

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (HI-242)	AVALOTCAGENE ONTAPARVOVEC
PRONUNCIATION	av" a lot' ka jeen on" ta par' voe vek
THERAPEUTIC CLAIM	Treatment of ornithine transcarbamylase (OTC) deficiency

DESCRIPTION

1. DNA (recombinant adeno-associated virus 8 vector 301OTC01 human ornithine carbamoyltransferase-specifying) (CAS)
2. A recombinant non-replicating adeno-associated virus type 2/8 (rAAV8) vector, encoding codon-optimized, wild-type human ornithine transcarbamylase (OTC) under the control of a hybrid human thyroxine-binding globulin (TBG) / human alpha 1-microglobulin/bikunin precursor (AMBP) promoter/enhancer. The vector genome is a head-to-head dimer, with the vector genome cassette located 5' of the mutated internal inverted terminal repeat (ITR) in a reverse orientation and the vector genome cassette located 3' of the mutated internal ITR in a forward orientation (WHO pINN list 121 revised in pINN list 123.)

TRADEMARK	None as of yet
SPONSOR	Ultragenyx Pharmaceutical Inc.
CODE DESIGNATIONS	DTX301
<u>CAS</u> REGISTRY NUMBER	2227000-30-6
UNII	20E4R0BF12
WHO NUMBER	11153

SCS