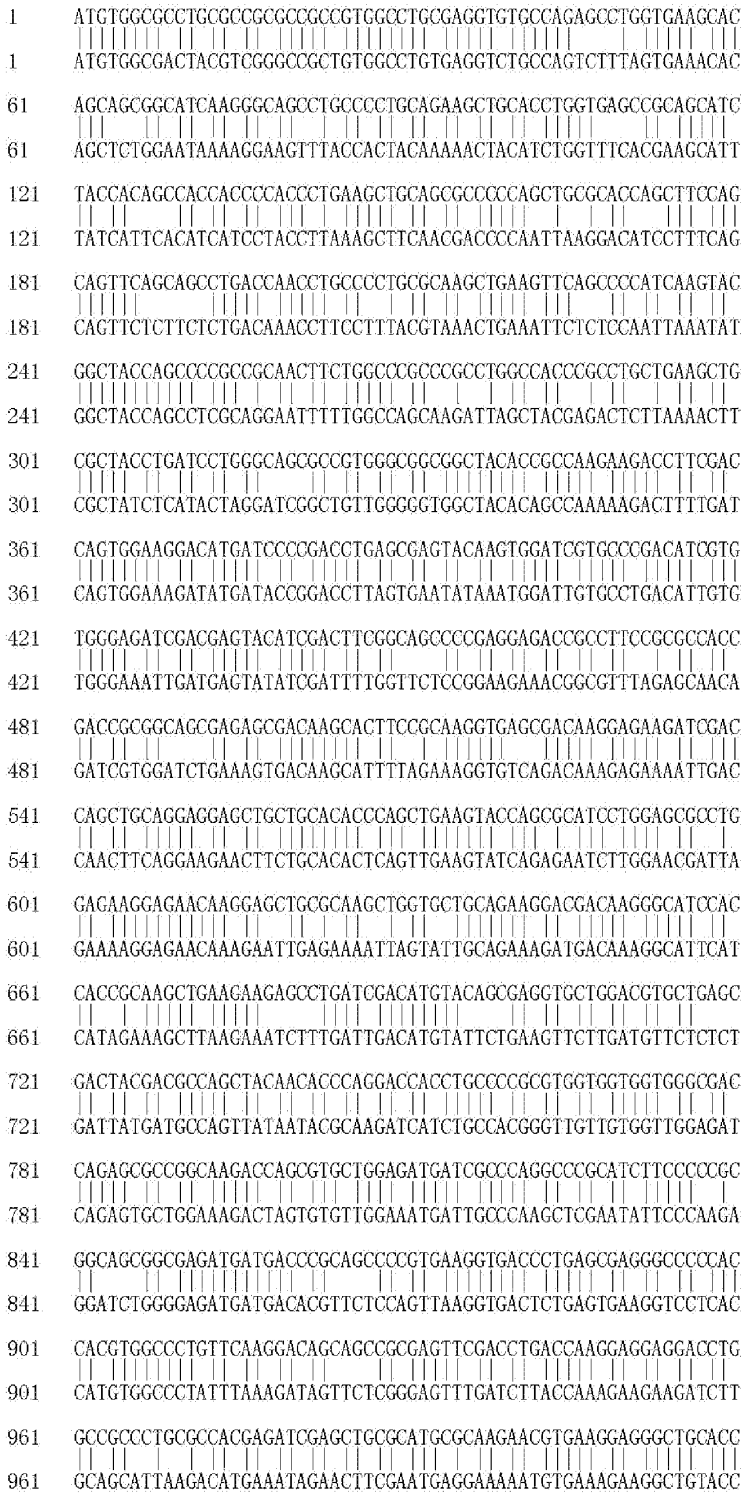

1 ATGTGGCGCCTGCGCCGCGCCGCCGTGGCCTGCGAGGTGTGCCAGAGCCTGGTGAAGCAC
1 ATGTGGCGACTACGTCGGGCCGCTGTGGCCTGTGAGGTCTGCCAGTCTTTAGTGAAACAC
61 AGCAGCGGCATCAAGGGCAGCCTGCCCCTGCAGAAGCTGCACCTGGTGAGCCGCAGCATC
61 AGCTCTGGAATAAAAGGAAGTTTACCACTACAAAAACTACATCTGGTTTCACGAAGCATT
121 TACCACAGCCACCACCCCACCCTGAAGCTGCAGCGCCCCCAGCTGCGCACCAGCTTCCAG
121 TATCATTCACATCATCCTACCTTAAAGCTTCAACGACCCCAATTAAGGACATCCTTTCAG
181 CAGTTCAGCAGCCTGACCAACCTGCCCCTGCGCAAGCTGAAGTTCAGCCCCATCAAGTAC
181 CAGTTCTCTTCTCTGACAAACCTTCCTTTACGTAAACTGAAATTCTCTCCAATTAAATAT
241 GGCTACCAGCCCCGCCGCAACTTCTGGCCCGCCCGCCTGGCCACCCGCCTGCTGAAGCTG
241 GGCTACCAGCCTCGCAGGAATTTTTGGCCAGCAAGATTAGCTACGAGACTCTTAAAACTT
301 CGCTACCTGATCCTGGGCAGCGCCGTGGGCGGCGGCTACACCGCCAAGAAGACCTTCGAC
301 CGCTATCTCATACTAGGATCGGCTGTTGGGGGTGGCTACACAGCCAAAAAGACTTTTGAT
361 CAGTGGAAGGACATGATCCCCGACCTGAGCGAGTACAAGTGGATCGTGCCCGACATCGTG
361 CAGTGGAAAGATATGATACCGGACCTTAGTGAATATAAATGGATTGTGCCTGACATTGTG
421 TGGGAGATCGACGAGTACATCGACTTCGGCAGCCCCGAGGAGACCGCCTTCCGCGCCACC
421 TGGGAAATTGATGAGTATATCGATTTTGGTTCTCCGGAAGAAACGGCGTTTAGAGCAACA
481 GACCGCGGCAGCGAGAGCGACAAGCACTTCCGCAAGGTGAGCGACAAGGAGAAGATCGAC
