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<110> Regeneron Pharmaceuticals, Inc.
University of Maryland, Baltimore

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uugagcccug gaccuuuugu ccuaugugug uggauuccag ggcaacucua  2000
gcaucagagc aaaagccuug gguuucucgc auucaguggc cuaucuccag  2050
auugucugau uucugaaugu aaaguuguug uguuuuuuuu uaaauaguag  2100
uuuguaguau uuuaaagaaa gaacagaucg aguucuaauu augaucuagc  2150
uugauuuugu guugauccaa auuugcauag cuguuuaaug uuaagucaug  2200
acaauuuauu uuucuuggca ugcuauguaa acuugaauuu ccuauguauu  2250
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gcuguguauu uugauuuccu guguaugcaa auguguguau uuaccauugu  4100
guagggggcu gugucugauc uugguguuca aaacagaacu guauuuuugc  4150
cuuuaaaauu aaauaauaua acgugaauaa augacccuau cuuuguaaca  4200
aaaaaaaaaa aaaa   4214
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<211> 1197
<212> DNA
<213> Homo sapien

<220>
<223> wild-type B4GALT1 cDNA sequence

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ccccaactgg tcggagtctc cacaccgctg cagggcggct cgaacagtgc 200
cgccgccatc gggcagtcct ccggggagct ccggaccgga ggggcccggc 250
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ctcattccaa tgaatgacca taatgcgtac aggtgttttt cacagccacg 800
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aatggatttc ctaataatta ttggggctgg ggaggagaag atgatgacat 950
ttttaacaga ttagttttta gaggcatgtc tatatctcgc ccaaatgctg 1000
tggtcgggag gtgtcgcatg atccgccact caagagacaa gaaaaatgaa 1050
cccaatcctc agaggtttga ccgaattgca cacacaaagg agacaatgct 1100
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acccattgta tacccaaatc acagtggaca tcgggacacc gagctag 1197

<210> 6
<211> 1197
<212> DNA
<213> Homo sapien

<220>
<223> variant B4GALT1 cDNA sequence

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ttggcgtcac cctcgtttac tacctggctg gccgcgacct gagccgcctg 150
ccccaactgg tcggagtctc cacaccgctg cagggcggct cgaacagtgc 200
cgccgccatc gggcagtcct ccggggagct ccggaccgga ggggcccggc 250
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agcccagtcg tggattctgg ccctggcccc gctagcaact tgacctcggt 350

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tccgcaaccg gcaggagcac ctcaagtact ggctatatta tttgcaccca  600
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gcacatttcc gttgcaatgg ataagtttgg attcagccta ccttatgttc  850
agtattttgg aggtgtctct gctctaagta aacaacagtt tctaaccatc  900
aatggatttc ctaataatta ttggggctgg ggaggagaag atgatgacat  950
ttttaacaga ttagttttta gaggcatgtc tatatctcgc ccaaatgctg  1000
tggtcgggag gtgtcgcatg atccgccact caagagacaa gaaaaatgaa  1050
cccagtcctc agaggtttga ccgaattgca cacacaaagg agacaatgct  1100
ctctgatggt ttgaactcac tcacctacca ggtgctggat gtacagagat  1150
acccattgta tacccaaatc acagtggaca tcgggacacc gagctag  1197
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<210> 7
<211> 398
<212> PRT
<213> Homo sapien

<220>
<223> wild-type B4GALT1 sequence

<400> 7

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Ala Ser Leu Gln Arg Ala Cys Arg Leu Leu Val Ala Val Cys Ala Leu
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His Leu Gly Val Thr Leu Val Tyr Tyr Leu Ala Gly Arg Asp Leu Ser
        35                  40                  45

Arg Leu Pro Gln Leu Val Gly Val Ser Thr Pro Leu Gln Gly Gly Ser
    50                  55                  60

