STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (KL-71)	VOXERALGAGENE AUTOTEMCEL
PRONUNCIATION	vox" e ral’ ga jeen aw toe tem’ sel
THERAPEUTIC CLAIM	Treatment of Fabry disease (AGA deficiency)

PRODUCT DESCRIPTION

AVR-RD-01 is an ex vivo lentiviral vector (LV)-mediated gene modified cell therapy for Fabry disease. AVR-RD-01 is autologous transplantation of a mobilized CD34+ cell-enriched population procured from the subject’s peripheral blood that is genetically modified, ex vivo with an LV encoding the human alpha-galactosidase A (AGA) complementary deoxyribonucleic acid (cDNA) sequence.

Autologous CD34+ hematopoietic stem cells (HSCs) transduced ex vivo with a self-inactivating lentiviral vector encoding human alpha-galactosidase A (GLA). Autologous CD34+ hematopoietic stem cells (HSCs) obtained by apheresis, transduced with a non-replicating, self-inactivating lentiviral vector encoding codon-optimized human alphagalactosidase A (GLA) under the control of the human elongation factor 1 alpha short (EFS-1 alpha) promoter and a modified woodchuck hepatitis virus post-transcriptional regulatory element (WPRE). The vector genome also contains a packaging signal, a partial gag sequence, a Rev response element (RRE), a central polypurine tract (cPPT) and a Kozak sequence. cell-based gene therapy (Source: INN Proposed List # 124)

TRADEMARK	None
SPONSOR	AVROBIO, Inc.
CODE DESIGNATIONS	AVR-RD-01
UNII	RZ8DCR7A94
WHO NUMBER	11441

SCS