

# 70/2/70 Co. 1/2/ Abcal Cas9-KO Strategy The state of the s Constant de Co

Condand Stock Consolarna de Co Designer:Lixin LYU

# **Project Overview**



**Project Name** 

Abca1

**Project type** 

Cas9-KO

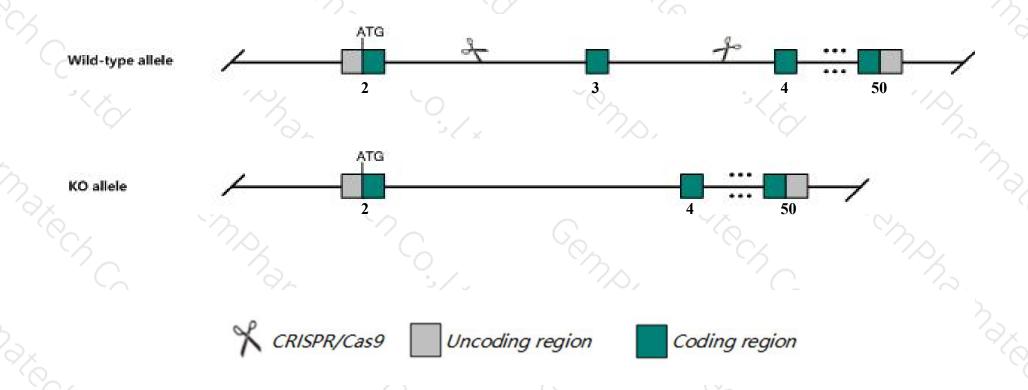
Strain background

C57BL/6JGpt

# **Knockout strategy**



This model will use CRISPR/Cas9 technology to edit the Abca1 gene. The schematic diagram is as follows:



## **Technical routes**



- ➤ The *Abca1* gene has 2 transcripts. According to the structure of *Abca1* gene, exon3 of *Abca1-201*(ENSMUST0000030010.3) transcript is recommended as the knockout region. The region contains 94bp coding sequence.

  Knock out the region will result in disruption of protein function.
- ➤ In this project we use CRISPR/Cas9 technology to modify *Abca1* gene. The brief process is as follows: CRISPR/Cas9 system

## **Notice**



- ➤ According to the existing MGI data, Many homozygous null mutants die perinatally with placental defects. Survivors show altered steroidogenesis, defective lipid export in Golgi, low serum cholesterol, lipid accumulation in macrophages and lung, reduced fertility and kidney and heart defects.
- > The *Abca1* gene is located on the Chr4. 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)



#### Abca1 ATP-binding cassette, sub-family A (ABC1), member 1 [Mus musculus (house mouse)]

Gene ID: 11303, updated on 19-Mar-2019

#### Summary



Official Symbol Abca1 provided by MGI

Official Full Name ATP-binding cassette, sub-family A (ABC1), member 1 provided by MGI

Primary source MGI:MGI:99607

See related Ensembl: ENSMUSG00000015243

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 ABC-1, Abc1

Summary The membrane-associated protein encoded by this gene is a member of the superfamily of ATP-binding cassette (ABC) transporters. ABC

proteins transport various molecules across extra- and intracellular membranes. ABC genes are divided into seven distinct subfamilies (ABC1, MDR/TAP, MRP, ALD, OABP, GCN20, White). This protein is a member of the ABC1 subfamily. Members of the ABC1 subfamily comprise the only major ABC subfamily found exclusively in multicellular eukaryotes. In humans, this protein functions as a cholesterol efflux pump in the cellular lipid removal pathway. Mutations in the human gene have been associated with Tangier's disease and familial high-density lipoprotein deficiency. [provided by RefSeq, Jul 2008]

Expression Ubiquitous expression in ovary adult (RPKM 16.1), subcutaneous fat pad adult (RPKM 14.9) and 25 other tissuesSee more

Orthologs <u>human</u> all

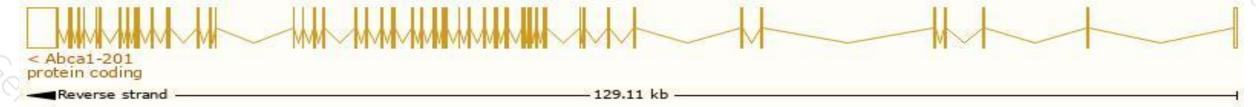
# Transcript information (Ensembl)



The gene has 2 transcripts, all transcripts are shown below:

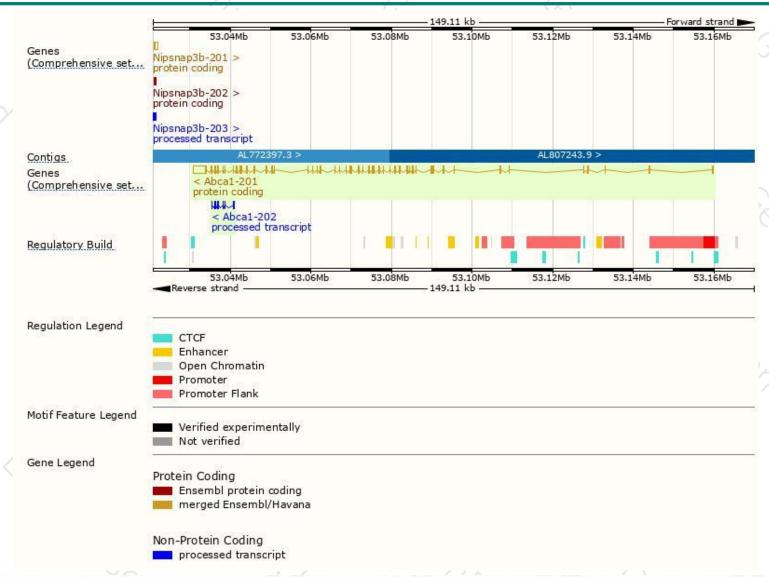
Name	Transcript ID	bp	Protein	Biotype	CCDS	UniProt	Flags
Abca1-201	ENSMUST00000030010.3	10262	2261aa	Protein coding	CCDS18187	P41233	TSL:1 GENCODE basic APPRIS P1
Abca1-202	ENSMUST00000149127.1	775	No protein	Processed transcript	197	676	TSL:5

The strategy is based on the design of Abca1-201 transcript, The transcription is shown below



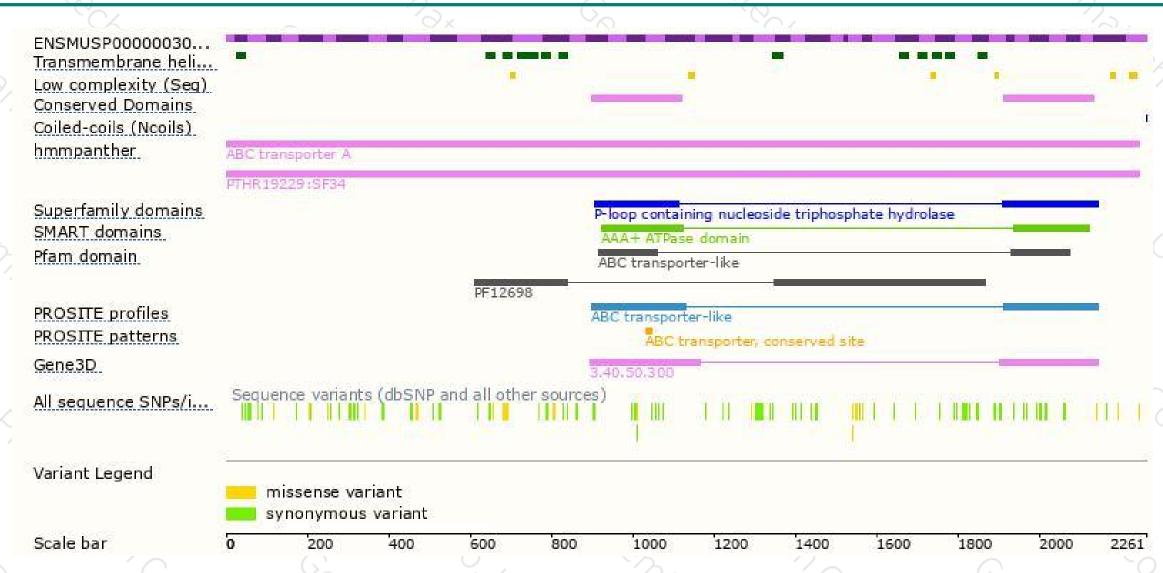
## Genomic location distribution





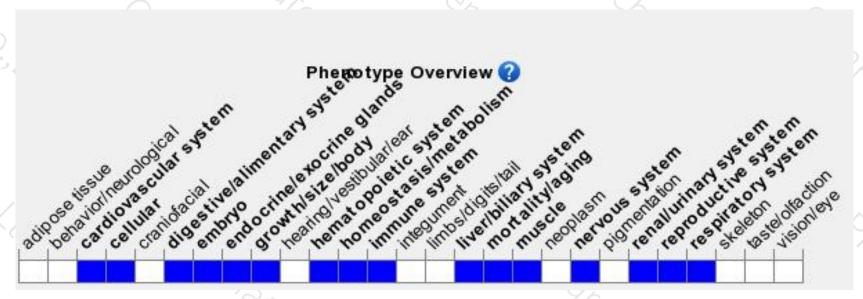
### Protein domain





## 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, Many homozygous null mutants die perinatally with placental defects. Survivors show altered steroidogenesis, defective lipid export in Golgi, low serum cholesterol, lipid accumulation in macrophages and lung, reduced fertility and kidney and heart defects.



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





