

Idua Cas9-KO Strategy

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Project Overview

Project Name

Idua

Project type

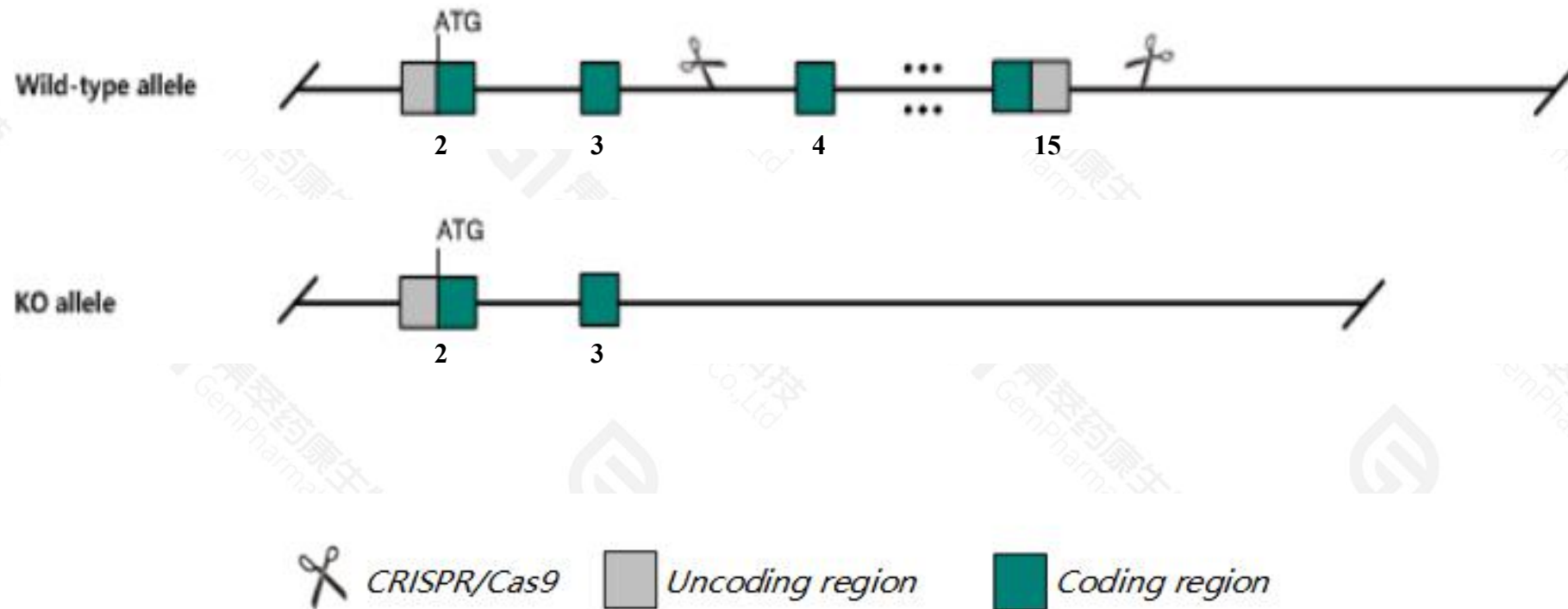
Cas9-KO

Strain background

C57BL/6JGpt

Knockout strategy

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



- The *Idua* gene has 8 transcripts. According to the structure of *Idua* gene, exon4-exon15 of *Idua*-201(ENSMUST00000071650.13) transcript is recommended as the knockout region. The region contains most of the coding sequence. Knock out the region will result in disruption of protein function.
- In this project we use CRISPR/Cas9 technology to modify *Idua* gene. The brief process is as follows: CRISPR/Cas9 system were microinjected into the fertilized eggs of C57BL/6JGpt 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/6JGpt mice.

- According to the existing MGI data, targeted mutants show lysosomal storage in multiple tissues, increased urinary GAG, craniofacial and skeletal defects, increased body weight, impaired habituation and long-term memory for aversive training, reduced ventricular function with valve insufficiency, and progressive hearing loss.
- This strategy may affect the 5-terminal regulation of *Slc26a1* gene.
- The *Idua* gene is located on the Chr5. 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.

