

INK2S00116R_GBA_D448V_D08_AA
10,248 bp

5'
3'

CTCTTTTCCAATCCAGGTCCC GCCCGACTCCCCAGGGCTGCTTTTCTCGCGGCTGCGGGTGGTTCGGGCTGCATCCTGCCTTCAG
GAGAAAAGGTTAGGTCCAGGGCGGGGCTGAGGGGTCCCGACGAAAAGAGCGCCGACGCCACCAGCCGACGTAGGACGGAAAGTC

85

GBA >

AGTCTTACTGCGCGGGGCCCCAGTCTCCAAGTCCC GCCCAGGGCGCCTTTGCAGGCTGCGGTGGGATTTTCGTTTTGCCTCCGGTTGG
TCAGAATGACGCGCCCCGGGGTCAGAGGTCAAGGGCGGGTCCCGCGGAAACGTCCGACGCCACCCTAAAGCAAAAACGGAGGGCCAACC

170

GBA >

GGCTGCTGTTTCTCTTCGCCGACGGTAGGGCGTAATGAATATTTTCGACCTTTGGATCTTAGCTGTCCCCTCCCTGCGTTTCGCACTT
CCGACGACAAAAGAGAAGCGGCTGCCATCCGCATTACTTATAAAGCTGGAAAACCTAGAATCGACAGGGGAGGGACGCAAGCGTGAA

255

GBA >

AACCTTTTTACCATTATTATTATTATTGTTATTATTATTTTTTTGAGGGAGTCTCGCCCTGTCGCCCAGGCTGGAGTGTAAT
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340

GBA >

GGCGCCTTCTTGGCTCACTGCAACCTCCGCCTCCCGGGTTCAGGGCATTCTCCGACCTCAGCCTCCCAAGTACGTGGGATTACAG
CCGCGGAAGAACCAGGTGACGTTGGAGGC GGAGGGCCCAAGTCCGCTAAGAGGCTGGAGTCGGAGGGTTCATGCACCCTAATGTC

425

GBA >

GCACCCGCCACCACGCACGGCTAATTTTTGTATCTTTTAGTAGAGACGGGGTTTTACCATGTTGGTCAGGCTGGTCTCCAATTC
CGTGGGCGGTGGTGCCTGCGGATTAAAAAACATAGAAAATCATCTCTGCCCCAAAGTGGTACAACCAGTCCGACCAGAGGTTAAG

510

GBA >

CTGACCTCGTGATCCGCCCGCCTCGGCCTGCCAAACAGCTGTGATTATAGGCGTGAGCCACC GCGCCCGCCAACCATCATTATT
GACTGGAGCACTAGGCGGGCGGAGCCGGACGGTTTTGTGACACTAATATCCGCACTCGGTGGCGCGGGCCGGTTGGTAGTAATAA

595

GBA >

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TAAAAATTGCCATTCTACCAGTCTAAAAATGATTACTTCTTCTCTAATATTTTAGAAGTTCAGAAAATATAGGTGAACGAAAAACT

680

GBA >

GGGGTGGAGTGGGAAGAAGGTTATGTAATTCATACGTTCTTCAGACATGTGACAAACATTCACGGAGCCCGGCGACGAGCGTCGG
CCCCACCTCACCTTCTTCCAATACATTAAGTATGCAAGAAGTCTGTACACTGTTTGTAAAGTGCTCGGGCCGCTGCTCGCAGCC

765

GBA >

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CCAACCCTAAGCGTGACCTCGACGTCTACCCACGGTCTTACCTGACCAGGGATGGGAGGCGAACTTGGATCCTCCGCCTCCAACG

850

GBA >

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935

GBA >

GGACTGGGCGCAGTGCCTCACCTGTAATCCAGCACTTTGCAAAGCCAAGGCGGGAGGATCCTTTGAGTTTAGGAGTTTGAGAC
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1020

GBA >

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1105

GBA >

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GBA

1190

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GBA

1275

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GBA

1360

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GBA

1445

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GBA

1530

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GBA

1615

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GBA

1700

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GBA

1785

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GBA

1870

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GBA

1955

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GBA

2040

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GBA

2125

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GBA

2210

AGCACTTTCGGATACCAAGGCAGCAGATCACCTGAGGTCCAGAGTTTCAGACCAGCCTGGCCAACATAGTGAAAACCCCATCTCTA
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GBA

2295

CTAAAAATAAAAAAATTAGCTGAGCATGTTGGAGGGCACCTGTAATCCCAGCTACTTGGGAGGCTGAGGCAGGAGAATCACTCGA
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GBA

2380

ACCCAGGAGGTGGAGGTTGCAGTGAGCCAAGATCACGCCACTGCACTCCAGCCTGGGCAACAGAGTGAGACTCTGTCTCAAAAAA
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GBA

