

# STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (HI-101)

PATIDISTROGENE BEXOPARVOVEC

PRONUNCIATION

pat" i dis' troe jeen bex" oh par' voe vek

THERAPEUTIC CLAIM

Treatment of limb-girdle muscular dystrophy type 2D (LGMD2D)

## DESCRIPTION

recombinant self-complementary (dimeric) nonreplicating adeno-associated virus serotype rh.74 (AAVrh74) vector encoding codon-optimized human alpha-sarcoglycan (hSGCA), under the control of a triple E box truncated muscle creatine kinase (tMCK) promoter/enhancer, followed by a chimeric intron, composed of the 5' donor site from the human beta globulin gene and the branch point and 3' splice acceptor site from the 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, hSGCA=Human Alpha-Sarcoglycan, pA=Polyadenylation Sequence, AAV=Adeno-Associated Virus

TRADEMARK

None as yet

SPONSOR

Sarepta Therapeutics

CODE DESIGNATION

SRP-9004

CAS REGISTRY NUMBER

2376210-48-7

UNII

F31AI96DHK

WHO NUMBER

11593

## SCS