

b

Splice donor

Intron

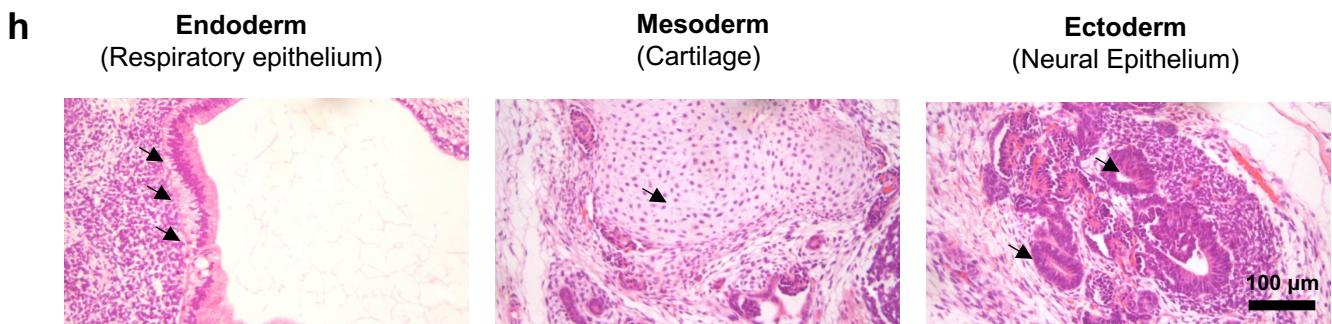
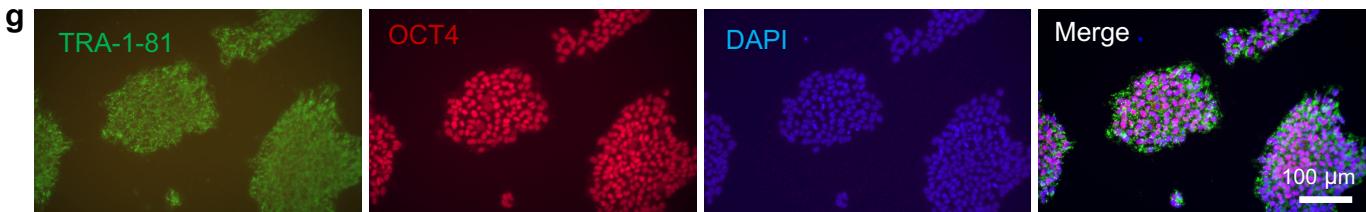
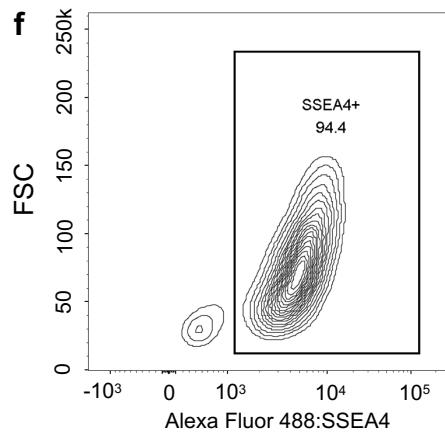
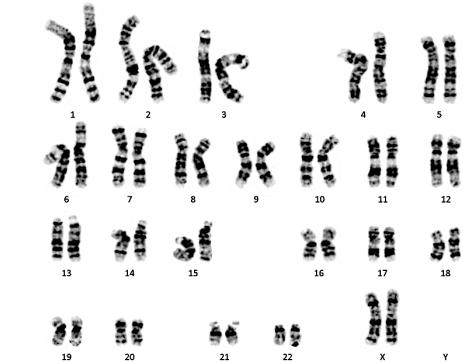
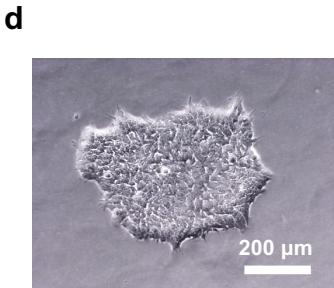
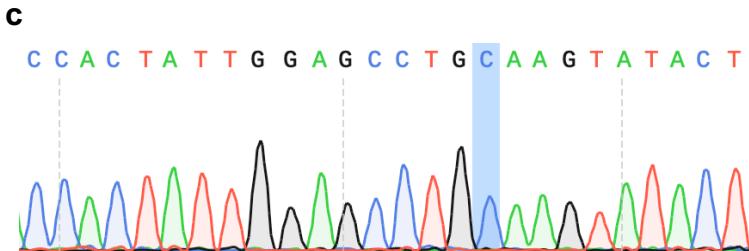
exon50