2465

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GBA

2550

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GBA

2635

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GBA

2720

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GBA

2805

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GBA

2890

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GBA

2975

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GBA

3060

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GBA

3145

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GBA

3230

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GBA

3315

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3400

GBA

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3485

GBA

GBA-202

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3570

GBA

GBA-202

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3655

GBA

GBA-202

1 M E F S S P S R E
ENSE00001890492

GBA-202

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ACCTTAGGACCCTGACCTTAAATTACTTACTGTCTGAGAGAAACTTAGGTCCCAGGTAGTACCGAGAAACTCGTTCCGTGTCTACC

3740

GBA

GBA-202

GBA-202

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3825

GBA

GBA-202

GBA-202

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3910

GBA

GBA-202

GBA-202

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3995

GBA

GBA-202

10 E C P K
ENSE00003469059

GBA-202

CTTTGAGTAGGGTAAGCATCATGGCTGGCAGCCTCACAGGATTGCTTCTACTTTCAGGCAGTGTCTGTTGGGCATCAGGTGAGTGAGT
GAAACTCATCCCATTTCGTAGTAGTACCGACCGTCGGAGTGTCTTAACGAAGATGAAGTCCGTCACAGCACCCGTTAGTCCACTCACTCA

4080

GBA

GBA-202

15 20 25 30 35
P L S R V S I M A G S L T G L L L L Q A V S W A S

ENSE00003469059

GBA-202

CAAGGCAGTGGGGAGGTAGCACAGAGCCTCCCTTCTGCCTCATAGTCCTTTGGTAGCCTTCCAGTAAGCTGGTGGTAGACTTTTA
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4165

GBA

GBA-202

GBA-202

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4250

GBA

GBA-202

GBA-202

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4335

GBA

GBA-202

GBA-202

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4420

GBA

GBA-202

GBA-202

GCTGGCAAGGCAGGTCTCAAACCTCCTCACCTCAGGTGATCCGCCACCTCGGCCTCCTAAAGTGCTAGGATTACAGGTGTGAGCC
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4505

GBA

GBA-202

GBA-202

CCTGCGCCCGGCCAAGGGGTGAGGAATTTGAAACCGTGTTCAGTCTCTCCTAGCAGATGTGTCCATTCTCCATGTCTTCATCAG
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4590

GBA

GBA-202

GBA-202

ACCTCACTCTGCTTGTACTCCCTCCCTCCCAGGTGCCCGCCCTGCATCCCTAAAAAGCTTCGGCTACAGCTCGGTGGTGTGTGTC
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4675

GBA

GBA-202

G A R P C I P K S F G Y S S V V C V

ENSE00003675620

GBA-202

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ACGTTACGGTGTATGACACTGAGGAAACTGGGGGGCTGGAAAGGACGGGAACCATGGAAGTCGGCGATACTCTCATGTGCGTCAC

4760

GBA

GBA-202

C N A T Y C D S F D P P T F P A L G T F S R Y E S T R S

ENSE00003675620

GBA-202

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4845

GBA

GBA-202

G R R M E L S M G P I Q A N H T G T

ENSE00003675620

GBA-202

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4930

GBA

GBA-202

GBA-202

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5015

GBA

GBA-202

G L L L T L Q P E Q K F Q K V K G F G G A M T D A A

ENSE00003499798

GBA-202

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5100

GBA

GBA-202

A L N I L A L S P P A Q N L L K S Y F S E E

ENSE00003499798

GBA-202

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5185

GBA

GBA-202

GBA-202

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5270

GBA

GBA-202

GBA-202

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5355

GBA

GBA-202

GBA-202

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5440

GBA

GBA-202

GBA-202

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5525

GBA

GBA-202

GBA-202

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5610

GBA

GBA-202

GBA-202

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5695

GBA

GBA-202

GBA-202

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5780

GBA

GBA-202

GBA-202

CCCTGACTTCTGCCCATCCCTGACATTTGGTTTCCTGTTTTAATGCCCTGTGAAATAAGATTTACCCGCCTATCATCTGCTAACTG
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5865

GBA

GBA-202

GBA-202

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5950

GBA

GBA-202

GBA-202

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6035

GBA

GBA-202

GBA-202

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6120

GBA

GBA-202

G I G Y N I I R V P M A S C D F S I R T Y T Y A
ENSE00003618264

GBA-202

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6205

GBA

GBA-202

D T P D D F Q L H N F S L P E E D T K L K
ENSE00003618264

GBA-202

GCCCTGAGGGCCCTGATGTCTGGGGGTTGAGAACTGTAGGGTAGGTCTGCTTGTACAGACATTTTGTCCCCTGCTGTTTTGTCC
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6290

GBA

GBA-202

GBA-202

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6375

GBA

GBA-202

GBA-202

CACTACCTTTGTCTCTAGATACCCCTGATTACCCGAGCCCTGCAGTTGGCCAGCGTCCCGTTTCACTCCTTGCCAGCCCTGGA
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6460

GBA

GBA-202

I P L I H R A L Q L A Q R P V S L L A S P W

ENSE00003562842

GBA-202

CATCACCCACTTGGCTCAAGACCAATGGAGCGGTGAATGGGAAGGGGTCCTCAAGGGACAGCCCGGAGACATCTACCACCAGAC
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6545

