STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

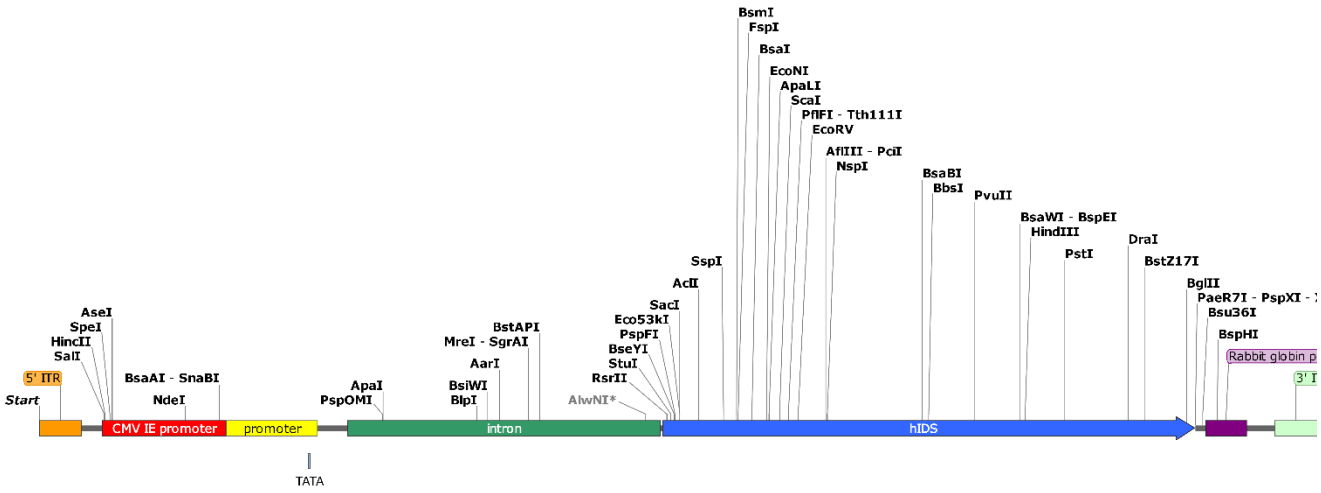
USAN (MN-159)	CLEMIDSOGENE LANPARVOVEC
PRONUNCIATION	kle mid' soe jeen lan par' voe vek
THERAPEUTIC CLAIM	Treatment of Treatment of mucopolysaccharidosis Type II (MPS II)

DESCRIPTION

DNA (recombinant replication-defective adeno-associated virus 9 vector RGX-121 cytomegalovirus immediate-early promoter-enhancer plus chicken β-actin promoter plus chicken β-actin intron plus human iduronate-2-sulfatase gene plus rabbit β-globin polyadenylation signal plus adeno-associated virus 2 inverted terminal repeat flanks-specifying).

Recombinant, non-replicating adeno-associated virus serotype 9 (rAAV9) vector encoding human iduronate 2-sulfatase (IDS, α-L-iduronate sulfate sulfatase) under control of a human cytomegalovirus (CMV) immediate early enhancer/chicken β-actin (CBA) promoter, with a chicken β-actin intron, terminated with the rabbit β-globin polyadenylation signal, and flanked by adeno-associated virus 2 (AAV2) inverted terminal repeats (ITRs)

Genetic Map of the entire product:



RGX-121 Packaged Sequence

Table with position of features:

Feature	Location (bp)
5’ Inverted Terminal Repeats (ITR)	0 - 130
Cytomegalovirus (CMV) immediate-early enhancer (IE) promoter	198 - 579
Chicken β-actin promoter	582 - 863
TATA	836-839
Chicken β-actin intron	956 - 1927
Human Iduronate-2-sulfatase cDNA	1934 - 3586
Rabbit β-globin poly A signal	3620 - 3746

3’ ITR	3835 - 3964

SEQUENCE

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SPONSOR	REGENXBIO Inc.
CODE DESIGNATIONS	RGX 121
CAS NUMBER	2766142-50-9
UNII	K5LW2WBP48
WHO NUMBER	12682

SCS