

# Atxn3 Cas9-KO Strategy

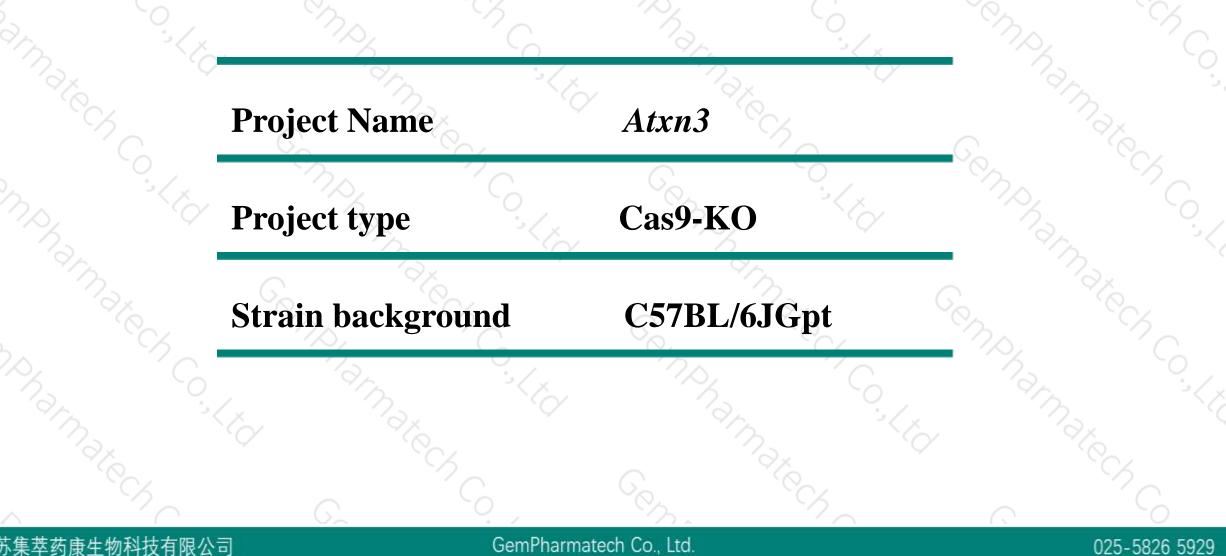
Designer: Design Date:

Q\_

Qiong Zhou 2018/5/31

### **Project Overview**



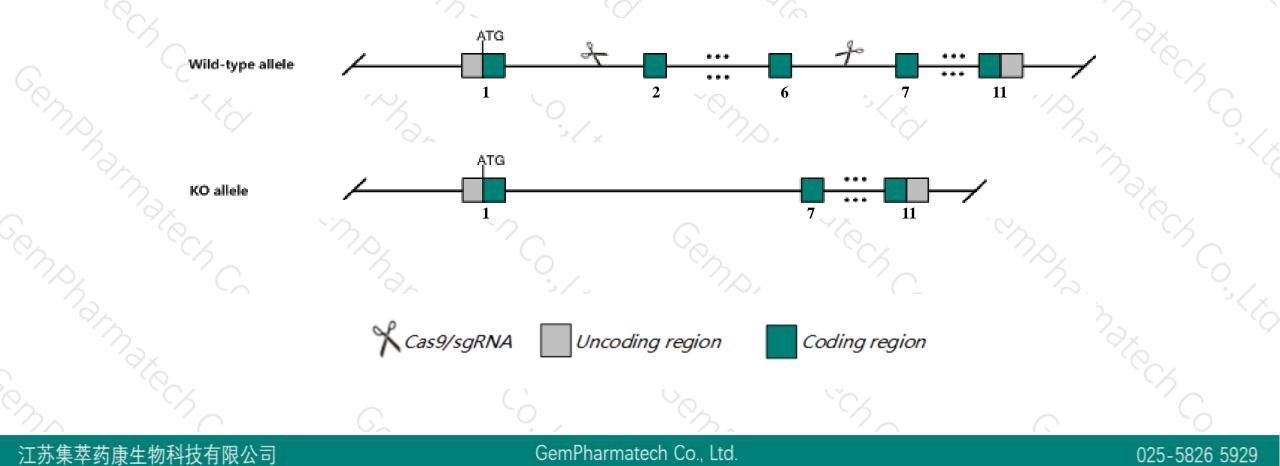


技有限公司 江苏集萃药康生

GemPharmatech Co., Ltd.