GBA

GBA-202

T S P T W L K T N G A V N G K G S L K G Q P G D I Y H Q T

ENSE00003562842

GBA-202

CTGGGCCAGATACTTTGTGAAGTAAGGGATCAGCAAGGATGTGGGATCAGGACTGGCCTCCCATTTAGCCATGCTGATCTGTGTC
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6630

GBA

GBA-202

W A R Y F V K

ENSE00003562842

GBA-202

CCAACCCTCAACCTAGTTCCACTTCCAGATCTGCCTGTCTCAGCTCACCTTTCTACCTTCTGGGCCTTTCAGCCTTGGGCCTGT
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6715

GBA

GBA-202

GBA-202

CAATCTTGCCCACTCCATCAGGCTTCTGTTCTCTCGGTCTGGCCCACTTTCTTTTTATTTTTCTTCTTTTTTTTTTTTTTTGAGA
GTTAGAACGGGTGAGGTAGTCCGAAGGACAAGAGAGCCAGACCGGGTGAAAGAAAAATAAAAAAGAAAAAAAAAAAAAAAAAACTCT

6800

GBA

GBA-202

GBA-202

AGGAGTCTCTCTCTGTACCCAGGCTGGAGTGCTGTGGCGCCATTTCACTCACTGTAACCTCTGCCTCCTGAGTTCAAGCAA
TCCTCAGAGAGAGACAGTGGGTCCGACCTCACGACACCGCGGTAGAAGTGAGTGACATTGGAGACGGAGGACTCAAGTTCGTT

6885

GBA

GBA-202

GBA-202

TTCTCCTGCCTCAGCCTTCCAAGTAGCTGGGATTATAGGCGCCTGCCACCAGGCCAGCTGATTTTTCTATTTTTAGTAGAGACG
AAGAGGACGGAGTCGGAAGGTTTCATCGACCTAATATCCGCGGACGGTGGTCCGGGTCGACTAAAAAGATAAAAAATCATCTCTGC

