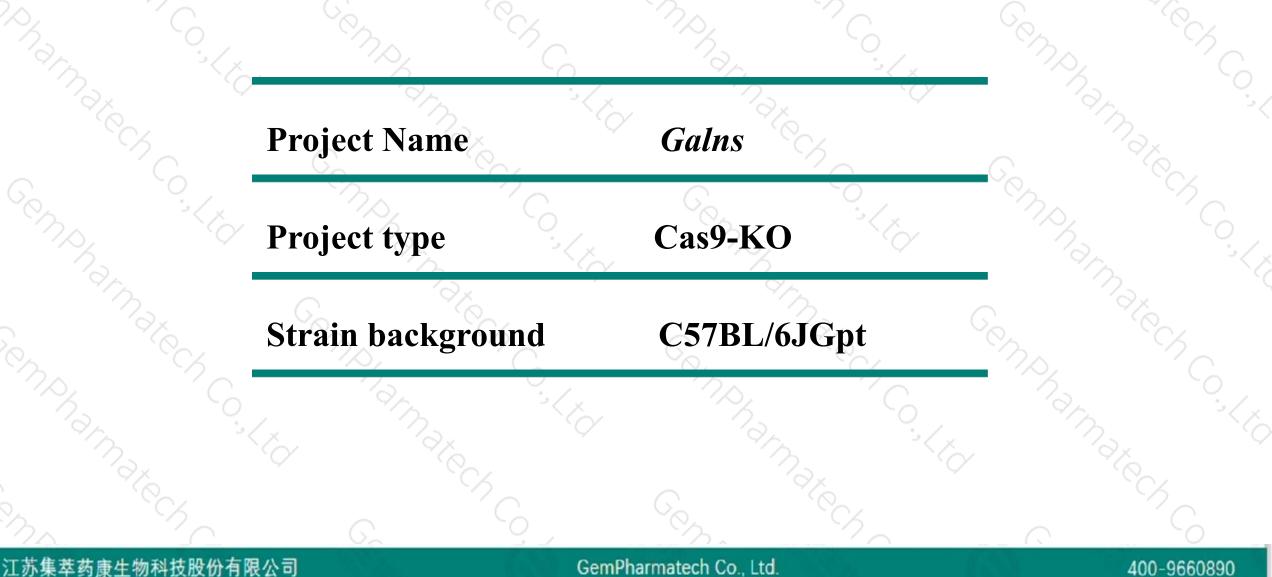


# Galns Cas9-KO Strategy

Designer: Reviewer: Design Date: JiaYu Xiaojing Li 2020-2-17

### **Project Overview**





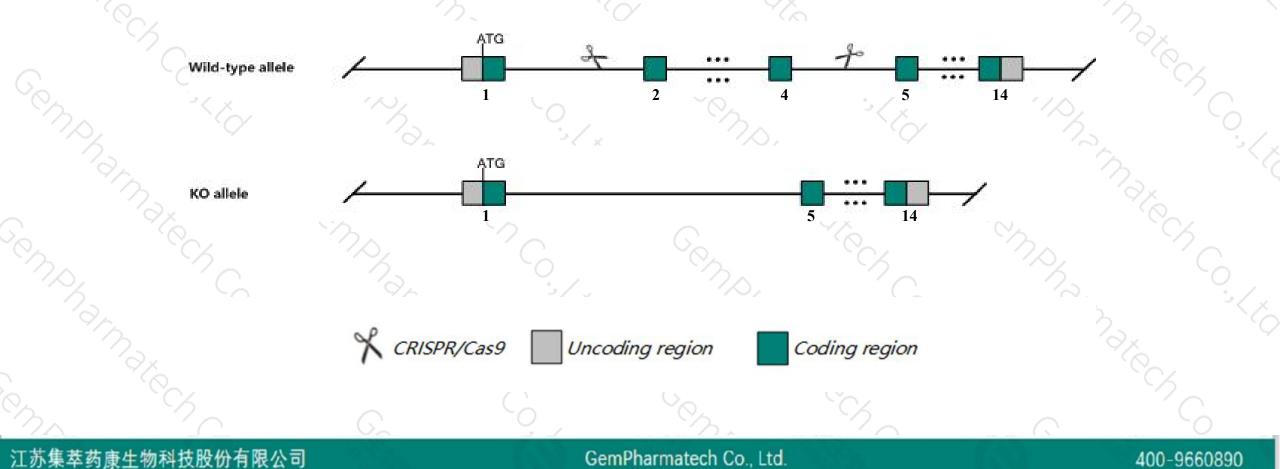
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# **Knockout strategy**