481 GATCGTGGATCTGAAAGTGACAAGCATTTTAGAAAGGTGTCAGACAAAGAGAAAATTGAC
541 CAGCTGCAGGAGGAGCTGCTGCACACCCAGCTGAAGTACCAGCGCATCCTGGAGCGCCTG
541 CAACTTCAGGAAGAACTTCTGCACACTCAGTTGAAGTATCAGAGAATCTTGGAACGATTA
601 GAGAAGGAGAACAAGGAGCTGCGCAAGCTGGTGCTGCAGAAGGACGACAAGGGCATCCAC
601 GAAAAGGAGAACAAAGAATTGAGAAAATTAGTATTGCAGAAAGATGACAAAGGCATTCAT
661 CACCGCAAGCTGAAGAAGAGCCTGATCGACATGTACAGCGAGGTGCTGGACGTGCTGAGC
661 CATAGAAAGCTTAAGAAATCTTTGATTGACATGTATTCTGAAGTTCTTGATGTTCTCTCT
721 GACTACGACGCCAGCTACAACACCCAGGACCACCTGCCCCGCGTGGTGGTGGTGGGCGAC
721 GATTATGATGCCAGTTATAATACGCAAGATCATCTGCCACGGGTTGTTGTGGTTGGAGAT
781 CAGAGCGCCGGCAAGACCAGCGTGCTGGAGATGATCGCCCAGGCCCGCATCTTCCCCCGC
781 CAGAGTGCTGGAAAGACTAGTGTGTTGGAAATGATTGCCCAAGCTCGAATATTCCCAAGA
841 GGCAGCGGCGAGATGATGACCCGCAGCCCCGTGAAGGTGACCCTGAGCGAGGGCCCCCAC
841 GGATCTGGGGAGATGATGACACGTTCTCCAGTTAAGGTGACTCTGAGTGAAGGTCCTCAC
901 CACGTGGCCCTGTTCAAGGACAGCAGCCGCGAGTTCGACCTGACCAAGGAGGAGGACCTG
901 CATGTGGCCCTATTTAAAGATAGTTCTCGGGAGTTTGATCTTACCAAAGAAGAAGATCTT
961 GCCGCCCTGCGCCACGAGATCGAGCTGCGCATGCGCAAGAACGTGAAGGAGGGCTGCACC
961 GCAGCATTAAGACATGAAATAGAACTTCGAATGAGGAAAAATGTGAAAGAAGGCTGTACC