6970

GBA

GBA-202

GBA-202

GGGTTTCGCCAGGCTGTTCTCGAACTCCTGAACTCAAGTGATCCACCTGCCTCGGCTTCCCAAAGTGCTGGGATTACAGGTGTGA

CCCAAAGCGGTCCGACAAGAGCTTGAGGACTTGAGTTCAGTACTAGGTGGACGGAGCCGAAGGGTTTCACGACCCTAATGTCCACACT

7055

GBA

GBA-202

GBA-202

GCCACCACACCCAGCTGGTCTGGTCCACTTTCTTGCCGGATCATTTCATGACCTTTCTCTTGCCAGGTTCTGGATGCCTATGCT

CGGTGGTGTGGGTCGACCAGACAGGTGAAAAGAACCGGCCTAGTAAGTACTGGAAAAGAGAACGGTCCAAGGACCTACGGATACGA

7140

GBA

GBA-202

255 F L D A Y A 260

ENSE00003488376

GBA-202

GAGCACAAGTTACAGTTCTGGGCAGTGACAGCTGAAAATGAGCCTTCTGCTGGGCTGTTGAGTGGATACCCCTTCCAGTGCCTGG

CTCGTGTTC AATGTCAAGACCCGTC ACTGTGCGACTTTTACTCGGAAGACGACCCGACA ACTCACCTATGGGG AAGGTCACGGACC

7225

GBA

GBA-202

E H K L Q F W A V T A E N E P S A G L L S G Y P F Q C L

ENSE00003488376

GBA-202

GCTTCACCCCTGAACATCAGCGAGACTTCATTGCCCGTGACCTAGGTCC TACCCTCGCCAACAGTACTCACCACAATGTCCGCCT

CGAAGTGGGGACTTGTAGTCGCTCTGAAGTAACGGGCACTGGATCCAGGATGGGAGCGGTTGT CATGAGTGGTGT TACAGGCGGA

7310

GBA

GBA-202

G F T P E H Q R D F I A R D L G P T L A N S T H H N V R L

ENSE00003488376

GBA-202

ACTCATGCTGGATGACCAACGCTTGCTGCTGCCCACTGGGCAAAGGTGGTAAGGCCTGGACCTCCATGGTGTCTCCAGTGACCTT

TGAGTACGACCTACTGGTTGCGAACGACGACGGGGTGACCCGTTTCCACCATTCCGGACCTGGAGGTACCACGAGGTCACTGGAA

7395

GBA

GBA-202

L M L D D Q R L L L P H W A K V

ENSE00003488376

GBA-202

CAAAATCCAGCATCCAAATGACTGGCTCCCAAACCTTAGAGCGATTTCTCTACCCA ACTATGGATTCC TAGAGCACCATTTCCCCTGG

GTTTAGGTCGTAGGTTTACTGACCGAGGGTTTGAATCTCGCTAAAGAGATGGGTTGATACCTAAGGATCTCGTGGTAAGGGGACC

7480

GBA

GBA-202

GBA-202

ACCTCCAGGGTGCCATGGATCCCACAGTTGTGCGCTTGAAACCTTTCTAGGGGCTGGGCGAGGTGGCTCACTCATGCAAACCCAGC

TGGAGGTCCACGGTACCTAGGGTGTCAACAGCGAACTTTGGAAAGATCCCCGACCCGCTCCACCGAGTGAGTACGTTTGGGTCG

7565

GBA

GBA-202

GBA-202

ACTTTGGGAAGCCGAGGCGGGTGATCACCTGAGGTCAGGAGTTTAAAGACCACCCTGGCCAACGTGTTGAAACCCTGTGTCTACTA
TGA AACCCCTTCGGCTCCGCCCCTAGTGGACTCCAGTCCTCAAATTTCTGGTGGGACCGGTTGCACAACCTTTGGGACACAGATGAT

7650

GBA

GBA-202

GBA-202

AAATACAAAAAAAAAAAAATTATCTGGGCATGATGGTGGGTGTCTGTAATCCCAGCTACTCAGGAGGCTGAGAAGGGAGAATCAGT
TTTATGTTTTTTTTTTTTAATAGACCCGTA CTACCACCCACAGACATTAGGGTCGATGAGTCCTCCGACTCTTCCCTCTTAGTCA

7735

GBA

GBA-202

GBA-202

TGAACCCGGGAGATGGTGGTTGCGGTGAGCCGAGATCGCGCCACTGCACTCCAGCCTGGGAGGCTGAGCGAGACTCCATCTCGAA
ACTTGGGCCCTCTACCACCAACGCCACTCGGCTCTAGCGCGGTGACGTGAGGTCGGACCCCTCCGACTCGCTCTGAGGTAGAGCTT

7820

GBA

GBA-202

GBA-202

ACAAAACAAAACAAAACACTATCTAGGCTGGGGGTGGTGGTTCATGTATGTATGTGTATATACATATATATGTGTTTTATATGTATAT
TGTTTTGTTTTGTTTTGATAGATCCGACCCCCACCACCAAGTACATACATACATATATGTATATATACACAAATATACATATA

7905

GBA

GBA-202

GBA-202

ATATATACACACACACATACATACACACACATACACACACAAATTAGCTGGGTGTGGCACCCGTGTAGTCCCAGCTACTCAGG
TATATATGTGTGTGTGTGTATGTATGTGTGTGTATGTGTGTGTTTAAATCGACCCACACCCGTGGGCACATCAGGGTCGATGAGTCC

7990

GBA

GBA-202

GBA-202

AGGCTAATGTGGGAGGATCAGTTGACCCTAGGAAGTCAAGGCTGCAGTGAGTCGTGATTGCGCCACTGTACTCCAGCCCGAGTGA
TCCGATTACACCCTCCTAGTCAACTGGGATCCTTCAGTTCAGCAGTCACTCAGCACTAACGCGGTGACATGAGGTCGGGCTCACT

8075

GBA

GBA-202

GBA-202

CAGAGTGACATCCTGTCTCAAAAACAAAAAAAAAATCTCCCCAACCTCTCTAGTTGCATTCTTCCCGTCACCCAACTCCAGGATT
GTCTCACTGTAGGACAGAGTTTTTTGTTTTTTTTTAGAGGGGTTTGGAGAGATCAACGTAAGAAGGGCAGTGGGTTGAGGTCCTAA

8160

GBA

GBA-202

GBA-202

CCTACAACAGGAACTAGAAAGTTCCAGAAGCCTGTGTGCAAGGTCCAGGATCAGTTGCTCTTCTCTTTCAGGTAAGTGCAGACACCA
GGATGTTGTCTTGTATCTTCAAGGTCTTCGGACACACGTTCCAGGTCCTAGTCAACGAGAAGGAAACGTCATGACTGTCTGGGT

8245

GBA

GBA-202

335
V L T D P
ENSE00001231060

GBA-202

GAAGCAGCTAAATATGTTTCATGGCATTGCTGTACATTGGTACCTGGACTTTCTGGCTCCAGCCAAAGCCACCCCTAGGGGAGACAC
CTTCGTCGATTTATACAAGTACCGTAACGACATGTAACCATGGACCTGAAAGACCCGAGGTCGGTTTTCCGGTGGGATCCCTCTGTG

8330

GBA

GBA-202

340 345 350 355 360 365
E A A K Y V H G I A V H W Y L D F L A P A K A T L G E T

ENSE00001231060

GBA-202

ACCGCCTGTTCCCAACACCATGCTCTTTGCCTCAGAGGCCTGTGTGGGCTCCAAGTTCTGGGAGCAGAGTGTGCGGCTAGGCTC
TGGCGGACAAGGGGTTGTGGTACGAGAAACGGAGTCTCCGGACACACCCGAGGTTCAAGACCCTCGTCTCACACGCCGATCCGAG