Asn Ser Ala Ala Ala Ile Gly Gln Ser Ser Gly Glu Leu Arg Thr Gly
65                  70                  75                  80

Gly Ala Arg Pro Pro Pro Pro Leu Gly Ala Ser Ser Gln Pro Arg Pro
                85                  90                  95

Gly Gly Asp Ser Ser Pro Val Val Asp Ser Gly Pro Gly Pro Ala Ser
            100                 105                 110

Asn Leu Thr Ser Val Pro Val Pro His Thr Thr Ala Leu Ser Leu Pro
        115                 120                 125
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Ala Cys Pro Glu Glu Ser Pro Leu Leu Val Gly Pro Met Leu Ile Glu
    130                 135                 140

Phe Asn Met Pro Val Asp Leu Glu Leu Val Ala Lys Gln Asn Pro Asn
145                 150                 155                 160

Val Lys Met Gly Gly Arg Tyr Ala Pro Arg Asp Cys Val Ser Pro His
                165                 170                 175

Lys Val Ala Ile Ile Ile Pro Phe Arg Asn Arg Gln Glu His Leu Lys
            180                 185                 190

Tyr Trp Leu Tyr Tyr Leu His Pro Val Leu Gln Arg Gln Gln Leu Asp
        195                 200                 205

Tyr Gly Ile Tyr Val Ile Asn Gln Ala Gly Asp Thr Ile Phe Asn Arg
    210                 215                 220

Ala Lys Leu Leu Asn Val Gly Phe Gln Glu Ala Leu Lys Asp Tyr Asp
225                 230                 235                 240

Tyr Thr Cys Phe Val Phe Ser Asp Val Asp Leu Ile Pro Met Asn Asp
                245                 250                 255

His Asn Ala Tyr Arg Cys Phe Ser Gln Pro Arg His Ile Ser Val Ala
            260                 265                 270

Met Asp Lys Phe Gly Phe Ser Leu Pro Tyr Val Gln Tyr Phe Gly Gly
        275                 280                 285

Val Ser Ala Leu Ser Lys Gln Gln Phe Leu Thr Ile Asn Gly Phe Pro
    290                 295                 300

Asn Asn Tyr Trp Gly Trp Gly Gly Glu Asp Asp Asp Ile Phe Asn Arg
305                 310                 315                 320

Leu Val Phe Arg Gly Met Ser Ile Ser Arg Pro Asn Ala Val Val Gly
                325                 330                 335

Arg Cys Arg Met Ile Arg His Ser Arg Asp Lys Lys Asn Glu Pro Asn
            340                 345                 350

Pro Gln Arg Phe Asp Arg Ile Ala His Thr Lys Glu Thr Met Leu Ser
        355                 360                 365

Asp Gly Leu Asn Ser Leu Thr Tyr Gln Val Leu Asp Val Gln Arg Tyr
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Pro Leu Tyr Thr Gln Ile Thr Val Asp Ile Gly Thr Pro Ser
385                 390                 395
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<210> 8
<211> 398
<212> PRT
<213> Homo sapien

<220>
<223> variant B4GALT1 sequence

<400> 8

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Ala Ser Leu Gln Arg Ala Cys Arg Leu Leu Val Ala Val Cys Ala Leu
            20                  25                  30

His Leu Gly Val Thr Leu Val Tyr Tyr Leu Ala Gly Arg Asp Leu Ser
        35                  40                  45

Arg Leu Pro Gln Leu Val Gly Val Ser Thr Pro Leu Gln Gly Gly Ser
    50                  55                  60

Asn Ser Ala Ala Ala Ile Gly Gln Ser Ser Gly Glu Leu Arg Thr Gly
65                  70                  75                  80

Gly Ala Arg Pro Pro Pro Pro Leu Gly Ala Ser Ser Gln Pro Arg Pro
                85                  90                  95

Gly Gly Asp Ser Ser Pro Val Val Asp Ser Gly Pro Gly Pro Ala Ser
            100                 105                 110

