

# *Abca4* Cas9-KO Strategy

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# Project Overview

**Project Name**

*Abca4*

**Project type**

**Cas9-KO**

**Strain background**

**C57BL/6JGpt**

# Knockout strategy

This model will use CRISPR/Cas9 technology to edit the *Abca4* gene. The schematic diagram is as follows:



- The *Abca4* gene has 10 transcripts. According to the structure of *Abca4* gene, exon2-exon4 of *Abca4-201* (ENSMUST00000013995.12) transcript is recommended as the knockout region. The region contains 376bp coding sequence. Knock out the region will result in disruption of protein function.
- In this project we use CRISPR/Cas9 technology to modify *Abca4* gene. The brief process is as follows: CRISPR/Cas9 system

- According to the existing MGI data, Mice homozygous for targeted mutations that inactivate the gene display delayed rod dark adaptation and are a model for juvenile macular degeneration.
- The *Abca4* gene is located on the Chr3. 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)

## Abca4 ATP-binding cassette, sub-family A (ABC1), member 4 [Mus musculus (house mouse)]

Gene ID: 11304, updated on 31-Jan-2019

### Summary



**Official Symbol** Abca4 provided by [MGI](#)

**Official Full Name** ATP-binding cassette, sub-family A (ABC1), member 4 provided by [MGI](#)

**Primary source** [MGI:MGI:109424](#)

**See related** [Ensembl:ENSMUSG00000028125](#)

**Gene type** protein coding

**RefSeq status** REVIEWED

**Organism** [Mus musculus](#)

**Lineage** Eukaryota; Metazoa; Chordata; Craniata; Vertebrata; Euteleostomi; Mammalia; Eutheria; Euarchontoglires; Glires; Rodentia; Myomorpha; Muroidea; Muridae; Murinae; Mus; Mus

**Also known as** AW050280, Abc10, Abcr, D430003I15Rik, RmP

**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. This protein was the first of the ABC transporters to be observed in photoreceptors and may play a role in the photoresponse. Mutations in the human gene are found in patients diagnosed with Stargardt disease and are associated with retinitis pigmentosa-19 and macular degeneration age-related 2. [provided by RefSeq, Jul 2008]

**Expression** Broad expression in kidney adult (RPKM 1.4), ovary adult (RPKM 1.3) and 20 other tissues [See more](#)

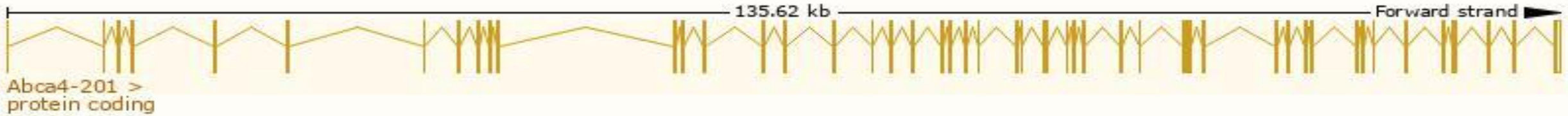
**Orthologs** [human](#) [all](#)

