

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (GH-150)	TIMREPIGENE EMPARVOVEC
PRONUNCIATION	tim rep' i jeen em par' voe vek
THERAPEUTIC CLAIM	Treatment of choroideremia (CHM)

DESCRIPTION

DNA (synthetic adeno-associated virus 2 clone AAV2-REP1 vector human Rab escort protein type 1 gene cDNA-specifying)

Each 20 nm AAV virion contains a single stranded DNA insert sequence comprising:

- 130 bp 5' inverted terminal repeat (ITR)
- 941 bp Cytomegalovirus enhancer/chicken-beta actin (CBA) hybrid promoter
- 1,962 bp human REP1 cDNA
- 589 bp Woodchuck hepatitis post-transcriptional regulatory element (WPRE)
- 269 bp Bovine growth hormone polyadenylation sequence (BGH-polyA)
- 121 bp 3' ITR

SCHEMATIC MAP



TRADEMARK	None as yet
SPONSOR	Nightstar, Inc.
CODE DESIGNATIONS	AAV2-REP1; NSR-REP1
<u>CAS</u> REGISTRY NUMBER	1905415-02-2
UNII	M9N48CJY6T
WHO NUMBER	10571

SCS