

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (HI-22)

AVALGLUCOSIDASE ALFA

PRONUNCIATION

ay" val gloo koe' si dase al' fa

THERAPEUTIC CLAIM

Enzyme replacement therapy for Pompe disease

CHEMICAL NAMES

1. Glucosidase, prepro- α - [199-arginine,223-histidine] (human), conjugate with 4- [(O-6-O-phosphono- α -D-mannopyranosyl-(1 \rightarrow 2)-O- α -D-mannopyranosyl-(1 \rightarrow 6)-O- α -D-mannopyranosyl-(1 \rightarrow 6)-O-[6-O-phosphono- α -D-mannopyranosyl-(1 \rightarrow 2)-O- α -D-mannopyranosyl-(1 \rightarrow 3)]- β -D-mannopyranosyl]oxy]butanoic acid 2-[2-(aminooxy)acetyl]hydrazide
2. Modified human acid α -glucosidase produced in Chinese hamster ovary (CHO) cells, glycoform alfa, conjugated to a synthetic branched hexasaccharide containing two terminal mannose-6-phosphates (M6Ps), via aminoxy linkers; [His¹⁴³>Arg,Arg¹⁶⁷>His,Val⁷²⁴>Ile]prepro-lysosomal α -glucosidase (EC=3.2.1.20) (human)-(57-952)-peptide, expressed in CHO cells, glycoform alfa, with 5~9 sialyl end groups of glycan residues being oxidized and chemically modified to 5-acetamido-3,5,7-trideoxy-7-[(E)-(2-oxo-2-{2-[4-({O-(6-O-phosphono- α -D-mannopyranosyl)-(1 \rightarrow 2)-O- α -D-mannopyranosyl-(1 \rightarrow 6)-O- α -D-mannopyranosyl-(1 \rightarrow 6)-O-[O-(6-O-phosphono- α -D-mannopyranosyl)-(1 \rightarrow 2)-O- α -D-mannopyranosyl-(1 \rightarrow 3)]- β -D-mannopyranosyl]oxy)butanoyl]hydrazinyl]ethoxy)imino]- β -L-*arabino*-2-heptulo-2,6-pyranosylonic acid groups

STRUCTURAL FORMULA

Sequence

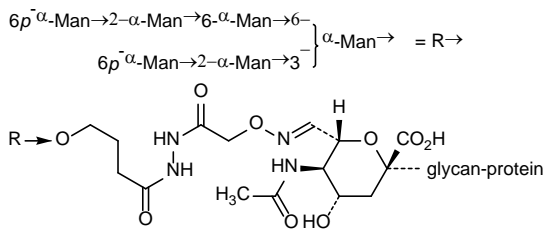
QQGASRPGPR	DAQAHPGRPR	AVPTQCDVPP	NSRFDAPDK	AITQEQCEAR	50
GCCYIPAKQG	LQGAQMGPW	CFPPSPYPSY	KLENLSSSEM	GYTATLTRTT	100
PTFFPKDILT	LRLDVMETE	NRLHFTIKDP	ANRRYEVPLE	TPRVHSRAPS	150
PLYSVEFSEE	PFGVIVHRQL	DGRVLLNTTV	APLFFADQFL	QLSTSLPSQY	200
ITGLAEHLSP	LMLSTSWTRI	TLWNRDLAPT	PGANLYGSHP	FYLALEDGGS	250
AHGVFLNSN	AMDVVLQPSP	ALSWRSTGGI	LDVYIFLQPE	PKSVVQQYLD	300
VVGYPFMPY	WGLGFHLCRW	GYSSAITRQ	VVENMTRAHF	PLDVQWNDLD	350
YMDSRRDFTF	NKDGFRDFPA	MVQELHQGGR	RYMMIVDPAI	SSSGPAGSYR	400
PYDEGLRRGV	FITNETGQPL	IGKVWPGSTA	FPDFTNPTAL	AWWEDMVAEF	450
HDQVPFDGMW	IDMNEPSNFI	RGSEDCPNN	ELENPPYVPG	VGGTTLQAAT	500
ICASSHQFLS	THYNLHNLG	LTEAIAASHRA	LVKARGTRPF	VISRSTFAGH	550
GRYAGHWTD	VWSSWEQLAS	SVPELLQFNL	LGVPLVGADV	CGFLGNTSEE	600
LCVRWTQLGA	FYPFMRNHNS	LLSLPQEPYS	FSEPAQQAMR	KALTLRYALL	650
PHLYTLFHQA	HVAGETVARP	LFLEFPKDSS	TWTVDHQLLW	GEALLITPVL	700
QAGKAEVTGY	FPLGTWYDLQ	TVPIEALGSL	PPPPAAPREP	AIHSEGQWVT	750
LPAPLDTINV	HLRAGYIIP	QGPGLTTTES	RQQPMALAVA	LTKGGEGARGE	800
LFWDDGESLE	VLERGAYTQV	IFLARNTTIV	NELVRVTSEG	AGLQLQKVTV	850
LGVATAPQQV	LSNGVPSNF	TYSPDTKVLD	ICVSLLMGEQ	FLVSWC	896

Disulfide bridges location

26-53 36-52 47-71 477-502 591-602 882-896

Glycosylation sites (N)

Asn-84 Asn-177 Asn-334 Asn-414 Asn-596 Asn-826 Asn-869



Methionine S-oxide (M)

Met-66 Met-90 Met-116 Met-117

MOLECULAR FORMULA	C ₄₄₉₀ H ₆₈₁₈ N ₁₁₉₇ O ₁₂₉₉ S ₃₂ (native protein, no post-translational modification)
MOLECULAR WEIGHT	99.38 kDa (native protein, no post-translational modification)
TRADEMARK	None as yet
SPONSOR	Genzyme Corp.
CODE DESIGNATIONS	GZ402666, neoGAA
<u>CAS</u> REGISTRY NUMBER	1802558-87-7
UNII	EO144CP0X9
WHO NUMBER	10416

gbk