

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (JK-136)	ATIDARSAGENE AUTOTEMCEL
PRONUNCIATION	at" i dar' sa jeen aw" toe tem' sel
THERAPEUTIC CLAIM	Treatment of Metachromatic Leukodystrophy

PRODUCT DESCRIPTION

Autologous CD34+ hematopoietic stem and progenitor cells transduced *ex vivo* with a replication-incompetent lentiviral vector encoding human arylsulfatase A (ARSA). Autologous CD34+ hematopoietic stem and progenitor cells (HSPC), obtained from bone marrow (BM) or mobilized peripheral blood (mPB), transduced *ex vivo* with a replication-incompetent pseudotyped HIV-I based lentiviral vector encoding the human arylsulfatase A (ARSA) transgene under the control of a human phosphoglycerate kinase (PGK) promoter. The vector is pseudotyped with the vesicular stomatitis vims (VSV) envelope glycoprotein. The vector genome also contains a mutated Woodchuck hepatitis vims post-transcriptional regulatory element (WPRE), a fragment of the HIV-I *gag*, the HIV-I Rev response element (RRE), the HIV-1 central polypurine tract (cPPT) and a fragment of *nef*. The CD34+ cell population corresponds to an heterogenous population of stem and progenitor cells, some expressing the CD90 and/or CD133 markers. (INN Programme recommended INN list #85)

TRADEMARK	Libmeldy™
SPONSOR	Orchard Therapeutics North America
CODE DESIGNATIONS	OTL-200
UNII	EPP8G99QG4
WHO NUMBER	11282

SCS