Idua iduronidase, alpha-L [Mus musculus (house mouse)]

Gene ID: 15932, updated on 9-Feb-2021

Summary



Official Symbol Idua provided by [MGI](#)

Official Full Name iduronidase, alpha-L provided by [MGI](#)

Primary source [MGI:MGI:96418](#)

See related [Ensembl:ENSMUSG00000033540](#)

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 6030426D08

Expression Ubiquitous expression in ovary adult (RPKM 10.1), adrenal adult (RPKM 10.0) and 28 other tissues [See more](#)

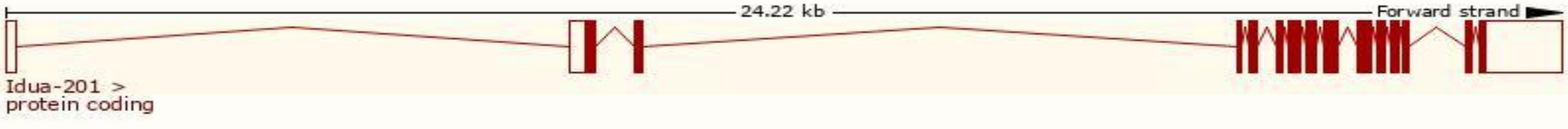
Orthologs [human](#) [all](#)

Transcript information (Ensembl)

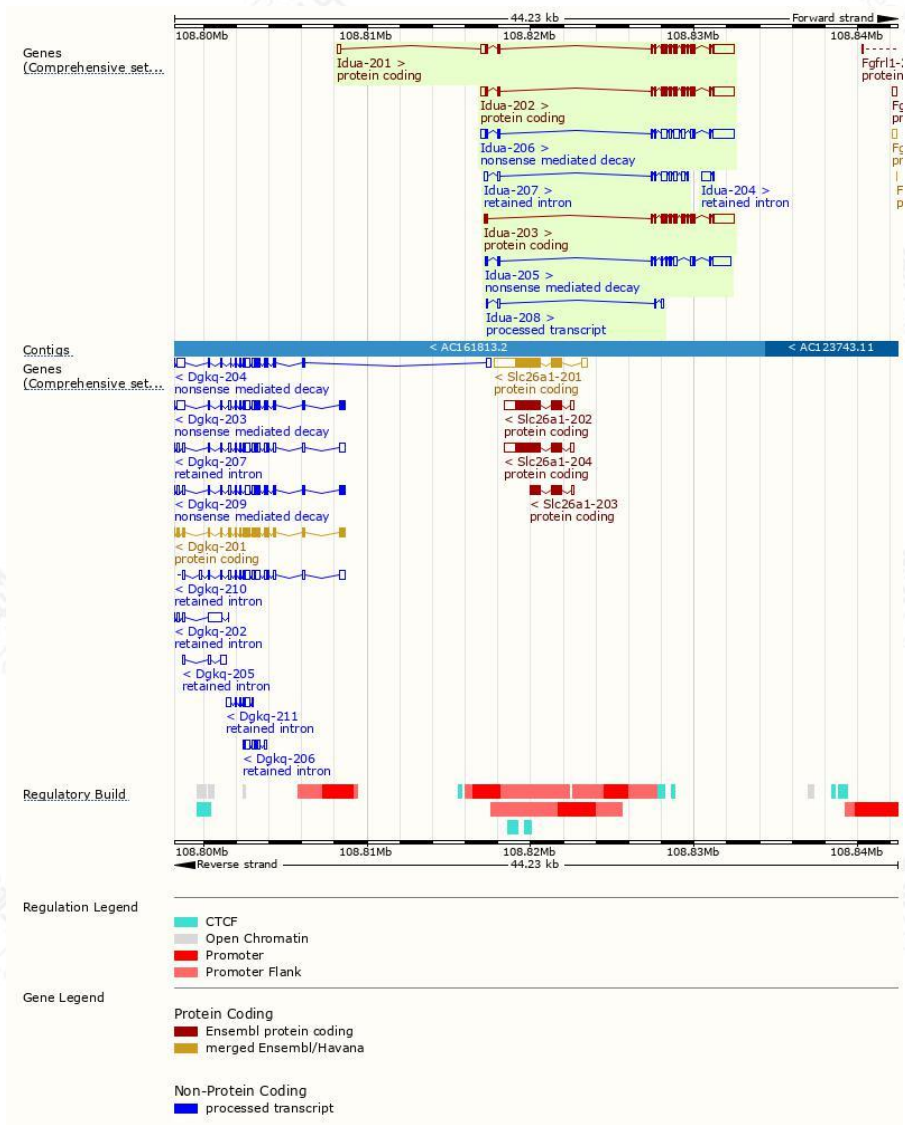
The gene has 8 transcripts,all transcripts are shown below:

Name	Transcript ID	bp	Protein	Biotype	CCDS	UniProt	Flags
Idua-201	ENSMUST00000071650.13	3536	643aa	Protein coding	CCDS19516		TSL:1 , GENCODE basic , APPRIS P1 ,
Idua-202	ENSMUST00000112563.9	3373	643aa	Protein coding	CCDS19516		TSL:1 , GENCODE basic , APPRIS P1 ,
Idua-203	ENSMUST00000119212.8	3022	596aa	Protein coding	CCDS71635		TSL:1 , GENCODE basic ,
Idua-206	ENSMUST00000140620.8	3412	151aa	Nonsense mediated decay	-		TSL:5 ,
Idua-205	ENSMUST00000139734.2	2539	281aa	Nonsense mediated decay	-		TSL:5 ,
Idua-208	ENSMUST00000159464.2	447	No protein	Processed transcript	-		TSL:2 ,
Idua-207	ENSMUST00000151445.8	1637	No protein	Retained intron	-		TSL:1 ,
Idua-204	ENSMUST00000133482.2	600	No protein	Retained intron	-		TSL:2 ,

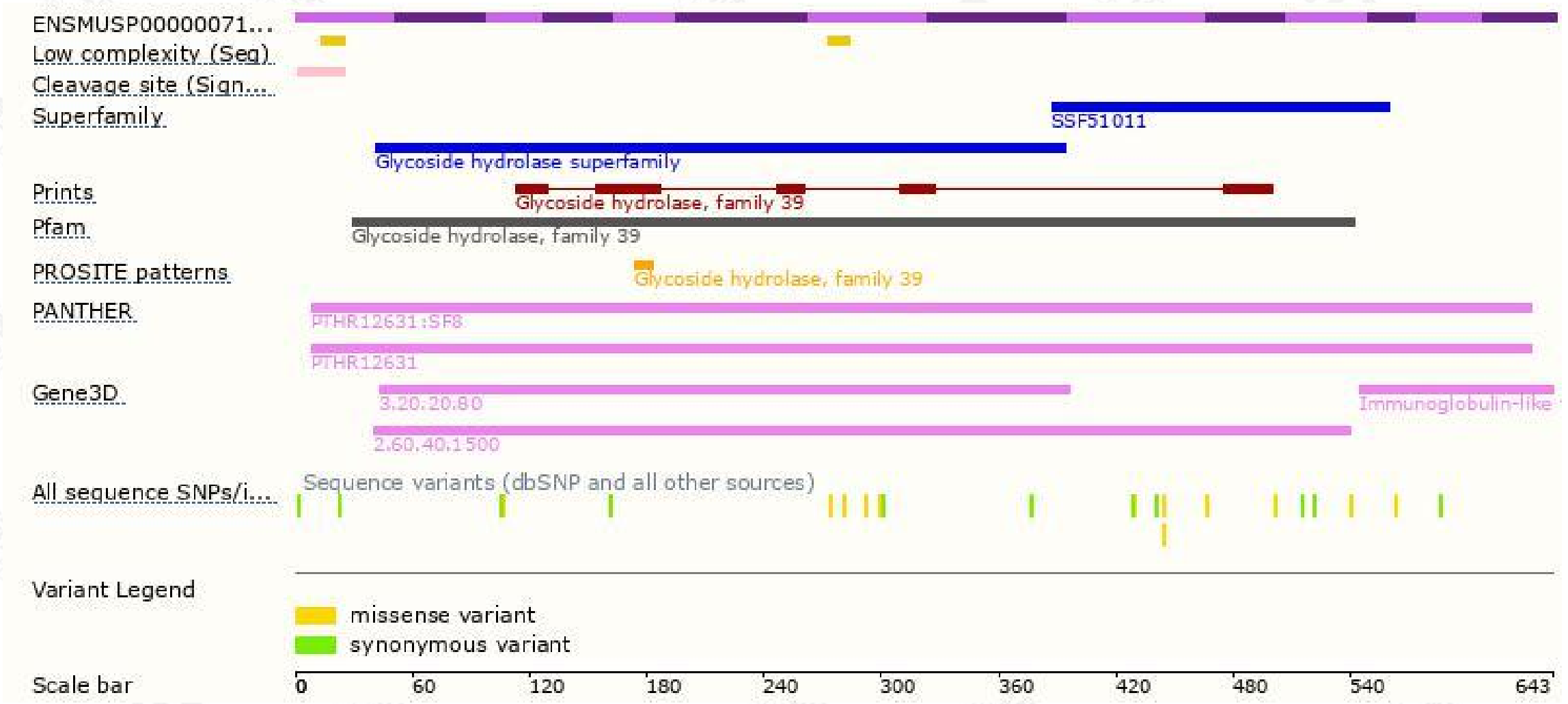
The strategy is based on the design of *Idua-201* transcript,the transcription is shown below:



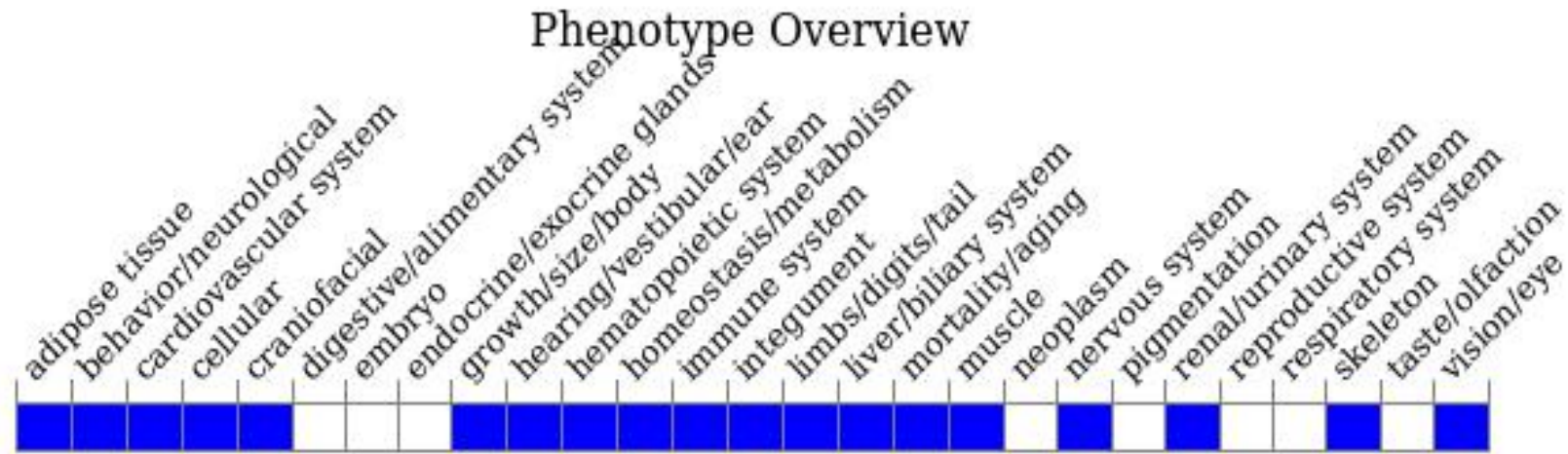
Genomic location distribution



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, targeted mutants show lysosomal storage in multiple tissues, increased urinary GAG, craniofacial and skeletal defects, increased body weight, impaired habituation and long-term memory for aversive training, reduced ventricular function with valve insufficiency, and progressive hearing loss.

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