图 1

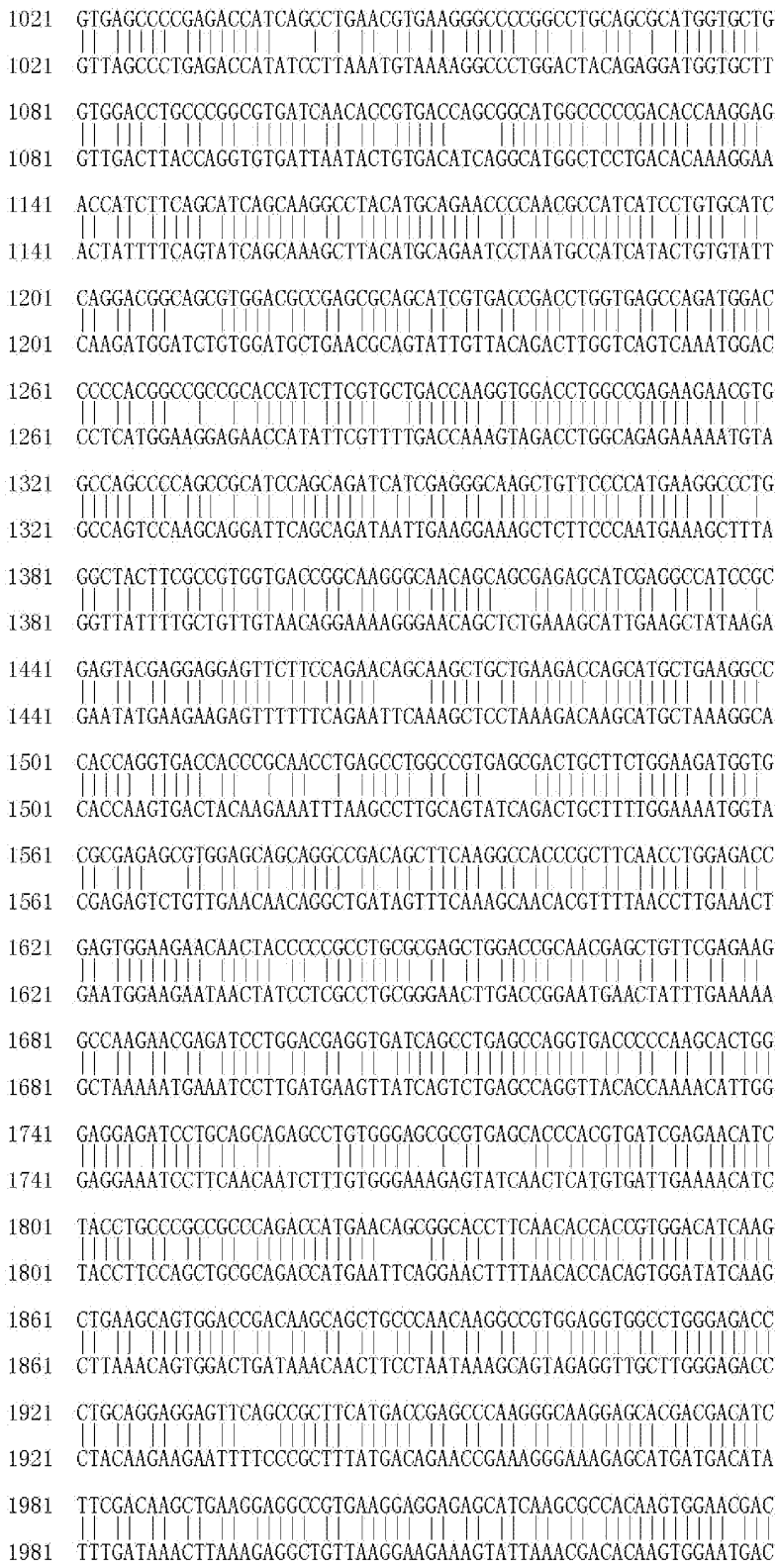


图 1(续)

```
2041  TTCGCCGAGGACAGCCTGCGCGTGATCCAGCACAACGCCCTGGAGGACCGCAGCATCAGC
      || || |||||||||| || | ||||||| |||||| ||  |||| |||||      |||
2041  TTTGCGGAGGACAGCTTGAGGGTTATTCAACACAATGCTTTGGAAGACCGATCCATATCT

2101  GACAAGCAGCAGTGGGACGCCGCCATCTACTTCATGGAGGAGGCCCTGCAGGCCCGCCTG
      || || ||||| |||||| || |||||||| || ||||| |||||| |||||||| || ||
2101  GATAAACAGCAATGGGATGCAGCTATTTATTTTATGGAAGAGGCTCTGCAGGCTCGTCTC

2161  AAGGACACCGAGAACGCCATCGAGAACATGGTGGGCCCCGACTGGAAGAAGCGCTGGCTG
      ||||| || || ||| || || |||||||||||| || |||||||| ||| |||| |||
2161  AAGGATACTGAAAATGCAATTGAAAACATGGTGGGTCCAGACTGGAAAAAGAGGTGGTTA

2221  TACTGGAAGAACCGCACCCAGGAGCAGTGCGTGCACAACGAGACCAAGAACGAGCTGGAG
      |||||||||||| || |||||| |||||||| || ||||| || |||||||| || |||||
2221  TACTGGAAGAATCGGACCCAAGAACAGTGTGTTCACAATGAAACCAAGAATGAATTGGAG

2281  AAGATGCTGAAGTGCAACGAGGAGCACCCCGCCTACCTGGCCAGCGACGAGATCACCACC
      |||||| |||| || ||| |||||||||||| || || || || |||| || ||||||
2281  AAGATGTTGAAATGTAATGAGGAGCACCCAGCTTATCTTGCAAGTGATGAAATAACCACA

2341  GTGCGCAAGAACCTGGAGAGCCGCGGCGTGGAGGTGGACCCCAGCCTGATCAAGGACACC
      || || |||||||||| ||| || || || || || || |||| |||| |||| ||||||
2341  GTCCGGAAGAACCTTGAATCCCGAGGAGTAGAAGTAGATCCAAGCTTGATTAAGGATACT

