

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (FG-117)

ELADOCAGENE EXUPARVOVEC

PRONUNCIATION

el" a doe' ka jeen ex" ue par' voe vek

THERAPEUTIC CLAIM

Treatment of AADC deficiency

DESCRIPTION

Gene of Interest (GOI): Human dopa decarboxylase (*DOC*) rAAV2-hAADC. The vector has a wild-type (WT) AAV2 capsid that encapsulates the DNA construct described below:

Viral Capsid Serotype: AAV2

Cytomegalovirus intermediate-early promoter (CMV IEP promoter), human beta globin partial intron 2/partial exon 3 (HBG2/3), unmodified human *ODCcDNA* (GOI), polyadenylation containing sequence (poly A), and an ITR linker.

The entire sequence is flanked by AAV2 Inverted Terminal Repeats (ITR).

TRADEMARK

None as yet

SPONSOR

PTC Therapeutics, Inc.

CODE DESIGNATION

AGIL-AADC

CAS REGISTRY NUMBER

2098615-91-7

WHO NUMBER

10885

SCS