8415

GBA

GBA-202

370 375 380 385 390 395
H R L F P N T M L F A S E A C V G S K F W E Q S V R L G S

ENSE00001231060

GBA-202

CTGGGATCGAGGGATGCAGTACAGCCACAGCATCATCACGGTAAGCCACCCAGTCTCCCTTCTGCAAAGCAGACCTCAGACCT
GACCTTAGCTCCCTACGTCATGTCTGGTGTCTGCTAGTAGTGCCATTCCGGTGGGGTCAGAGGGAAGGACGTTTTCTGCTCTGGAGTCTGGA

8500

GBA

GBA-202

400 405
W D R G M Q Y S H S I I T

ENSE00001231060

GBA-202

Sanger Sequencing

GGGGAA

PCR Reverse

GGGGAA

CTTACTAGTTTTACCAAAGACTGACAGAAGCCCTTCTGTCCAGCTTTCCCCAGCTAGCCTGCCCTTTTTGAGCAACTCTGGGGAA
GAATGATCAAAGTGGTTTTCTGACTGTCTTCGGGAAGGACAGGTCGAAAGGGGTCGATCGGACGGGAAAACCTCGTTGAGACCCCTT

8585

GBA

GBA-202

GBA-202

Sanger Sequencing

CCATGATTCCCTATCTTC

PCR Reverse

CCATGATTCCCTATCTTC

CCATGATTCCCTATCTTCCCTTTCTTCCAGGCTGCACACCTCATTGCCCTTTTTGCAACTACTGAGGCACTTGCAGCTGCCT
GGTACTAAGGGATAGAAGGGAAAGGAAAGTGTCCAGACGTGTGGAGTAACGGGGAAAACGTTGATGACTCCGTGAACGTCGACGGA

8670

GBA

GBA-202

GBA-202

CAGACTTCTCAGCTCCCCTTGAGATGCCTGGATCTTCACACCCCAACTCCTTAGCTACTAAGGAATGTGCCCTCACAGGGCTG
GTCTGAAGAGTTCGAGGGGAACCTCTACGGACCTAGAAGTGTGGGGGTTGAGGAATCGATGATTCTTACACGGGGAGTGTCCCGAC

8755

GBA

GBA-202

GBA-202

ACCTACCCACAGCTGCCTCTCCACATGTGACCCCTTACCTACACTCTCTGGGGACCCCCAGTGTGGCGCTTTGTCTCTTTGCCT
TGGATGGGTGTTCGACGGAGAGGGGTGTACACTGGGAATGGATGTGAGAGACCCCTGGGGGTCACAACCGCGAAACAGAGAAACGGA

8840

GBA

GBA-202

GBA-202

Donor Template SNV -> REV

CGAAGGAGGACCCAATT

TTGTCCTTACCCTAGAACCTCCTGTACCATGTGGTCGGCTGGACCGACTGGAACCTTGCCTGAACCCCGAAGGAGGACCCAATT
AACAGGAATGGGATCTTGGAGGACATGGTACACCAGCCGACCTGGCTGACCTTGGAACGGGACTTGGGGCTTCTCTCTGGGTTAA

8925

GBA

GBA-202

410 415 420 425 430
N L L Y H V V G W T D W N L A L N P E G G P N

ENSE00003506590

GBA-202

Donor Template SNV -> REV

Donor Template SNV -> REV

GGGTGCGTAACTTTGTTCGACAGTCCCATCATTGTAGACATCACCAAGGACACGTTTTACAAACAGCCCATGTTCTACCACCTT
GGGTGCGTAACTTTGTTCGACAGTCCCATCATTGTAGACATCACCAAGGACACGTTTTACAAACAGCCCATGTTCTACCACCTTGG
CCCACGCATTGAAACAGCTGTCAGGGTAGTAACATCTGTAGTGGTTCCGTGTGCAAAATGTTTGTTCGGGTACAAGATGGTGGAAAC

9010

GBA

GBA-202

435 440 445 450 455 460
W V R N F V D S P I I V D I T K D T F Y K Q P M F Y H L G

ENSE00003506590

GBA-202

Donor Template SNV -> REV

PAM Protospacer Sequence

SNV

TCCA GTGCAAAATGTTTGTGTC
gRNA Protospacer

CCACTTCAGGTGAGTGGAGGGCGGGCACCCCATTCATACCAGGCCTATCATCTCCTACATCGGATGGCTTACATCACTCTACA
GGTGAAGTCCACTCACCTCCCGCCCGTGGGGGTAAGGTATGGTCCGGATAGTAGAGGATGTAGCCTACCGAATGTAGTGAGATGT

9095

GBA

GBA-202

H F S
ENSE00003...