2401  TGGCACCAGGTGTACCGCCGCCACTTCCTGAAGACCGCCCTGAACCACTGCAACCTGTGC
      ||||| || || ||  | ||||  || || || || |||||||| |||||| ||
2401  TGGCATCAAGTTTATAGAAGACATTTTTTAAAAACAGCTCTAAACCATTGTAACCTTTGT

2461  CGCCGCGGCTTCTACTACTACCAGCGCCACTTCGTGGACAGCGAGCTGGAGTGCAACGAC
      ||  | || || ||||||||||  |||| || || ||  ||| |||| |||||  ||
2461  CGAAGAGGTTTTTATTACTACCAAAGGCATTTTGTAGATTCTGAGTTGGAATGCAATGAT

2521  GTGGTGCTGTTCTGGCGCATCCAGCGCATGCTGGCCATCACCGCCAACACCCTGCGCCAG
      ||||| |||| |||||||| || |||||||||||| || |||||||| |||||||  ||
2521  GTGGTCTTGTTTTGGCGTATACAGCGCATGCTTGCTATCACCGCAAATACTTTAAGGCAA

2581  CAGCTGACCAACACCGAGGTGCGCCGCCTGGAGAAGAACGTGAAGGAGGTGCTGGAGGAC
      || || || || |||  || |||  |||||| || || |||| |||||  |||| ||
2581  CAACTTACAAATACTGAAGTTAGGCGATTAGAGAAAAATGTTAAAGAGGTATTGGAAGAT

2641  TTCGCCGAGGACGGCGAGAAGAAGATCAAGCTGCTGACCGGCAAGCGCGTGCAGCTGGCC
      || || || || ||  |||||||||||  ||  |||| || || |||| || ||||||
2641  TTTGCTGAAGATGGTGAGAAGAAGATTAAATTGCTTACTGGTAAACGCGTTCAACTGGCG

2701  GAGGACCTGAAGAAGGTGCGCGAGATCCAGGAGAAGCTGGACGCCTTCATCGAGGCCCTG
      || ||||| |||||  | |||||||| || || || |||||| |||| || ||
2701  GAAGACCTCAAGAAAGTTAGAGAAATTCAAGAAAAACTTGATGCTTTCATTGAAGCTCTT

2761  CACCAGGAGAAGTAA
      || |||||||| |||
2761  CATCAGGAGAAATAA
```

(SEQ ID No.: 1 & 2)

图 1(续)

