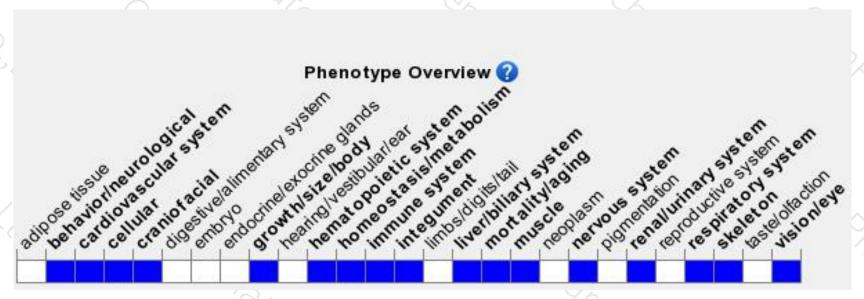
### Protein domain





## Mouse phenotype description(MGI)





Phenotypes affected by the gene are marked in blue.Data quoted from MGI database(http://www.informatics.jax.org/).

According to the existing MGI data, Nullizygous mice develop features of early-onset lysosomal storage disease (sialidosis), including severe nephropathy, edema, splenomegaly, kyphosis and oligosacchariduria, and display myoclonus, lordosis, extramedullary hematopoiesis, dyspnea, weight loss, gait defects, tremors and premature death.



If you have any questions, you are welcome to inquire.

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