

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (GH-01) CIPAGLUCOSIDASE ALFA  
 PRONUNCIATION sip" a gloo koe' si dase al' fa  
 THERAPEUTIC CLAIM Treatment of Pompe disease

CHEMICAL NAMES

1.  $\alpha$ -Glucosidase
2. [*Homo sapiens* Lysosomal alpha-glucosidase (Acid maltase, Aglucosidase alfa) precursor]-(57-952)-peptide (1-896), produced in recombinant CHO cells, glycoform alfa

STRUCTURAL FORMULA

Sequence					
QQGASRPGPR	DAQAHGPRPR	AVPTQCDVPP	NSRFDCAPDK	AITQEQCEAR	50
GCCYIPAKQG	LQGAQMGPW	CFPPPSYPSY	KLENLSSEM	GYTATLTRTT	100
PTFFPKDILT	LRLDVMMETE	NRLHFTIKDP	ANRRYEVPLE	TPHVHSRAPS	150
PLYSVEFSEE	PFGVIVRRQL	DGRVLLNTTV	APLFFADQFL	QLSTSLPSQY	200
ITGLAEHLSP	IMLSTSWTRI	TLWNRDLAPT	PGANLYGSHP	FYLALEDGGS	250
AHGVFLLSNS	AMDVVLQPSP	ALSWRSTGGI	LDVYIFLQPE	PKSVVQQYLD	300
VVGYPFMPY	WLGGFHLCRW	GYSSTAITRQ	VVENMTRAHF	PLDVQWNDLD	350
YMDSRRDFTF	NKDGFRDFPA	MVQELHQGGR	RYMMIVDPAI	SSSGPAGSYR	400
PYDEGLRRGV	FITNETGQPL	IGKVWPGSTA	FPDFTNPTAL	AWWEDMVAEF	450
HDQVPFDGMW	IDMNEPSNFI	RGSEDCPNN	ELENPPYVPG	VVGGTLQAAT	500
ICASSHQFLS	THYNLHNLYG	LTEAIAASHRA	LVKARGTRPF	VISRSTFAGH	550
GRYAGHWTD	VWSSWEQLAS	SVPEILQFNL	LGVPLVGADV	CGFLGNTSEE	600
LCVRWTQLGA	FYPFMRNHNS	LLSLPQEPYS	FSEPAQQAMR	KALTLRYALL	650
PHLYTLFHQA	HVAGETVARP	LFLEFPKDSS	TWTVDHQLLW	GEALLITPVL	700
QAGKAEVTGY	FPLGTWYDLQ	TVPVEALGSL	PPPPAAPREP	AIHSEGQWVT	750
LPAPLDTINV	HLRAGYIIPL	QGPGLTTTES	RQQPMALAVA	LTKGGEGARGE	800
LFWDDGESLE	VLERGAYTQV	IFLARNTTIV	NELVRVTSEG	AGLQLQKVTV	850
LGVATAPQQV	LSNGVPVSNF	TYSPDTKVLD	ICVSLLMGEQ	FLVSWC	896

Disulfide bridges location

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

Glycosylation sites (N)

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

MOLECULAR FORMULA      C<sub>4489</sub>H<sub>6817</sub>N<sub>1197</sub>O<sub>1298</sub>S<sub>32</sub>  
 MOLECULAR WEIGHT      99.35 kDa (non-glycosylated)  
 TRADEMARK      None as yet  
 SPONSOR      Amicus Therapeutics Inc.  
 CODE DESIGNATIONS      ATB200  
 CAS REGISTRY NUMBER      2359727-71-0

UNII

4SED7F4BSG

WHO NUMBER

11346

gbk