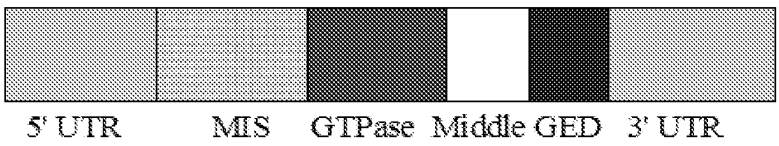


图 2

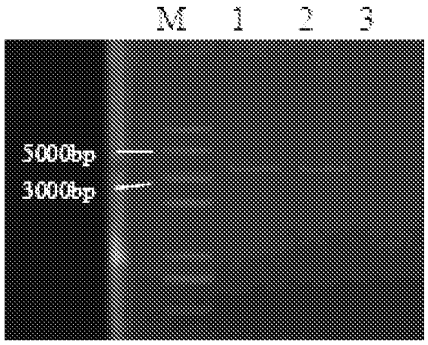

M 1 2 3
5000bp
3000bp

图 3

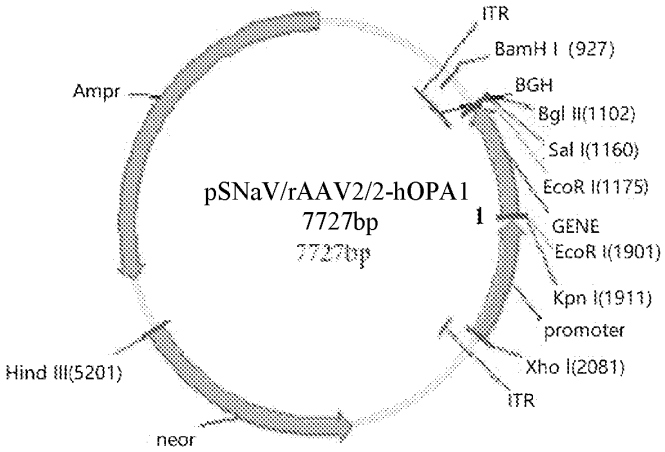

ITR
BamH I (927)
BGH
Bgl II(1102)
Sal I(1160)
EcoR I(1175)
GENE
EcoR I(1901)
Kpn I(1911)
promoter
Xho I(2081)
ITR
neor
Hind III(5201)
Ampr
pSNaV/rAAV2/2-hOPA1
7727bp
7727bp
1

