SEQUENCE LISTING

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<120> Arimoclomol for the treatment of Niemann Pick disease, type C, in patients with ER type missense mutations

<130> P5898PC00

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<170> PatentIn version 3.5

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Lys Pro Leu Pro Lys Asp Gly Tyr Asp Leu Val Gln Glu Leu Cys Pro
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Gly Phe Phe Phe Gly Asn Val Ser Leu Cys Cys Asp Val Arg Gln Leu
65                  70                  75                  80

Gln Thr Leu Lys Asp Asn Leu Gln Leu Pro Leu Gln Phe Leu Ser Arg
                85                  90                  95

Cys Pro Ser Cys Phe Tyr Asn Leu Leu Asn Leu Phe Cys Glu Leu Thr
            100                 105                 110

Cys Ser Pro Arg Gln Ser Gln Phe Leu Asn Val Thr Ala Thr Glu Asp
        115                 120                 125

Tyr Val Asp Pro Val Thr Asn Gln Thr Lys Thr Asn Val Lys Glu Leu
    130                 135                 140

Gln Tyr Tyr Val Gly Gln Ser Phe Ala Asn Ala Met Tyr Asn Ala Cys
145                 150                 155                 160

Arg Asp Val Glu Ala Pro Ser Ser Asn Asp Lys Ala Leu Gly Leu Leu
                165                 170                 175

Cys Gly Lys Asp Ala Asp Ala Cys Asn Ala Thr Asn Trp Ile Glu Tyr
            180                 185                 190

Met Phe Asn Lys Asp Asn Gly Gln Ala Pro Phe Thr Ile Thr Pro Val
        195                 200                 205

Phe Ser Asp Phe Pro Val His Gly Met Glu Pro Met Asn Asn Ala Thr
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Lys Gly Cys Asp Glu Ser Val Asp Glu Val Thr Ala Pro Cys Ser Cys
225                 230                 235                 240

Gln Asp Cys Ser Ile Val Cys Gly Pro Lys Pro Gln Pro Pro Pro Pro
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Pro Ala Pro Trp Thr Ile Leu Gly Leu Asp Ala Met Tyr Val Ile Met
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Trp Ile Thr Tyr Met Ala Phe Leu Leu Val Phe Phe Gly Ala Phe Phe
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Ala Ser Cys Cys Asp Pro Val Ser Ala Ala Phe Glu Gly Cys Leu Arg
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Arg Leu Phe Thr Arg Trp Gly Ser Phe Cys Val Arg Asn Pro Gly Cys
            340                 345                 350

Val Ile Phe Phe Ser Leu Val Phe Ile Thr Ala Cys Ser Ser Gly Leu
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Val Phe Val Arg Val Thr Thr Asn Pro Val Asp Leu Trp Ser Ala Pro
    370                 375                 380

Ser Ser Gln Ala Arg Leu Glu Lys Glu Tyr Phe Asp Gln His Phe Gly
385                 390                 395                 400

Pro Phe Phe Arg Thr Glu Gln Leu Ile Ile Arg Ala Pro Leu Thr Asp
                405                 410                 415

Lys His Ile Tyr Gln Pro Tyr Pro Ser Gly Ala Asp Val Pro Phe Gly
            420                 425                 430

Pro Pro Leu Asp Ile Gln Ile Leu His Gln Val Leu Asp Leu Gln Ile
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Ala Ile Glu Asn Ile Thr Ala Ser Tyr Asp Asn Glu Thr Val Thr Leu
    450                 455                 460

Gln Asp Ile Cys Leu Ala Pro Leu Ser Pro Tyr Asn Thr Asn Cys Thr
465                 470                 475                 480

Ile Leu Ser Val Leu Asn Tyr Phe Gln Asn Ser His Ser Val Leu Asp
                485                 490                 495

His Lys Lys Gly Asp Asp Phe Phe Val Tyr Ala Asp Tyr His Thr His
            500                 505                 510

Phe Leu Tyr Cys Val Arg Ala Pro Ala Ser Leu Asn Asp Thr Ser Leu
        515                 520                 525

Leu His Asp Pro Cys Leu Gly Thr Phe Gly Gly Pro Val Phe Pro Trp
    530                 535                 540

Leu Val Leu Gly Gly Tyr Asp Asp Gln Asn Tyr Asn Asn Ala Thr Ala
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Leu Val Ile Thr Phe Pro Val Asn Asn Tyr Tyr Asn Asp Thr Glu Lys
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Leu Gln Arg Ala Gln Ala Trp Glu Lys Glu Phe Ile Asn Phe Val Lys
            580                 585                 590

Asn Tyr Lys Asn Pro Asn Leu Thr Ile Ser Phe Thr Ala Glu Arg Ser
        595                 600                 605

Ile Glu Asp Glu Leu Asn Arg Glu Ser Asp Ser Asp Val Phe Thr Val
    610                 615                 620

