

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (JK-07) DIRLOCTOCOGENE SAMOPARVOVEC  
PRONUNCIATION dir lok" toe koe' jeen sam" oh par' voe vek  
THERAPEUTIC CLAIM Treatment of Hemophilia A

DESCRIPTION

1. DNA (recombinant adeno-associated virus 2 vector AAVSPK200hFVIII8011 human B-domain-deleted blood-coagulation factor VIII-specifying) (CAS)
2. A recombinant non-replicating adeno-associated virus vector with a bio-engineered capsid derived by DNA shuffling of 10 AAV capsid sequences (AAV1, 2, 3B, 4, 5, 6 8, 9, avian and bovine AAV) followed by in vivo selection in Fah<sup>-/-</sup>/Rag2<sup>-/-</sup>/Il2rg<sup>-/-</sup> (FRG) mice partially repopulated with human hepatocytes. The vector has AAV2 genomic inverted terminal repeats (ITR) and encodes a human B-domain-deleted blood coagulation factor VIII (hF8, FVIII) under the control of a modified transthyretin (TTRm) promoter (proposed INN list #121)

TRADEMARK None as of yet  
SPONSOR Spark Therapeutics  
CODE DESIGNATION SPK-8011; AAV-Spark 200- hFVIII  
CAS REGISTRY NUMBER 2163074-23-3  
UNII I2G2SZ8B0C  
WHO NUMBER 11154

SCS