# Transcript information (Ensembl)

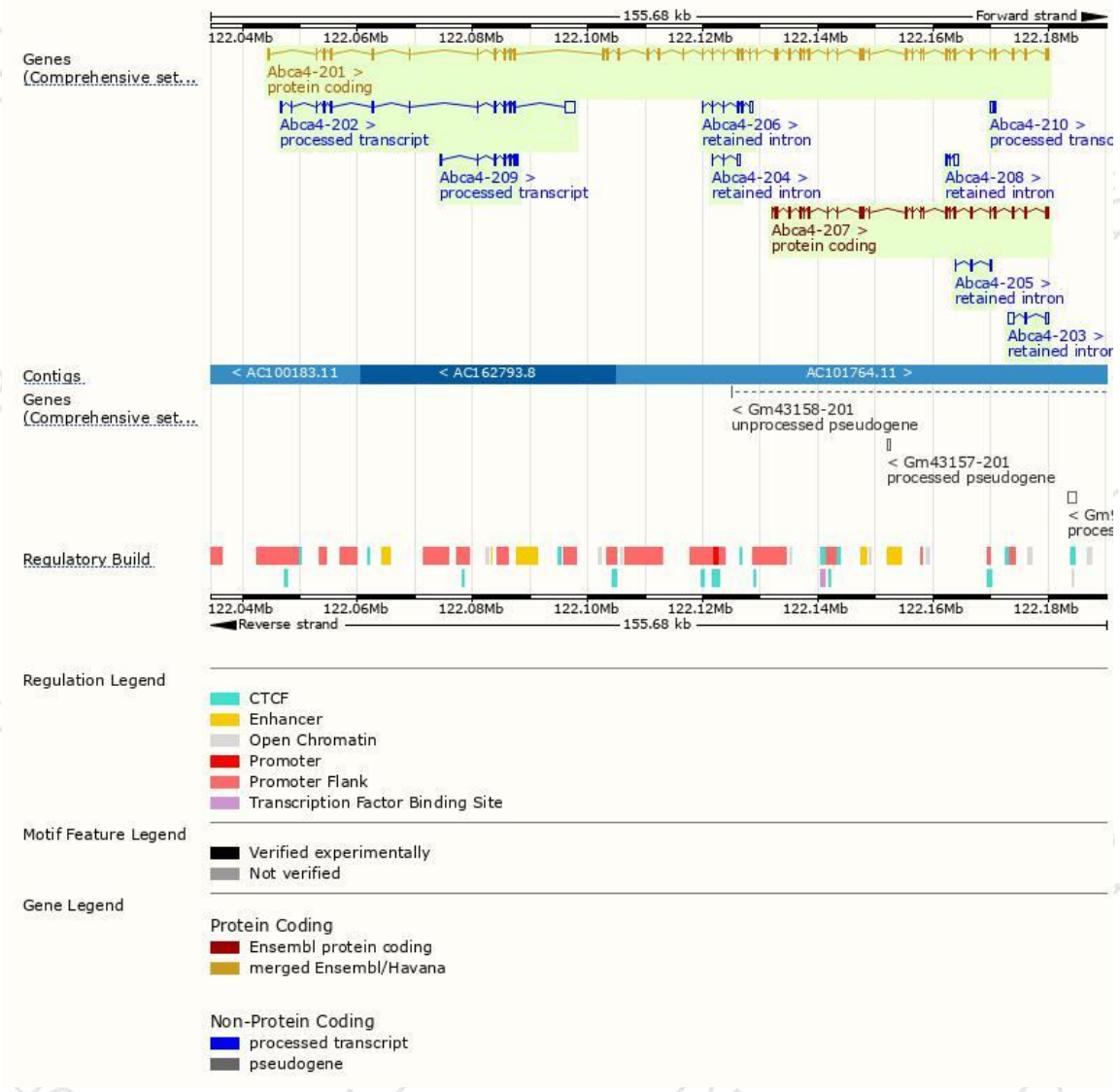
The gene has 10 transcripts,all transcripts are shown below:

Name	Transcript ID	bp	Protein	Biotype	CCDS	UniProt	Flags
Abca4-201	<a href="#">ENSMUST00000013995.12</a>	7263	<a href="#">2310aa</a>	Protein coding	<a href="#">CCDS38617</a>	<a href="#">Q35600</a>	TSL:1 GENCODE basic APPRIS P1
Abca4-207	<a href="#">ENSMUST00000141135.4</a>	3606	<a href="#">1102aa</a>	Protein coding	-	<a href="#">A0A0G2JGG8</a>	TSL:1 GENCODE basic
Abca4-202	<a href="#">ENSMUST00000132199.7</a>	3511	No protein	Processed transcript	-	-	TSL:1
Abca4-209	<a href="#">ENSMUST00000150738.2</a>	1128	No protein	Processed transcript	-	-	TSL:1
Abca4-210	<a href="#">ENSMUST00000197728.1</a>	765	No protein	Processed transcript	-	-	TSL:3
Abca4-203	<a href="#">ENSMUST00000136358.2</a>	1520	No protein	Retained intron	-	-	TSL:5
Abca4-206	<a href="#">ENSMUST00000140913.7</a>	1198	No protein	Retained intron	-	-	TSL:1
Abca4-208	<a href="#">ENSMUST00000144949.1</a>	700	No protein	Retained intron	-	-	TSL:3
Abca4-205	<a href="#">ENSMUST00000137616.2</a>	652	No protein	Retained intron	-	-	TSL:3
Abca4-204	<a href="#">ENSMUST00000136624.1</a>	531	No protein	Retained intron	-	-	TSL:3

The strategy is based on the design of *Abca4-201* transcript,The transcription is shown below

