

SEQUENCE LISTING

<110> ORPHAN TECHNOLOGIES, LTD.
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<120> DRUG PRODUCT FOR ENZYME THERAPY FOR TREATMENT OF HOMOCYSTINURIA

<130> 2089.1005PCT

<140> PCT/USXXXX/XXXXXX
 <141> 2020-06-26

<150> 62/983,860
 <151> 2020-03-02

<150> 62/866,810
 <151> 2019-06-26

<160> 5

<170> PatentIn version 3.5

<210> 1
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 <212> PRT
 <213> Homo sapiens

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 Glu Asp Lys Glu Ala Lys Glu Pro Leu Trp Ile Arg Pro Asp Ala Pro
 35 40 45
 Ser Arg Cys Thr Trp Gln Leu Gly Arg Pro Ala Ser Glu Ser Pro His
 50 55 60
 His His Thr Ala Pro Ala Lys Ser Pro Lys Ile Leu Pro Asp Ile Leu
 65 70 75 80
 Lys Lys Ile Gly Asp Thr Pro Met Val Arg Ile Asn Lys Ile Gly Lys
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Lys Phe Gly Leu Lys Cys Glu Leu Leu Ala Lys Cys Glu Phe Phe Asn
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Ala Gly Gly Ser Val Lys Asp Arg Ile Ser Leu Arg Met Ile Glu Asp
115 120 125

Ala Glu Arg Asp Gly Thr Leu Lys Pro Gly Asp Thr Ile Ile Glu Pro
130 135 140

Thr Ser Gly Asn Thr Gly Ile Gly Leu Ala Leu Ala Ala Ala Val Arg
145 150 155 160

Gly Tyr Arg Cys Ile Ile Val Met Pro Glu Lys Met Ser Ser Glu Lys
165 170 175

Val Asp Val Leu Arg Ala Leu Gly Ala Glu Ile Val Arg Thr Pro Thr
180 185 190

Asn Ala Arg Phe Asp Ser Pro Glu Ser His Val Gly Val Ala Trp Arg
195 200 205

Leu Lys Asn Glu Ile Pro Asn Ser His Ile Leu Asp Gln Tyr Arg Asn
210 215 220

Ala Ser Asn Pro Leu Ala His Tyr Asp Thr Thr Ala Asp Glu Ile Leu
225 230 235 240

Gln Gln Cys Asp Gly Lys Leu Asp Met Leu Val Ala Ser Val Gly Thr
245 250 255

Gly Gly Thr Ile Thr Gly Ile Ala Arg Lys Leu Lys Glu Lys Cys Pro
260 265 270

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

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 Leu Ile Ala Gln Glu Gly Leu Leu Cys Gly Gly Ser Ala Gly Ser Thr
 340 345 350
 Val Ala Val Ala Val Lys Ala Ala Gln Glu Leu Gln Glu Gly Gln Arg
 355 360 365
 Cys Val Val Ile Leu Pro Asp Ser Val Arg Asn Tyr Met Thr Lys Phe
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 Leu Ser Asp Arg Trp Met Leu Gln Lys Gly Phe Leu Lys Glu Glu Asp
 385 390 395 400
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 Pro Glu Asp Lys Glu Ala Lys Glu Pro Leu Trp Ile Arg Pro Asp Ala
 35 40 45
 Pro Ser Arg Cys Thr Trp Gln Leu Gly Arg Pro Ala Ser Glu Ser Pro
 50 55 60
 His His His Thr Ala Pro Ala Lys Ser Pro Lys Ile Leu Pro Asp Ile
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Lys Lys Phe Gly Leu Lys Cys Glu Leu Leu Ala Lys Cys Glu Phe Phe
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Asn Ala Gly Gly Ser Val Lys Asp Arg Ile Ser Leu Arg Met Ile Glu
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Asp Ala Glu Arg Asp Gly Thr Leu Lys Pro Gly Asp Thr Ile Ile Glu
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Pro Thr Ser Gly Asn Thr Gly Ile Gly Leu Ala Leu Ala Ala Ala Val
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Arg Gly Tyr Arg Cys Ile Ile Val Met Pro Glu Lys Met Ser Ser Glu
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Lys Val Asp Val Leu Arg Ala Leu Gly Ala Glu Ile Val Arg Thr Pro
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Thr Asn Ala Arg Phe Asp Ser Pro Glu Ser His Val Gly Val Ala Trp
 195 200 205

