

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (HI-62) CLERVONAFUSP ALFA
 PRONUNCIATION kler von' a fusp al' fa
 THERAPEUTIC CLAIM Treatment of Pompe disease

CHEMICAL NAMES

1. Immunoglobulin G1, (humanized monoclonal 3E10 γ 1-chain Fab fragment) fusion protein with 67-952-lysosomal α -glucosidase (human recombinant), disulfide with humanized monoclonal 3E10 κ -chain
2. Immunoglobulin G1-kappa, cell-penetrating humanized Fab fragment targeting the Equilibrative nucleoside transporter 2 (Delayed-early response protein 12; Solute carrier family 29 member 2) fused with human Lysosomal alpha-glucosidase (Acid maltase; Aglucosidase alfa; EC=3.2.1.20): γ 1 short humanized monoclonal antibody (1-224) [VH (IGHV3-48*01 (90%) –(IGHD)-IGHJ4*01 (93%)) [8.8.9] (1-116) -*Homo sapiens* IGHG1*01 {hinge[del(11-15)], CH2[del], CH3[del]} (117-224)] fusion protein with diglycylserylbis(triglycylseryl)diglycyl (linker 225-237) and with human pro-Lysosomal alpha-glycosidase (40-925-peptide) (238-1123) (219-218')-disulfide with κ light chain (1'-218') [humanized V-KAPPA (*Homo sapiens* IGKV1-39*01 (82%) –IGKJ2*02 (82%))[10.3.9] (1'-111') -*Homo sapiens* IGKC*01 (112'-218')], produced in CHO cells, glycoform alfa

STRUCTURAL FORMULA

Heavy chain					
EVQLQESGGG	VVQPGGSLRL	SCAASGFTFS	NYGMHWIRQA	PGKGLEWVSY	50
ISSGSSTIYY	ADSVKGRFTI	SRDNSKNTLY	LQMNSLRSED	TAVVYCARRG	100
LLLDYWGQGT	LVTVSSASTK	GPSVFLAPS	SKSTSGGTAA	LGCLVKDYFP	150
EPVTVSWNSG	ALTSGVHTFP	AVLQSSGLYS	LSSVVTVPSS	SLGTQTYICN	200
VNHKPSNTKV	DKKVEPKSCD	KTHTGGSGGG	SGGGSGGDAQ	AHPGRPRAVP	250
TQCDVPPNSR	FDCAPDKAIT	QEQCEARGCC	YIPAKQGLQG	AQMGPWCFF	300
PPSYPSYKLE	NLSSEMGYT	ATLTRTPTF	FPKDILLRL	DVMETENRL	350
HFTIKDPANR	RYEVPLETPH	VHSRAPSPLY	SVEFSEEPFG	VIVRRQLDGR	400
VLLNTTVAPL	FFADQFLQLS	TSLPSQYITG	LAEHLSPML	STSWTRITLW	450
NRDLAFTPGA	NLYGSHPFYL	ALEDGGSAGH	VFLNLSNAMD	VVLQPSALS	500
WRSTGGILDV	YIFLGPPEPKS	VVQQYLDVVG	YPFMPYWG	GFHLCRWGYS	550
STAITRQVVE	NMTRAHFPLD	VQWNLDYMD	SRRDFTFNKD	GFRDFPAMVQ	600
ELHQGGRRYM	MIVDPAISSS	GPAGSYRPYD	EGLRRGVFIT	NETGQPLIGK	650
VWPGSTAFPD	FTNPATALAW	EDMVAEFHDQ	VPPDGMWIDM	NEPSNFIRGS	700
EDGCPNNELE	NPYPVPGVVG	GTLQAATICA	SSHQFLSTHY	NLHNLGLTE	750
AIASHRALVK	ARGTRPFVIS	RSTFAGHGRY	AGHWTDVWS	SWEQLASSVP	800
EILQFNLLGV	PLVGADVCGF	LGNTSEELCV	RWTQLGAFYP	FMRNHNSLLS	850
LPQEPYSFSE	PAQQAMRKAL	TLRYALLPHL	YTLFHQAQVA	GETVARPLFL	900
EFPKDSSTWT	VDHQLLWGEA	LLITPVLQAG	KAETVGYFPL	GTWYDLQTVP	950
VEALGSLPPP	PAAPREPAIH	SEGQWVTLPA	PLDTINVHLR	AGYIIPLQGP	1000
GLTTTESRQQ	PMALAVALTK	GGEARGELFW	DDGESLEVLE	RGAYTQVIFL	1050
ARNNTIVNEL	VRVTSSEGAGL	QLQKVTVLGV	ATAPQQVLSN	GVPVSNFTYS	1100
PDTKVLDDICV	SLLMGEQFLV	SWC			1123

Light chain

DIQMTQSPSS	LSASVGDRVT	ISCRASKSVS	TSSYSYMHWY	QQKPEKAPKL	50'
LIKYASYLQS	GVPSRFGSG	SGTDFTLTIS	SLQPEDVATY	YCQHSREFPW	100'
TFGAGTKLEL	KRTVAAPSVF	IFPPSDEQLK	SGTASVVCLL	NNFYPREAKV	150'
QWKVDNALQS	GNSQESVTEQ	DSKDSTYSLS	STLTLSKADY	EKHKVYACEV	200'
THQGLSSPVT	KSFNRGEC				218'

Disulfide bridges location

22-96	23'-92'	138'-198'	143-199	218'-219	253-280
263-279	274-298	704-729	818-829	1109-1123	

Glycosylation sites (N)

Asn-311 Asn-404 Asn-561 Asn-641 Asn-823 Asn-1053 Asn-1096

MOLECULAR FORMULA C₆₅₈₆H₁₀₀₅₉N₁₇₅₉O₁₉₇₅S₄₆

MOLECULAR WEIGHT 147.0 kDa

TRADEMARK None as yet

SPONSOR Valerion Therapeutics, LLC

CODE DESIGNATIONS VAL-1221

CAS REGISTRY NUMBER 2145123-44-8

UNII I6M9Q08XBP

WHO NUMBER 10973

gbk