

November 24, 2015

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (CD-115) VESTRONIDASE ALFA

PRONUNCIATION ves tron' i dase

THERAPEUTIC CLAIM Enzyme replacement therapy

CHEMICAL NAMES

1. β -Glucuronidase (synthetic human), homotetramer
2. Human beta-glucuronidase (L⁶²⁷>P variant rs9530 in dbSNP), homotetramer

STRUCTURAL FORMULA

Monomer amino acid sequence

LQGGMLYPQE	SPSRECKELD	GLWSFRADFS	DNRRRGFEEQ	WYRRPLWESG	50
PTVDMPVPSS	FNDISQDWRL	RHFVGVVWYE	REVILPERWT	QDLRTRVVL	100
IGSAHSYAIV	WVNGVDTLEH	EGGYLPFEAD	ISNLVQVGPL	PSRLRITIAI	150
NNTLTPPTLP	PGTIQYLTDT	SKYPKGYFVQ	NTYFDFFNVA	GLQRSVLLYT	200
TPTYIDDDIT	VTTSVEQDSG	LVNYQISVKG	SNLFKLEVRL	LDAENKVVAN	250
GTGTQGQLKV	PGVSLWVWY	MHERPAYLYS	LEVQLTAQTS	LGPVSDFYTL	300
PVGIRTVAVT	KSQFLINGKP	FYFHGVNKHE	DADIRGKGF	WPLLVKDFNL	350
LRWLGANAFR	TSHYPYAEV	MQMCDRYGIV	VIDECPGVGL	ALPQFFNNVS	400
LHHMQVMEE	VRRDKNHPA	VVMWSVANEP	ASHLESAGYY	LKMVIAHTKS	450
LDPSRPVTFV	SNSNYAADKG	APYVDVICLN	SYYSWYHDYG	HLELIQLQLA	500
TQFENWYKKY	QKPIIQSEYG	AETIAGFHQD	PPLMFTEEYQ	KSLLEQYHLG	550
LDQKRRKYVV	GELIWNFADF	MTEQSPTRVL	GNKKGIFTRQ	RQPKSAAFLL	600
RERYWKIANE	TRYPHSAVKS	QCLENSPFT			629

Glycosylation sites (N)

Asn-151 Asn-250 Asn-398 Asn-609

MOLECULAR FORMULA C₃₃₀₈H₄₉₉₆N₈₇₄O₉₄₀S₁₆ (non-glycosylated monomer)

MOLECULAR WEIGHT 72.56 kDa (non-glycosylated monomer)

TRADEMARK None as yet

SPONSOR Ultragenyx Pharmaceutical Inc.

CODE DESIGNATIONS UX003

CAS REGISTRY NUMBER 1638194-78-1

UNII 7XZ4062R17

WHO NUMBER 10305

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