

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (JK-90) VANGLUSAGENE ENSIPARVOVEC

PRONUNCIATION van gloo' sa jeen en" si par' voe vek

THERAPEUTIC USE Treatment of Pompe Disease

DESCRIPTION

A recombinant liver-directed adeno-associated virus vector expressing human acid alpha-glucosidase (GAA). A recombinant liver-directed adeno-associated virus serotype rh74 (AAVrh74) vector that contains a bioengineered capsid derived from AAVrh74 by four amino-acid substitutions and a codon-optimized transgene expression cassette to drive hepatic synthesis and secretion of the lysosomal enzyme human acid alpha-glucosidase (GAA, α -1,4-glucosidase, acid maltase) via an apolipoprotein E hepatic control region 1 (ApoE HCR1) enhancer/ human alpha 1-antitrypsin (hAAT) promoter, a modified human beta-globin intron 2 (HBB2) and a bovine growth hormone (bGH) polyA signal sequence, flanked by adeno-associated virus 2 (AAV2) inverted terminal repeats (ITRs). (Source INN proposed list #124)

CAS NAME

DNA (recombinant adeno-associated virus vector AAV-Spark100-GAA human liver-specific expression cassette including human lysosomal α -glucosidase codon-optimized gene GAA plus adeno-associated virus 2 inverted terminal repeat flanks) (CA INDEX NAME)

SPONSOR Spark Therapeutics, Inc.

CODE DESIGNATION SPK-3006

CAS REGISTRY NUMBER 2382896-00-4

UNII Y47XZ2EW2J

WHO NUMBER 11603

SCS