This model will use CRISPR/Cas9 technology to edit the Atxn3 gene. The schematic diagram is as follows:





- The Atxn3 gene has 5 transcripts. According to the structure of Atxn3 gene, exon2-exon6 of Atxn3-201 (ENSMUST0000021606.11) transcript is recommended as the knockout region. The region contains 451bp coding sequence. Knock out the region will result in disruption of protein function.
- In this project we use CRISPR/Cas9 technology to modify *Atxn3* gene. The brief process is as follows: sgRNA was transcribed in vitro.Cas9 and sgRNA 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.





025-5826 5929

- According to the existing MGI data, Decreased exploratory behavior is reported for mice homozygous for a disruption of this marker.
- The Atxn3 gene is located on the Chr12. 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)**



< ?

### Atxn3 ataxin 3 [Mus musculus (house mouse)]

Gene ID: 110616, updated on 9-Mar-2019

#### Summary

Official Symbol	Atxn3 provided by MGI					
Official Full Name	ataxin 3 provided by <u>MGI</u>					
Primary source	MGI:MGI:1099442					
See related	Ensembl:ENSMUSG0000021189					
Gene type	protein coding					
RefSeq status	VALIDATED					
Organism	Mus musculus					
Lineage	e Eukaryota; Metazoa; Chordata; Craniata; Vertebrata; Euteleostomi; Mammalia; Eutheria; Euarchontoglires; Glires; Rodentia; Myomorpha;					
	Muroidea; Muridae; Murinae; Mus; Mus					
Also known as	2210008M02Rik, AI463012, AI647473, ATX3, MJD1, Mjd, Sca3, ataxin-3					
Expression	<ul> <li>Ubiquitous expression in testis adult (RPKM 13.9), bladder adult (RPKM 6.4) and 24 other tissues</li> </ul>					
Orthologs	human all					

#### 江苏集萃药康生物科技有限公司

GemPharmatech Co., Ltd.



## **Transcript information (Ensembl)**



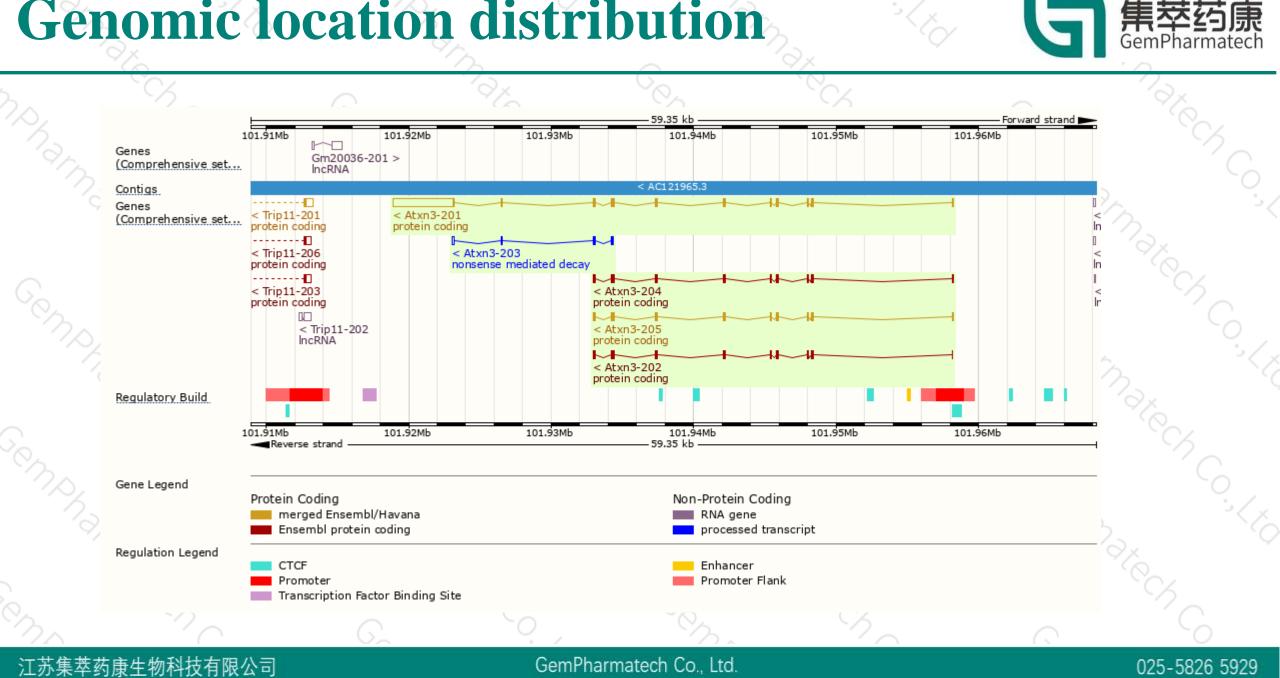
The gene has 5 transcripts, all transcripts are shown below:

						1			
Atxn3-201	ENSMUST0000021606.11	5363	<u>355aa</u>	Protein coding	<u>CCDS26115</u> 교	<u>Q546X9</u> & <u>Q9CVD2</u> &	TSL:1	SENCODE basic APPRIS P3	
Atxn3-205	ENSMUST00000161011.7	1000	<u>291aa</u>	Protein coding	<u>CCDS49144</u> &	<u>E9Q717</u> &	TSL:1 GE	ENCODE basic APPRIS ALT2	
Atxn3-204	ENSMUST00000160251.7	998	<u>296aa</u>	Protein coding	-	<u>Q5M8S1</u> @	TS	TSL:1 GENCODE basic	
Atxn3-202	ENSMUST00000159883.1	879	<u>287aa</u>	Protein coding	-	<u>F6T5L3</u> &	CD	S 5' incomplete TSL:5	
Atxn3-203	ENSMUST00000160186.1	439	<u>69aa</u>	Nonsense mediated decay	-	<u>F6TRN1</u> 교	CD	S 5' incomplete TSL:5	

The strategy is based on the design of Atxn3-201 transcript, The transcription is shown below



### **Genomic location distribution**



### **Protein domain**

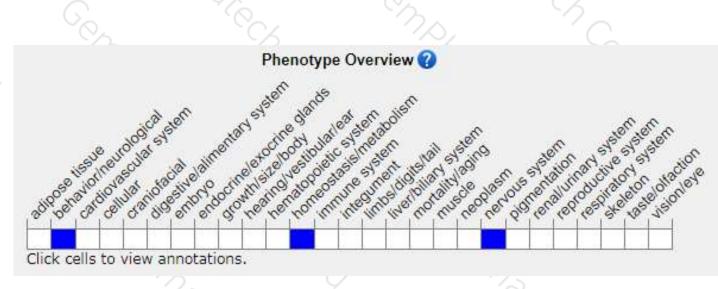


江苏集萃药康生	物科技有限公司		GemPharma	tech Co., Ltd.	2 1		025-5826 5929
npharma ma	X.C.C.C.C.C.C.C.C.C.C.C.C.C.C.C.C.C.C.C			Cen .	Nate Charles	i ann	-< x 2 2 2 2 2 3 3 3 3 3 3 3 3 3 3 3 3 3 3
	Scale bar	missense variant 0 40	80 120	synonymo 160 200	240 280		355
	Variant Legend						- `%
	All sequence SNPs/i	3.90.70.40 Sequence variants (dbSNP and	all other sources)			т. н.	
CMPhar.	Gene3D	Machado-Joseph disease protein 1.10.287.10					
	PANTHER	PTHR14159:SF1					
	PROSITE profiles	Josephin domain			PF16619 Ubiquitin interacting motif		
	Pfam	Josephin domain			Ubiquitin interacting motif		
	Prints	PR01233					
armaker	MobiDB lite Low complexity (Seg) Coiled-coils (Ncoils) SMART	Josephin domain			Ubiquitin interacting motif	<u> </u>	
	ENSMUSP00000021		5	~~X	C	~ C>	_ `%

### Mouse phenotype description(MGI)



025-5826



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, Decreased exploratory behavior is reported for mice homozygous for a disruption of this marker.



If you have any questions, you are welcome to inquire. Tel: 025-5864 1534