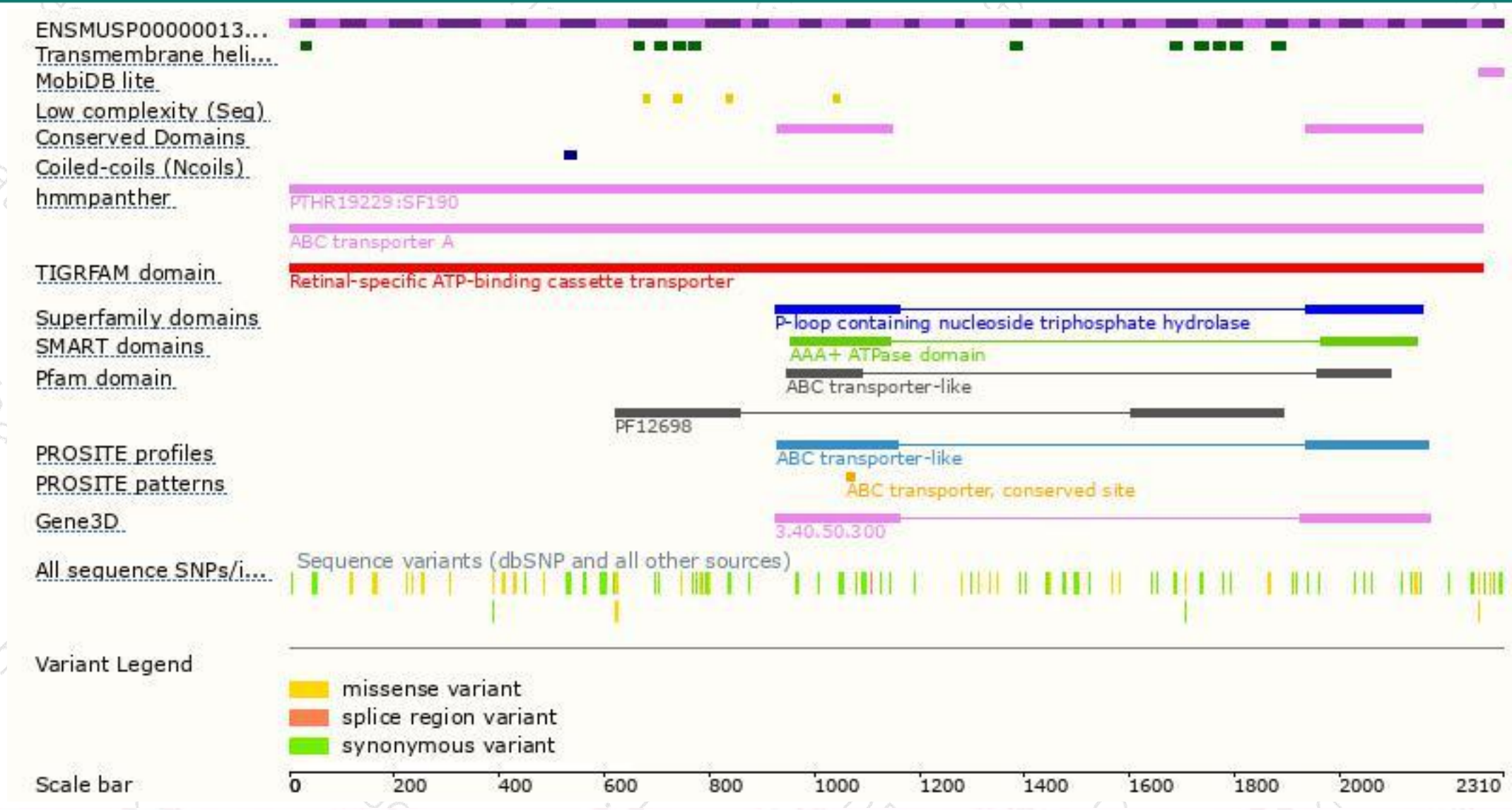


# Genomic location distribution

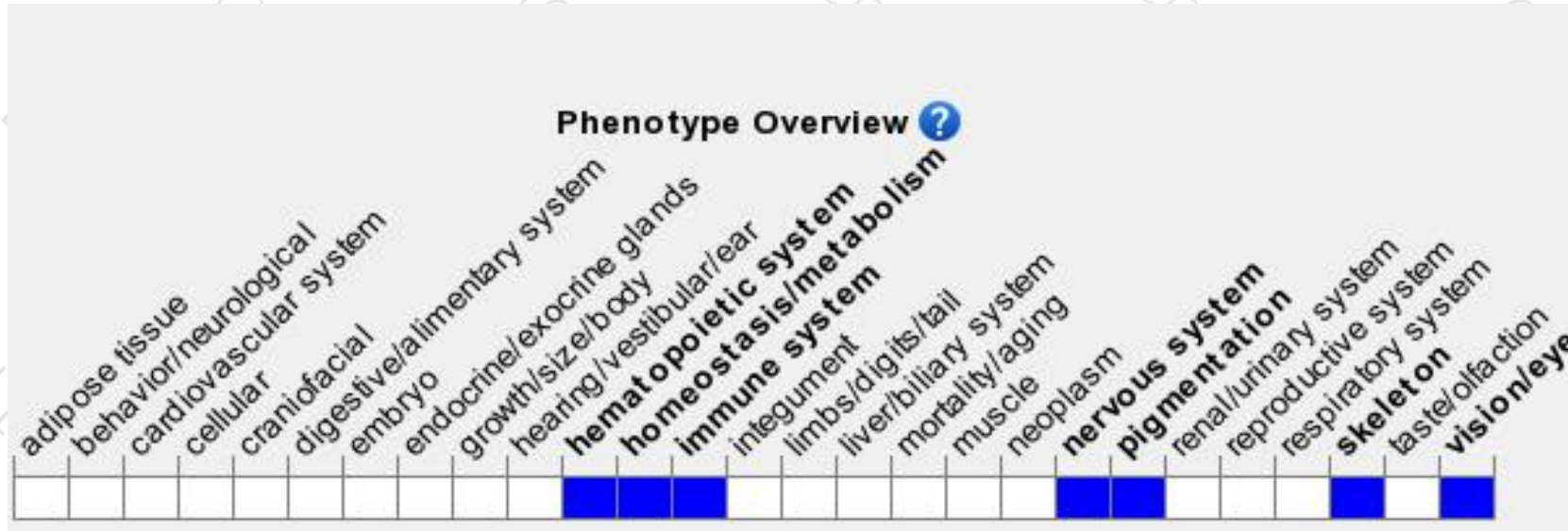




# Protein domain



# 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, Mice homozygous for targeted mutations that inactivate the gene display delayed rod dark adaptation and are a model for juvenile macular degeneration.

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

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