GBA-202

CCACGAGGGAGCAGGAAGGTGTTTCAGGGTGGAACTCGGAAGAGGCACACCCATCCCCTTTTGCACCATGGAGGCAGGAAGTGAC
GGTGCTCCCTCGTCTTCCACAAGTCCACCTTGGAGCCTTCTCCGTGTGGGTAGGGGAAAACGTGGTACCTCCGTCCTTCACTG

9180

GBA

GBA-202

GBA-202

TAGGTAGCAACAGAAAACCCCAATGCCTGAGGCTGGACTGCGATGCAGAAAAGCAGGGTTCAGTGCCAGCAGCATGGCTCCAGGC
ATCCATCGTTGTCTTTTGGGGTTACGGACTCCGACCTGACGCTACGTCTTTTCGTCCAGTACACGGGTCTGTCGTACCGAGGTCCG

9265

GBA

GBA-202

GBA-202

CTAGAGAGCCAGGGCAGAGCCTCTGCAGGAGTTATGGGGTGGGTCCGTGGGTGGGTGACTTCTTAGATGAGGGTTTCATGGGAGG
GATCTCTCGGTCCCGTCTCGGAGACGTCTCAATACCCACCCAGGCACCCACCCACTGAAGAATCTACTCCCAAAGTACCCTCC

9350

GBA

GBA-202

GBA-202

CGGAGACGTCCTCAATACCC
PCR Forward

TACCCCGAGGGACTCTGACCATCTGTTCCACATTCAGCAAGTTTCATTCTGAGGGCTCCCAGAGAGTGGGGCTGGTTGCCAGTC
ATGGGGCTCCCTGAGACTGGTAGACAAGGGTGTAAAGTCGTTCAAGTAAGGACTCCCGAGGGTCTCTCACCCGACCAACGGTCAG

9435

GBA

GBA-202

GBA-202

K 465 I P E G 470 S Q R V G 475 L V A S
ENSE00003644399

AGAAGAACGACCTGGACGCAGTGGCACTGATGCATCCCGATGGCTCTGCTGTTGTGGTCGTGCTAAACCGGTGAGGGCAATGGTG
TCTTCTTGCTGGACCTGCGTCACCGTACTACGTAGGGCTACCGAGACGACAACACCAGCACGATTTGGCCACTCCCGTTACCAC

9520

GBA

GBA-202

GBA-202

Q 480 K N D L D 485 A V A L M 490 H P D G S 495 A V V V V 500 L N R
ENSE00003644399

AGGTCTGGGAAGTGGGCTGAAGACAGCGTTGGGGGCCTTGGCAGGATCACACTCTCAGCTTCTCCTCCCTGCTCCCTAGCTCCTC
TCCAGACCTTACCCGACTTCTGTGCAACCCCGGAACCGTCTAGTGTGAGAGTCGAAGAGGAGGGACGAGGGATCGAGGAG

9605

GBA

GBA-202

GBA-202

S S
ENSE0...

TAAGGATGTGCCTCTTACCATCAAGGATCCTGCTGTGGGCTTCTGGAGACAATCTCACCTGGCTACTCCATTCACACCTACCTG
ATTCCTACACGGAGAATGGTAGTTCCTAGGACGACACCCGAAGGACCTCTGTTAGAGTGACCGATGAGGTAAGTGTGGATGGAC

9690

GBA

GBA-202

505 510 515 520 525 530
K D V P L T I K D P A V G F L E T I S P G Y S I H T Y L

ENSE00001917720

GBA-202

TGGCGTCGCCAGTGATGGAGCAGATACTCAAGGAGGCACTGGGCTCAGCCTGGGCATTAAAGGGACAGAGTCAGCTCACACGCTG
ACCGCAGCGGTCACTACCTCGTCTATGAGTTCCCTCCGTGACCCGAGTCGGACCCGTAATTTCCCTGTCTCAGTCGAGTGTGCGAC

9775

GBA

GBA-202

535
W R R Q
ENSE00001917720
GBA-202

TCTGTGACTAAAGAGGGCACAGCAGGGCCAGTGTGAGCTTACAGCGACGTAAGCCCAGGGGCAATGGTTTTGGGTGACTCACTTTC
AGACACTGATTTCTCCCGTGTGTCGTTCCCGGTCACACTCGAATGTCGCTGCATTCCGGGTCCTCCGTTACCAAACCCACTGAGTGAAAAG

9860

GBA

GBA-202

CCCTCTAGGTGGTGCCAGGGGCTGGAGGCCCTAGAAAAAGATCAGTAAGCCCCAGTGTCCCCCAGCCCCATGCTTATGTGAA
GGGAGATCCACCACGGTCCCGGACCTCCGGGGATCTTTTTCTAGTCATTCCGGGTTCACAGGGGGGTCGGGGGTACGAATACACTT

9945

GBA

GBA-202

CATGCGCTGTGTGCTGCTTGGCTTTGGAAACTGGGCCTGGGTCCAGGCCTAGGGTGAGCTCACTGTCCGTACAAACACAAGATCAG
GTACGCGACACACGACGAACGAAACCTTTGACCCGGACCCAGGTCCGGATCCCCTCGAGTGACAGGCATGTTTGTGTTCTAGTC

10,030

GBA

GBA-202

GGCTGAGGGTAAGGAAAAAGAAGAGACTAGGAAAAGCTGGGCCCCAAACTGGAGACTGTTTGTCTTTCTGAGATGCAGAAGTGGG
CCGACTCCCATTCTTTTCTTCTCTGATCCTTTGACCCGGGTTTTGACCTCTGACAAACAGAAAGGACCTCTACGTCTTGACCC

