

# STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (HI-78)

BIDRIDISTROGENE XEBOPARVOVEC

PRONUNCIATION

bid" ri dis' troe jeen zeb" oh par' voe vek

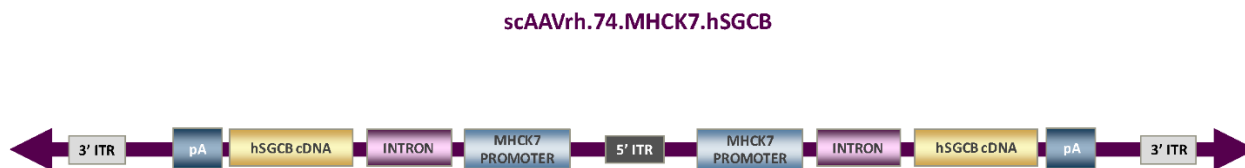
THERAPEUTIC CLAIM

Treatment of limb-girdle muscular dystrophy Type 2E (LGMD2E)

## DESCRIPTION

bidridistrogene xeboparovec recombinant self-complementary (dimeric) non-replicating adeno-associated virus (AAV) serotype rh.74 vector encoding codon-optimized human beta-sarcoglycan (hSGCB) under control of a hybrid promoter (MHCK7) consisting of enhancer/promoter regions of murine muscle creatine kinase (CK) and alpha-myosin heavy chain genes, followed by a chimeric intron, composed of the 5' donor site from the human beta-globin gene and the branch point and 3' splice acceptor site from IgG heavy chain variable region, and terminated by a small synthetic polyadenylation (polyA) signal sequence, flanked by adeno-associated virus 2 (AAV2) inverted terminal repeats (ITRs). gene therapy (*muscular dystrophy*)

## SCHEMATIC MAP



ITR = Inverted Terminal Repeat, hSGCB = Human Beta-Sarcoglycan, pA = Polyadenylation sequence, AAV = Adeno-Associated Virus

TRADEMARK

None as yet

SPONSOR

Sarepta Therapeutics, Inc.

CODE DESIGNATION

SRP-9003

CAS REGISTRY NUMBER

2365142-14-7

UNII

NM4FH5S2OW

WHO NUMBER

11619

## SCS