Arg Leu Lys Asn Glu Ile Pro Asn Ser His Ile Leu Asp Gln Tyr Arg
 210 215 220

Asn Ala Ser Asn Pro Leu Ala His Tyr Asp Thr Thr Ala Asp Glu Ile
 225 230 235 240

Leu Gln Gln Cys Asp Gly Lys Leu Asp Met Leu Val Ala Ser Val Gly
 245 250 255

Thr Gly Gly Thr Ile Thr Gly Ile Ala Arg Lys Leu Lys Glu Lys Cys
 260 265 270

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

Thr Val Ala Val Ala Val Lys Ala Ala Gln Glu Leu Gln Glu Gly Gln
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Arg Cys Val Val Ile Leu Pro Asp Ser Val Arg Asn Tyr Met Thr Lys
370 375 380

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

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Ala Gln Glu Arg Asp Gln Lys
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<212> DNA

<213> Homo sapiens

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 <213> Artificial Sequence

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 His Arg Ser Gly Pro His Ser Ala Lys Gly Ser Leu Glu Lys Gly Ser
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 Pro Glu Asp Lys Glu Ala Lys Glu Pro Leu Trp Ile Arg Pro Asp Ala
 35 40 45

 Pro Ser Arg Cys Thr Trp Gln Leu Gly Arg Pro Ala Ser Glu Ser Pro
 50 55 60

 His His His Thr Ala Pro Ala Lys Ser Pro Lys Ile Leu Pro Asp Ile
 65 70 75 80

 Leu Lys Lys Ile Gly Asp Thr Pro Met Val Arg Ile Asn Lys Ile Gly
 85 90 95

Lys Lys Phe Gly Leu Lys Cys Glu Leu Leu Ala Lys Cys Glu Phe Phe
100 105 110

Asn Ala Gly Gly Ser Val Lys Asp Arg Ile Ser Leu Arg Met Ile Glu
115 120 125

Asp Ala Glu Arg Asp Gly Thr Leu Lys Pro Gly Asp Thr Ile Ile Glu
130 135 140

Pro Thr Ser Gly Asn Thr Gly Ile Gly Leu Ala Leu Ala Ala Ala Val
145 150 155 160

Arg Gly Tyr Arg Cys Ile Ile Val Met Pro Glu Lys Met Ser Ser Glu
165 170 175

Lys Val Asp Val Leu Arg Ala Leu Gly Ala Glu Ile Val Arg Thr Pro
180 185 190

Thr Asn Ala Arg Phe Asp Ser Pro Glu Ser His Val Gly Val Ala Trp
195 200 205

Arg Leu Lys Asn Glu Ile Pro Asn Ser His Ile Leu Asp Gln Tyr Arg
210 215 220

Asn Ala Ser Asn Pro Leu Ala His Tyr Asp Thr Thr Ala Asp Glu Ile
225 230 235 240

Leu Gln Gln Cys Asp Gly Lys Leu Asp Met Leu Val Ala Ser Val Gly
245 250 255

Thr Gly Gly Thr Ile Thr Gly Ile Ala Arg Lys Leu Lys Glu Lys Cys
260 265 270

Pro Gly Cys Arg Ile Ile Gly Val Asp Pro Glu Gly Ser Ile Leu Ala
275 280 285

Glu Pro Glu Glu Leu Asn Gln Thr Glu Gln Thr Thr Tyr Glu Val Glu
290 295 300

Gly Ile Gly Tyr Asp Phe Ile Pro Thr Val Leu Asp Arg Thr Val Val
305 310 315 320

Asp Lys Trp Phe Lys Ser Asn Asp Glu Glu Ala Phe Thr Phe Ala Arg
325 330 335

Met Leu Ile Ala Gln Glu Gly Leu Leu Cys Gly Gly Ser Ala Gly Ser
