STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (NO-163)	AZAMIDUGENE AUTOTEMCEL
PRONOUNCIATION	ay'' za mi' doo jeen aw'' toe tem' sel
THERAPEUTIC CLAIM	Treatment of Mucopolysaccharidosis type I Hurler (MPS-IH)

DESCRIPTION

Autologous CD34+ cells obtained from mobilized peripheral blood, transduced with a self-inactivating lentiviral vector expressing human alpha-L-iduronidase (IDUA) under the control of a human phosphoglycerate kinase (PGK) promoter.

SEQUENCE

Sequence

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SCHEMATIC AND TABLE OF FEATURES

Schematic of the vector

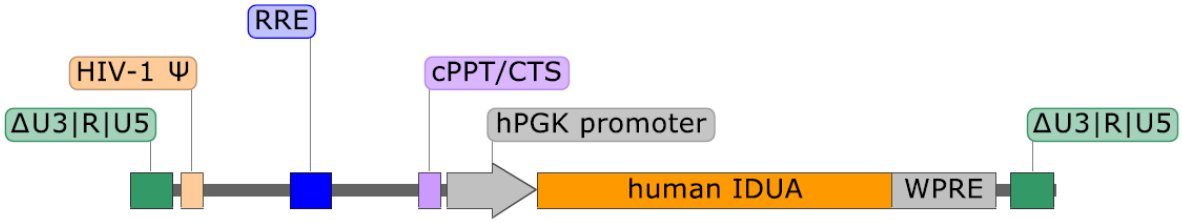

Table of Features

Specific feature	Description	Location (bp)	Color code
ΔU3 | R | U5 (LTR)	Long terminal repeat (LTR)	1-235	green
HIV-1 Ψ	ψ packaging signal	281-406	light orange
RRE	Rev response element	899-1132	blue
cPPT/CTS	central polypurine tract/central termination sequence	1628-1744	purple
hPGK promoter	human phosphoglycerate kinase (PGK) promoter	1777-2291	grey
human IDUA	human alpha-L-iduronidase (IDUA)	2292-4269	orange
WPRE	Woodchuck hepatitis virus post-transcriptional regulatory element	4291-4882	grey
ΔU3 | R | U5 (LTR)	Long terminal repeat (LTR)	4966-5200	green

TRADEMARKS	None
SPONSOR	Orchard Therapeutics North America
DESIGNATION CODES	OTL-203
UNII	7BAL95SK9C
WHO NUMBER	11773

SCS