CC ACT ATT GGG AGC CT **g t** a a g t a t a c t

GGT GATAACCTCGGA **cat t cat a** t ca

PAM

gRNA for ABEmax



a

| | | | |
|------------|---------|---------|---------|
| Mutation 1 | Exon 50 | 74bp | Exon 51 |
| Mutation 2 | Exon 49 | Exon 51 | |

Lane1: 100bp ladder
Lane2-3: Mut1/Mut2
Lane4-6: WT, 858bp
Mutation1: insert 74bp
Mutation2: 109bp deletion

b**WT**

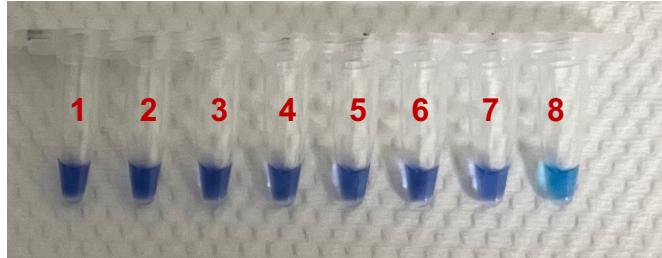
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K T Q I P E N W E H A K Y K W Y L K E L Q D G I G Q R Q T V V R T L N A T G E E I I Q Q S S K T D A S I L Q E K L G S L N L R W Q E V C K Q L S D R
K K R L E E Q K N I L S E F Q R D L N E F V L W L E E A D N I A S I P L E P G K E Q Q L K E K L E Q V K L L V E E L P L R Q G I L K Q L N E T G G P V L
V S A P I S P E E Q D K L E N K L K Q T N L Q W I K V S R A L P E K Q G E I E A Q I K D L G Q L E K K L E D L E E Q L N H L L L W L S P I R N Q L E I Y N
Q P N Q E G P F D V K E T E I A V Q A K Q P D V E E I L S K G Q H L Y K E K P A T Q P V K R K L E D L S S E W K A V N R L L Q E L R A K Q P D L A P
G L T T I G A S P T Q T V T L V T Q P V V T K E T A I S K L E M P S S L M L E V P A L A D F N R A W T E L T D W L S L L D Q V I K S Q R V M V G D L E
D I N E M I I K Q K A T M Q D L E Q R R P Q L E E L I T A A Q N L K N K T S N Q E A R T I I T D R I E R I Q N ...*

Mutation1

...KTAALQSATPVERV р K L Q E A L S Q L D F Q W E K V N K M Y K D R Q G R F D R S V E K W R R F H Y D I K I F N Q W L T E A E Q F L R
K T Q I P E N W E H A K Y K W Y L K E L Q D G I G Q R Q T V V R T L N A T G E E I I Q Q S S K T D A S I L Q E K L G S L N L R W Q E V C K Q L S D R
K K R L E E Q K N I L S E F Q R D L N E F V L W L E E A D N I A S I P L E P G K E Q Q L K E K L E Q V K L L V E E L P L R Q G I L K Q L N E T G G P V L
V S A P I S P E E Q D K L E N K L K Q T N L Q W I K V S R A L P E K Q G E I E A Q I K D L G Q L E K K L E D L E E Q L N H L L L W L S P I R N Q L E I Y N
Q P N Q E G P F D V K E T E I A V Q A K Q P D V E E I L S K G Q H L Y K E K P A T Q P V K R K L E D L S S E W K A V N R L L Q E L R A K Q P D L A P
G L T T I G A C K Y T G S H S I W L *

Mutation2

...KTAALQSATPVERV р K L Q E A L S Q L D F Q W E K V N K M Y K D R Q G R F D R S V E K W R R F H Y D I K I F N Q W L T E A E Q F L R
K T Q I P E N W E H A K Y K W Y L K E L Q D G I G Q R Q T V V R T L N A T G E E I I Q Q S S K T D A S I L Q E K L G S L N L R W Q E V C K Q L S D R
K K R L E E Q K N I L S E F Q R D L N E F V L W L E E A D N I A S I P L E P G K E Q Q L K E K L E Q V K L L V E E L P L R Q G I L K Q L N E T G G P V L
V S A P I S P E E Q D K L E N K L K Q T N L Q W I K V S R A L P E K Q G E I E A Q I K D L G Q L E K K L E D L E E Q L N H L L L W L S P I R N Q L E I Y N
Q P N Q E G P F D V K E T E I A V Q A K Q P D V E E I L S K G Q H L Y K E K P A T Q P V K L L R L L L W *

c

- Lane1-4: ΔEx50 cell
Lane5-7: Negative control
Lane8: Positive control
Blue : Positive;
Purple : Negative