10,115

GBA

GBA-202

CCCGTGGAGCAGCAGTGTGTCAGCATCAGGGCGGAAGCCTTAAAGCAGCAGCGGGTGTGCCAGGCACCCAGATGATTCCTATGGCA
GGGCACCTCGTCTCAGTCTGAGTCCCGCCTTCGGAATTTTCGTCGTCGCCACACGGGTCCGTGGGTCTACTAAGGATACCGT

10,200

GBA

GBA-202

CCAGCCAGGAAAAATGGCAGCTCTTAAAGGAGAAAAATGTTTGGACCCA
GGTCGGTCCCTTTTACCGTCGAGAATTTCTCTTTTACAAACTCGGGT

3'






10,248

5'

GBA

GBA-202

Feature	Location	Size	Type
✓ GBA	1 .. 10,248	10,248 bp	gene
/note	= gene ENSG00000177628 Protein coding		
GBA-204	1 .. 9832	9832 bp	prim_transcript
/note	= primary transcript ENST00000428024		
GBA-201	30 .. 10,248	10,219 bp	prim_transcript
/note	= primary transcript ENST00000327247		
GBA-213	64 .. 6487	6424 bp	prim_transcript
/note	= primary transcript ENST00000493842 protein_coding_CDS_not_defined		
GBA-209	78 .. 6136	6059 bp	prim_transcript
/note	= primary transcript ENST00000473570 protein_coding_CDS_not_defined		
GBA-207	94 .. 5065	4972 bp	prim_transcript
/note	= primary transcript ENST00000467918 protein_coding_CDS_not_defined		
GBA-203	3425 .. 10,141	6717 bp	prim_transcript
/note	= primary transcript ENST00000427500		
✓ GBA-202	3451 .. 10,248	6798 bp	prim_transcript
/note	= primary transcript ENST00000368373		
GBA-208	3457 .. 4740	1284 bp	prim_transcript
/note	= primary transcript ENST00000470104 protein_coding_CDS_not_defined		
GBA-211	3469 .. 9684	6216 bp	prim_transcript
/note	= primary transcript ENST00000484489 protein_coding_CDS_not_defined		
GBA-201	3588 .. 9705	6118 bp	CDS
▶ 11 segments = 1611 bp			
/note	= coding sequence ENSP00000314508		
/translation	= MEFSSPSRE,,ECPKPLSRVSIMAGSLTGLLLLQAVSWAS,,GARPCIPKSFYSSVVCVCNATYCDSFDPPTFPALGTFSTRYESTRSGRRMELSM GPIQANHTGT,,GLLLTLPQEQKFQVKVKGFGGAMTDAALNILALSPPAQNLLKSYFSEE,,GIGYNIIRVPMASCDFSIRTYTYADTPDDFQLH NFSLPEEDTKLK,,IPLIHRAQLAQRPVSLASPWTSPWLKTNGAVNGKSLKGGQPGDIYHQTWARYFVK,,FLDAYAEHKLQFWAVTAENEP SAGLLSGYPFQCLGFTPEHQRFIARDLGPPTLANSTHNNVRLMLDDQRLLLPHWAKV,,VLTDPAAKYVHGIAVHWYLDLFLAPAKATLGETH RLFPNTMLFASEACVGSKFWEQSVRLGSWDRGMQYSHSIIT,,NLLYHVVGWTDWNLANPEGGPNWVRNFVDSPIIVDITKDTFYKQPMFYHL 536 SSKDVPLTIKDPVGFLETISP GYSIHTYLWRRQ*		
✓ GBA-202	3588 .. 9705	6118 bp	CDS
▶ 11 segments = 1611 bp			
/note	= coding sequence ENSP00000357357		
/translation	= MEFSSPSRE,,ECPKPLSRVSIMAGSLTGLLLLQAVSWAS,,GARPCIPKSFYSSVVCVCNATYCDSFDPPTFPALGTFSTRYESTRSGRRMELSM GPIQANHTGT,,GLLLTLPQEQKFQVKVKGFGGAMTDAALNILALSPPAQNLLKSYFSEE,,GIGYNIIRVPMASCDFSIRTYTYADTPDDFQLH NFSLPEEDTKLK,,IPLIHRAQLAQRPVSLASPWTSPWLKTNGAVNGKSLKGGQPGDIYHQTWARYFVK,,FLDAYAEHKLQFWAVTAENEP SAGLLSGYPFQCLGFTPEHQRFIARDLGPPTLANSTHNNVRLMLDDQRLLLPHWAKV,,VLTDPAAKYVHGIAVHWYLDLFLAPAKATLGETH RLFPNTMLFASEACVGSKFWEQSVRLGSWDRGMQYSHSIIT,,NLLYHVVGWTDWNLANPEGGPNWVRNFVDSPIIVDITKDTFYKQPMFYHL 536 SSKDVPLTIKDPVGFLETISP GYSIHTYLWRRQ*		
GBA-203	3588 .. 9705	6118 bp	CDS
▶ 10 segments = 1464 bp			
/note	= coding sequence ENSP00000402577		
/translation	= MEFSSPSRE,,ECPKPLSRVSIMAGSLTGLLLLQAVSWAS,,GARPCIPKSFYSSVVCVCNATYCDSFDPPTFPALGTFSTRYESTRSGRRMELSM GPIQANHTGT,,GIGYNIIRVPMASCDFSIRTYTYADTPDDFQLHNFSLPEEDTKLK,,IPLIHRAQLAQRPVSLASPWTSPWLKTNGAVNGK GSLKGGQPGDIYHQTWARYFVK,,FLDAYAEHKLQFWAVTAENEPSAGLLSGYPFQCLGFTPEHQRFIARDLGPPTLANSTHNNVRLMLDDQRL LLPHWAKV,,VLTDPAAKYVHGIAVHWYLDLFLAPAKATLGETHRLFPNTMLFASEACVGSKFWEQSVRLGSWDRGMQYSHSIIT,,NLLYHVV WTDWNLANPEGGPNWVRNFVDSPIIVDITKDTFYKQPMFYHLGHFS,,KFIPEGSQRVGLVASQKNDLDAVALMHPDGSVAVVVV LNR,,SSK 481 IKDPAVGFLETISP GYSIHTYLWRRQ*		
GBA-214	4738 .. 8304	3567 bp	prim_transcript
/note	= primary transcript ENST00000497670 protein_coding_CDS_not_defined		