图 4

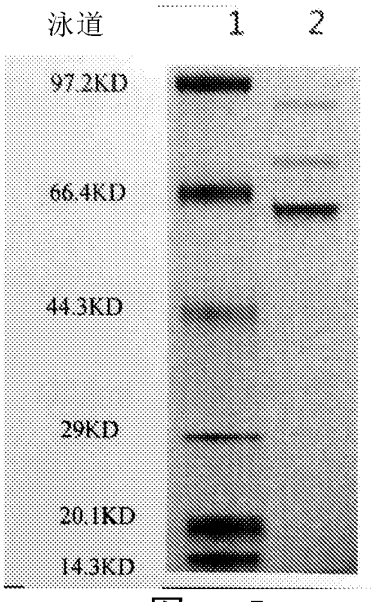

泳道 1 2
97.2KD
66.4KD
44.3KD
29KD
20.1KD
14.3KD

图 5

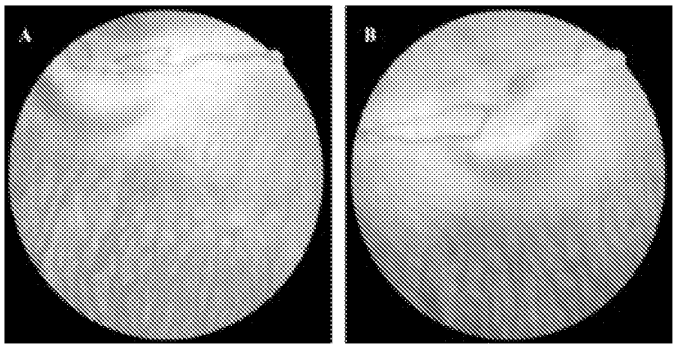


图 6

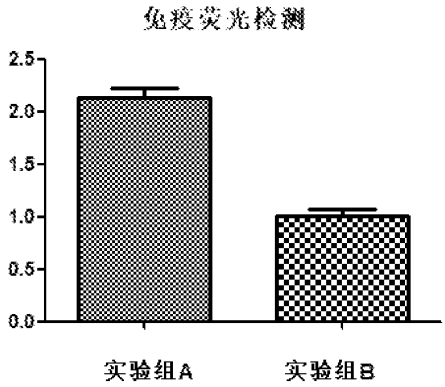


图 7

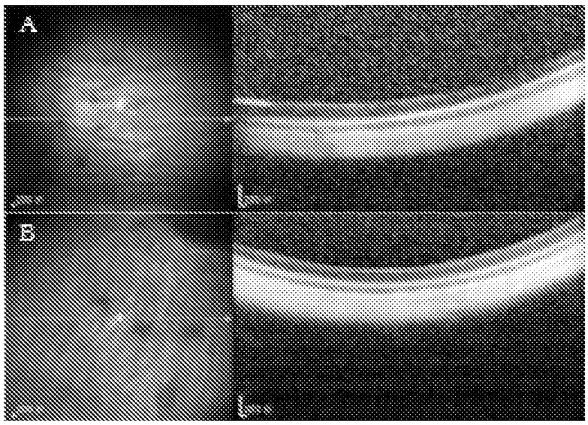


图 8

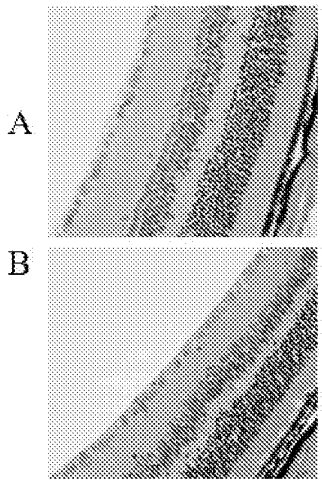

A
B

图 9

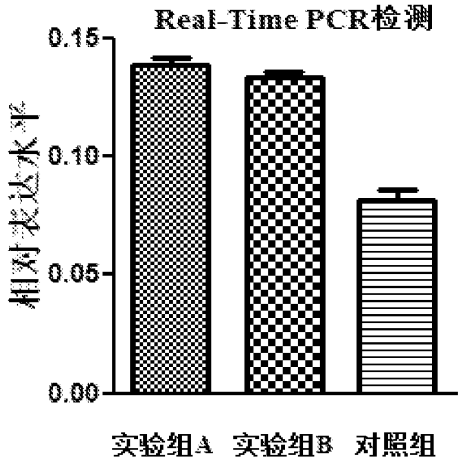

Real-Time PCR检测
相对表达水平
0.15
0.10
0.05
0.00
实验组A
实验组B
对照组

图 10

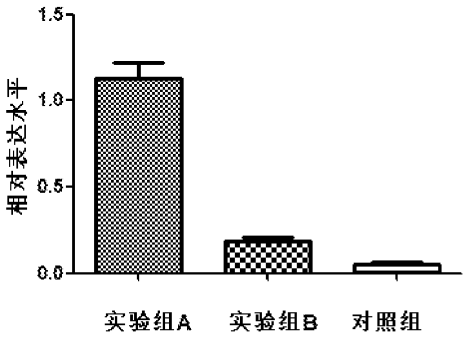

相对表达水平