Asn Leu Thr Ser Val Pro Val Pro His Thr Thr Ala Leu Ser Leu Pro
        115                 120                 125

Ala Cys Pro Glu Glu Ser Pro Leu Leu Val Gly Pro Met Leu Ile Glu
    130                 135                 140

Phe Asn Met Pro Val Asp Leu Glu Leu Val Ala Lys Gln Asn Pro Asn
145                 150                 155                 160

Val Lys Met Gly Gly Arg Tyr Ala Pro Arg Asp Cys Val Ser Pro His
                165                 170                 175

Lys Val Ala Ile Ile Ile Pro Phe Arg Asn Arg Gln Glu His Leu Lys
            180                 185                 190

Tyr Trp Leu Tyr Tyr Leu His Pro Val Leu Gln Arg Gln Gln Leu Asp
        195                 200                 205

Tyr Gly Ile Tyr Val Ile Asn Gln Ala Gly Asp Thr Ile Phe Asn Arg
    210                 215                 220

Ala Lys Leu Leu Asn Val Gly Phe Gln Glu Ala Leu Lys Asp Tyr Asp
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225 230 235 240

Tyr Thr Cys Phe Val Phe Ser Asp Val Asp Leu Ile Pro Met Asn Asp
245 250 255

His Asn Ala Tyr Arg Cys Phe Ser Gln Pro Arg His Ile Ser Val Ala
260 265 270

Met Asp Lys Phe Gly Phe Ser Leu Pro Tyr Val Gln Tyr Phe Gly Gly
275 280 285

Val Ser Ala Leu Ser Lys Gln Gln Phe Leu Thr Ile Asn Gly Phe Pro
290 295 300

Asn Asn Tyr Trp Gly Trp Gly Gly Glu Asp Asp Asp Ile Phe Asn Arg
305 310 315 320

Leu Val Phe Arg Gly Met Ser Ile Ser Arg Pro Asn Ala Val Val Gly
325 330 335

Arg Cys Arg Met Ile Arg His Ser Arg Asp Lys Lys Asn Glu Pro Ser
340 345 350

Pro Gln Arg Phe Asp Arg Ile Ala His Thr Lys Glu Thr Met Leu Ser
355 360 365

Asp Gly Leu Asn Ser Leu Thr Tyr Gln Val Leu Asp Val Gln Arg Tyr
370 375 380

Pro Leu Tyr Thr Gln Ile Thr Val Asp Ile Gly Thr Pro Ser
385 390 395

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<211> 20
<212> DNA
<213> Artificial Sequence

<220>
<223> guide RNA recognition sequences

<400> 9
attagttttt agaggcatgt 20

<210> 10
<211> 20
<212> DNA
<213> Artificial Sequence

<220>
<223> guide RNA recognition sequences

<400> 10
ggctctcagg ccaagtgtat 20

<210> 11
<211> 20
<212> DNA
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<220>
<223> guide RNA recognition sequences

<400> 11
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<210> 12
<211> 20
<212> DNA
<213> Artificial Sequence

<220>
<223> guide RNA recognition sequences

<400> 12
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<210> 13
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<222> (1) .. (2)

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<221> r is A or G
<222> (4) .. (5)

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<210> 14
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<220>
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<222> (1) .. (2)

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<222> (4) .. (5)

<400> 14
nngrr 5

<210> 15
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<212> DNA
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<220>
<223> target motif preceding NGG recognized by Cas9 protein

<220>
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<222> (2) .. (21)

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<210> 16
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<212> DNA
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<220>
<223> target motif preceding NGG recognized by Cas9 protein

<220>
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<222> (1) .. (21)

<400> 16
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<210> 17
<211> 25
<212> DNA

<213> Artificial Sequence

<220>
<223> RNA recognition sequence

<220>
<221> n is A, G, C, or T
<222> (3) .. (23)

<400> 17
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