Feature	Location	Size		Type
GBA-204	4769 .. 9705	4937 bp	 →	CDS
▶ 9 segments = 1350 bp				
/note	= coding sequence ENSP00000397986			
/translation	= MELSMGPIQANHTGT,,GLLLTLQPEQKFQKVKGFGGAMTDAAAALNILALSPPAQNLLLKSYFSEE,,GIGYNIIRVPMASCDIFSIRTYTYADTPD DFQLHNFSLPEEDTKLK,,IPLIHRALQLAQRVSLASPWTSPWLKTNGAVNGKGSKGGQPGDIYHQTWARYFVK,,FLDAYAEHKLQFWAVT AENEPSAGLLSGYPFQCLGFTPEHQRFIARDLGPTLANSTHHNVRLMLDDQRLLPHWAKV,,VLTDPAAKYVHGIAVHWYLDFLAPAKAT LGETHRLFPNTMLFASEACVGSKFWEQSVRLGSWDRGMQYSHSIT,,NLLYHVVGWTDWNLALNPEGGPNWVRNFVDSPIIVDITKDTFYKQP MFYHLGHFS,,KFIPEGSQRVGLVASQKNDLDAVALMHPDGSVVVVV LNR,,SSKDVP LTIKDPAVGFLETISP GYSIHTYLWRRQ * 449 amino acids = 50.3 kDa			
GBA-205	5809 .. 6768	960 bp	 →	prim_transcript
/note	= primary transcript ENST00000460156 protein_coding_CDS_not_defined			
GBA-212	5991 .. 8518	2528 bp	 →	prim_transcript
/note	= primary transcript ENST00000491081 protein_coding_CDS_not_defined			
GBA-210	8241 .. 9739	1499 bp	 →	prim_transcript
/note	= primary transcript ENST00000478472 protein_coding_CDS_not_defined			
GBA-206	8865 .. 10,247	1383 bp	 →	prim_transcript
/note	= primary transcript ENST00000464536 protein_coding_CDS_not_defined			
✓ Donor Template SNV -> REV	8909 .. 9008	100 bp	 ⇌	misc_feature
✓ PAM	8968 .. 8970	3 bp	 ⇌	misc_feature
✓ Protospacer Sequence	8971 .. 8990	20 bp	 ⇌	misc_feature
✓ SNV	8974 .. 8974	1 bp	 ⇌	misc_feature
/note	= REV = A SNV = T			

Primer	Length	Binding Sites	Tm	Date Added
✓ PCR Reverse	24-mer	8580 .. 8603	59°C	Jan 10, 2023
/sequence = GGGGAACCATGATTCCCTATCTTC 50% GC / 7303.8 Da				
✓ Sanger Sequencing	24-mer	8580 .. 8603	59°C	Jan 10, 2023
/sequence = GGGGAACCATGATTCCCTATCTTC 50% GC / 7303.8 Da				
✓ Donor Template SNV -> REV	100-mer	8909 .. 9008	77°C	Jan 10, 2023
/sequence = CGAAGGAGGACCCAATTGGGTGCGTAACTTTGTGACAGTCCCATCATTGTAGACATCACCAAGGACACGTTTTACAAACAGCCCATG TTTACCAGC , 690.0 Da				
✓ gRNA Protospacer	20-mer	8975 .. 8990	48°C	Jan 10, 2023
/sequence = CTGTTTGTAAAACGTGACCT 40% GC / 6107.1 Da				
✓ PCR Forward	20-mer	9284 .. 9303	59°C	Jan 10, 2023
/sequence = CCCATAACTCCTGCAGAGGC 60% GC / 6047.0 Da				