1.5
1.0
0.5
0.0
实验组A
实验组B
对照组

图 11

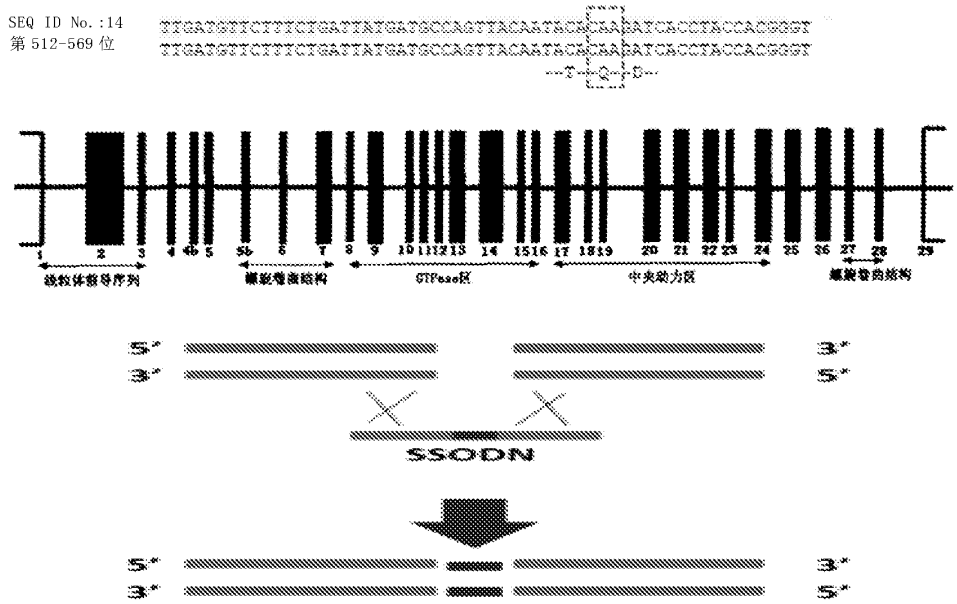
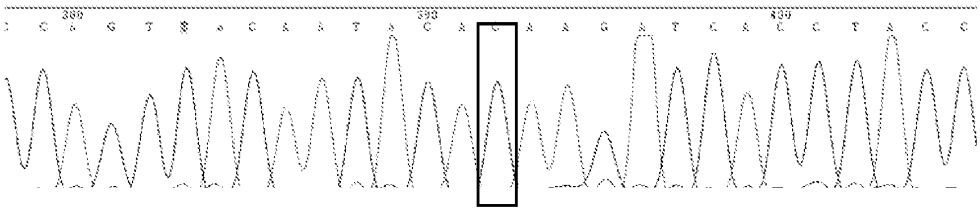


图 12

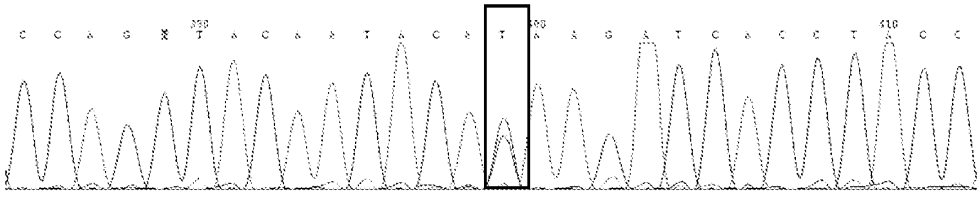
1#,2#,6#:W T



3#,4#,5#,7#:PM,Hz

ATTATGATGCCAGTTACAATACATAAGATCACCTACCACGGgtaaggggaaaat MUT

ATTATGATGCCAGTTACAATACACAAGATCACCTACCACGGgtaaggggaaaat WT



W T:野生型；PM:点突变；Hz：杂合

图 13

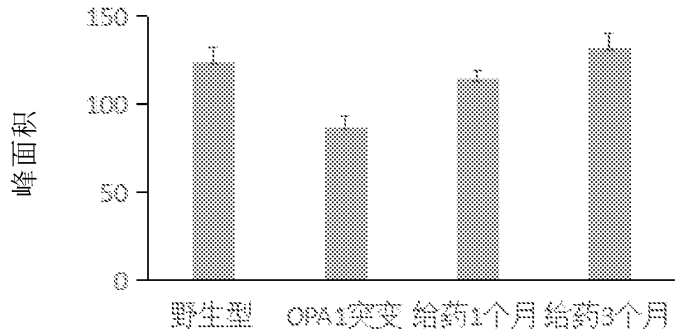


图 14

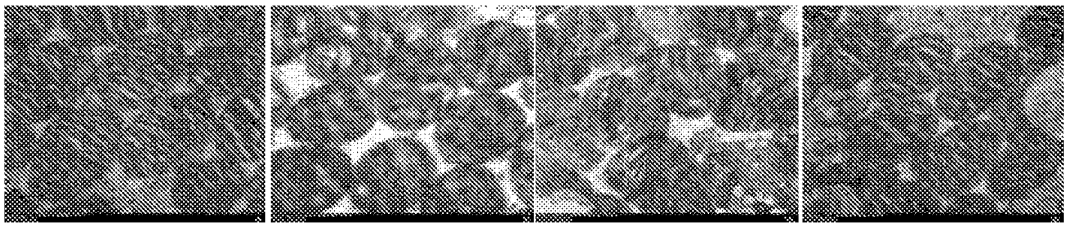


图 15