Val Ile Ser Tyr Ala Ile Met Phe Leu Tyr Ile Ser Leu Ala Leu Gly
625                 630                 635                 640

His Met Lys Ser Cys Arg Arg Leu Leu Val Asp Ser Lys Val Ser Leu
                645                 650                 655

Gly Ile Ala Gly Ile Leu Ile Val Leu Ser Ser Val Ala Cys Ser Leu
            660                 665                 670

Gly Val Phe Ser Tyr Ile Gly Leu Pro Leu Thr Leu Ile Val Ile Glu
        675                 680                 685

Val Ile Pro Phe Leu Val Leu Ala Val Gly Val Asp Asn Ile Phe Ile
    690                 695                 700

Leu Val Gln Ala Tyr Gln Arg Asp Glu Arg Leu Gln Gly Glu Thr Leu
705                 710                 715                 720

Asp Gln Gln Leu Gly Arg Val Leu Gly Glu Val Ala Pro Ser Met Phe
                725                 730                 735

Leu Ser Ser Phe Ser Glu Thr Val Ala Phe Phe Leu Gly Ala Leu Ser
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Val Met Pro Ala Val His Thr Phe Ser Leu Phe Ala Gly Leu Ala Val
        755                 760                 765

Phe Ile Asp Phe Leu Leu Gln Ile Thr Cys Phe Val Ser Leu Leu Gly
    770                 775                 780

Leu Asp Ile Lys Arg Gln Glu Lys Asn Arg Leu Asp Ile Phe Cys Cys
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Val Arg Gly Ala Glu Asp Gly Thr Ser Val Gln Ala Ser Glu Ser Cys
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Trp Met Arg Pro Ile Val Ile Ala Ile Phe Val Gly Val Leu Ser Phe
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Ser Ile Ala Val Leu Asn Lys Val Asp Ile Gly Leu Asp Gln Ser Leu
    850                 855                 860

Ser Met Pro Asp Asp Ser Tyr Met Val Asp Tyr Phe Lys Ser Ile Ser
865                 870                 875                 880

Gln Tyr Leu His Ala Gly Pro Pro Val Tyr Phe Val Leu Glu Glu Gly
                885                 890                 895

His Asp Tyr Thr Ser Ser Lys Gly Gln Asn Met Val Cys Gly Gly Met
            900                 905                 910

Gly Cys Asn Asn Asp Ser Leu Val Gln Gln Ile Phe Asn Ala Ala Gln
        915                 920                 925

Leu Asp Asn Tyr Thr Arg Ile Gly Phe Ala Pro Ser Ser Trp Ile Asp
    930                 935                 940

Asp Tyr Phe Asp Trp Val Lys Pro Gln Ser Ser Cys Cys Arg Val Asp
945                 950                 955                 960

Asn Ile Thr Asp Gln Phe Cys Asn Ala Ser Val Val Asp Pro Ala Cys
                965                 970                 975

Val Arg Cys Arg Pro Leu Thr Pro Glu Gly Lys Gln Arg Pro Gln Gly
            980                 985                 990

Gly Asp Phe Met Arg Phe Leu Pro  Met Phe Leu Ser Asp  Asn Pro Asn
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Pro Lys  Cys Gly Lys Gly Gly  His Ala Ala Tyr Ser  Ser Ala Val
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Asn Ile  Leu Leu Gly His Gly  Thr Arg Val Gly Ala  Thr Tyr Phe
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Met Thr  Tyr His Thr Val Leu  Gln Thr Ser Ala Asp  Phe Ile Asp
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Ile Phe  Asn Leu Gly Val Ser  Leu Gly Ala Ile Phe  Leu Val Thr
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Met Val  Leu Leu Gly Cys Glu  Leu Trp Ser Ala Val  Ile Met Cys
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Ala Thr  Ile Ala Met Val Leu  Val Asn Met Phe Gly  Val Met Trp
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Leu Trp  Gly Ile Ser Leu Asn  Ala Val Ser Leu Val  Asn Leu Val
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Met Ser  Cys Gly Ile Ser Val  Glu Phe Cys Ser His  Ile Thr Arg
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Ala Phe  Thr Val Ser Met Lys  Gly Ser Arg Val Glu  Arg Ala Glu
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Glu Ala  Leu Ala His Met Gly  Ser Ser Val Phe Ser  Gly Ile Thr
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Leu Thr  Lys Phe Gly Gly Ile  Val Val Leu Ala Phe  Ala Lys Ser
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Leu Leu  Gly Ala Thr His Gly  Leu Ile Phe Leu Pro  Val Leu Leu
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Ser Tyr  Ile Gly Pro Ser Val  Asn Lys Ala Lys Ser  Cys Ala Thr
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Glu Glu  Arg Tyr Lys Gly Thr  Glu Arg Glu Arg Leu  Leu Asn Phe
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