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





- The Galns gene has 3 transcripts. According to the structure of Galns gene, exon2-exon4 of Galns-201 (ENSMUST00000015171.10) transcript is recommended as the knockout region. The region contains 302bp coding sequence. Knock out the region will result in disruption of protein function.
- > In this project we use CRISPR/Cas9 technology to modify Galns gene. The brief process is as follows: CRISPR/Cas9 system

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- According to the existing MGI data, Homozygous mutant mice are viable, fertile, and healthy in spite of lysosmal storage.
- ► The flox region is in the intron of the Gm20388 gene, which may affect the regulation of this gene.
- The Galns gene is located on the Chr8. 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.

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# **Gene information (NCBI)**



\$ ?

### Galns galactosamine (N-acetyl)-6-sulfate sulfatase [Mus musculus (house mouse)]

Gene ID: 50917, updated on 31-Jan-2019

#### Summary

| Official Symbol           | Gains provided by MGI  |
|---------------------------|--|
| <b>Official Full Name</b> | galactosamine (N-acetyl)-6-sulfate sulfatase provided by MGI   |
| Primary source            | MGI:MGI:1355303  |
| See related               | Ensembl:ENSMUSG0000015027  |
| Gene type                 | protein coding   |
| <b>RefSeq status</b>      | 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             | mFLJ00319  |
| Expression                | Ubiquitous expression in placenta adult (RPKM 15.5), kidney adult (RPKM 12.0) and 28 other tissues See more                          |
| Orthologs                 | human all  |
|                           |  |

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## **Transcript information (Ensembl)**



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

| Name Transcript ID |                       | bp Protein Biot |              | Biotype        | type CCDS UniProt |               | Flags                         |  |
|--------------------|-----------------------|-----------------|--------------|----------------|-------------------|---------------|-------------------------------|--|
| Gains-201          | ENSMUST00000015171.10 | 2557            | <u>520aa</u> | Protein coding | CCDS40504         | <u>Q571E4</u> | TSL:1 GENCODE basic APPRIS P1 |  |
| Galns-203          | ENSMUST00000212319.1  | 2111            | <u>440aa</u> | Protein coding | CCDS85625         | <u>Q8CC47</u> | TSL:1 GENCODE basic           |  |
| Gains-202          | ENSMUST00000211906.1  | 381             | No protein   | IncRNA         | -                 | 2             | TSL:1                         |  |