340 345 350

Thr Val Ala Val Ala Val Lys Ala Ala Gln Glu Leu Gln Glu Gly Gln
355 360 365

Arg Cys Val Val Ile Leu Pro Asp Ser Val Arg Asn Tyr Met Thr Lys
370 375 380

Phe Leu Ser Asp Arg Trp Met Leu Gln Lys Gly Phe Leu Lys Glu Glu
385 390 395 400

Asp Leu Thr Glu Lys Lys Pro Trp Trp Trp His Leu Arg
405 410

<210> 5

<211> 413

<212> PRT

<213> Artificial Sequence

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<223> Description of Artificial Sequence: Synthetic
Truncated human C15S mutant CBS polypeptide

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His Arg Ser Gly Pro His Ser Ala Lys Gly Ser Leu Glu Lys Gly Ser
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Pro Glu Asp Lys Glu Ala Lys Glu Pro Leu Trp Ile Arg Pro Asp Ala
35 40 45

Pro Ser Arg Cys Thr Trp Gln Leu Gly Arg Pro Ala Ser Glu Ser Pro
50 55 60

His His His Thr Ala Pro Ala Lys Ser Pro Lys Ile Leu Pro Asp Ile
65 70 75 80

Leu Lys Lys Ile Gly Asp Thr Pro Met Val Arg Ile Asn Lys Ile Gly
85 90 95

Lys Lys Phe Gly Leu Lys Cys Glu Leu Leu Ala Lys Cys Glu Phe Phe
100 105 110

Asn Ala Gly Gly Ser Val Lys Asp Arg Ile Ser Leu Arg Met Ile Glu
115 120 125

Asp Ala Glu Arg Asp Gly Thr Leu Lys Pro Gly Asp Thr Ile Ile Glu
130 135 140

Pro Thr Ser Gly Asn Thr Gly Ile Gly Leu Ala Leu Ala Ala Val
145 150 155 160

Arg Gly Tyr Arg Cys Ile Ile Val Met Pro Glu Lys Met Ser Ser Glu
165 170 175

Lys Val Asp Val Leu Arg Ala Leu Gly Ala Glu Ile Val Arg Thr Pro
180 185 190

Thr Asn Ala Arg Phe Asp Ser Pro Glu Ser His Val Gly Val Ala Trp
195 200 205

Arg Leu Lys Asn Glu Ile Pro Asn Ser His Ile Leu Asp Gln Tyr Arg
210 215 220

Asn Ala Ser Asn Pro Leu Ala His Tyr Asp Thr Thr Ala Asp Glu Ile
225 230 235 240

Leu Gln Gln Cys Asp Gly Lys Leu Asp Met Leu Val Ala Ser Val Gly
245 250 255

Thr Gly Gly Thr Ile Thr Gly Ile Ala Arg Lys Leu Lys Glu Lys Cys
260 265 270

Pro Gly Cys Arg Ile Ile Gly Val Asp Pro Glu Gly Ser Ile Leu Ala
275 280 285

Glu Pro Glu Glu Leu Asn Gln Thr Glu Gln Thr Thr Tyr Glu Val Glu
290 295 300

Gly Ile Gly Tyr Asp Phe Ile Pro Thr Val Leu Asp Arg Thr Val Val
305 310 315 320

Asp Lys Trp Phe Lys Ser Asn Asp Glu Glu Ala Phe Thr Phe Ala Arg
325 330 335

Met Leu Ile Ala Gln Glu Gly Leu Leu Cys Gly Gly Ser Ala Gly Ser
340 345 350

Thr Val Ala Val Ala Val Lys Ala Ala Gln Glu Leu Gln Glu Gly Gln
355 360 365

Arg Cys Val Val Ile Leu Pro Asp Ser Val Arg Asn Tyr Met Thr Lys
370 375 380

Phe Leu Ser Asp Arg Trp Met Leu Gln Lys Gly Phe Leu Lys Glu Glu
385 390 395 400

Asp Leu Thr Glu Lys Lys Pro Trp Trp Trp His Leu Arg
405 410