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

#### < Galns-201 protein coding

Reverse strand

- 33.22 kb -

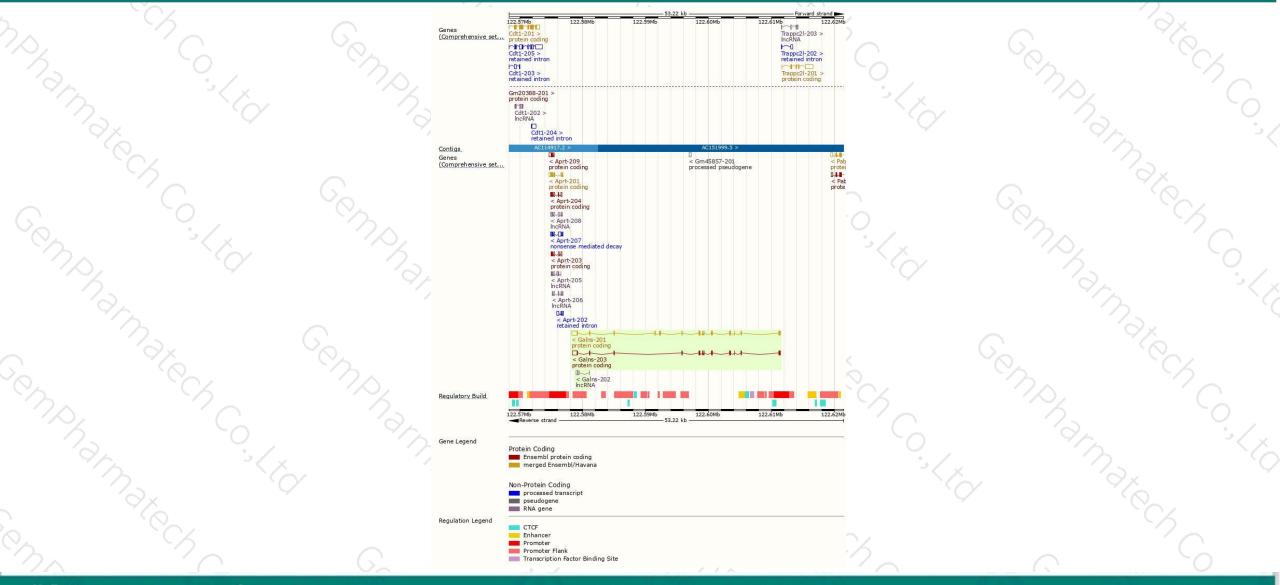
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### **Genomic location distribution**





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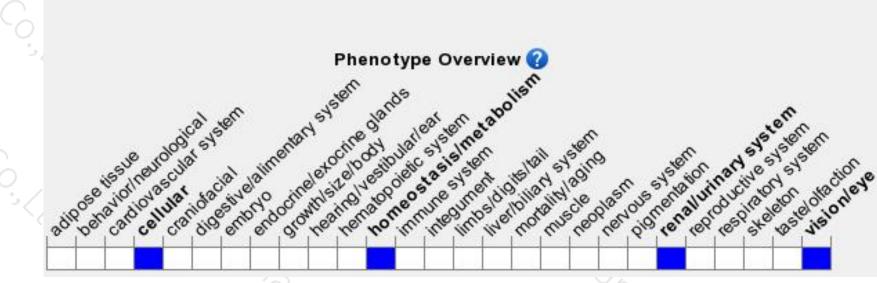
### **Protein domain**



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|--------|---|------------------------------------|--------------------------|--------------------|--------------|------------|------------------|
|        | Scale bar   | 0 60<br>G                          | 120 1                    | 80 240             | 300 360      | 420        | 520              |
|        | Variant Legend  | missense variar                    |                          | - 75               | 50° 50       |            |                  |
|        | All sequence SNPs/i   | Sequence variants (c               | dbSNP and all other s    | ources)            | <b>1</b> (1) | C 1930     |                  |
| 0      | CDD   | N-acetylgalactos                   | amine-6-sulfatase        |                    |              |            | -                |
|        | Gene3D  | PTHR42693 :SF8<br>Alkaline-phospha | atase-like, core domain  | soperfamily        | 3.           | 30.1120.10 |                  |
| Gen    | PROSITE patterns<br>PANTHER   | PTHR42693                          | tase, conserved site     |                    |              |            |                  |
|        | Pfam.   | Sulfatase, N-terr                  | minal                    |                    | PF           | 14707      |                  |
| 20131. | ENSMUSP00000015<br>Low complexity (Seg)<br>Cleavage site (Sign<br>Superfamily | Alkaline-phosphat                  | tase-like, core domain s | uperfamily         |              |            |                  |
| 2      |   |                                    |                          |                    | $\sim CZ$    |            | $\odot$ $\times$ |

### 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, Homozygous mutant mice are viable, fertile, and healthy in spite of lysosmal

storage.



If you have any questions, you are welcome to inquire. Tel: 400-9660890



