

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (HI-204) AUTOGENE CEVUMERAN

PRONUNCIATION aw' toe jeen sev'' ue mer' an

THERAPEUTIC CLAIM Antineoplastic

CHEMICAL NAMES

1. RNA (recombinant 5'-[1,2-[m7Gm-(5'→5')-p-[P(R)]-sp-p-G]]-capped 5'-UTR (human α-globin RNA-Kozak)/secretory signal peptide sec2.0-specifying/patient-specific tumor neoantigen-specifying insertion site/major histocompatibility complex class I transmembrane and cytoplasmic domains-specifying/3'-UTR (AES mRNA-mitochondrial 12S rRNA)-containing poly(A)-tailed messenger RO7198457 constant region), inner salt (CA INDEX NAME)
2. A 5'-capped single-stranded messenger RNA (mRNA) construct for the expression of patient-specific tumour neoantigens comprising a 5'-cap, a 5'-untranslated region derived from human alpha-globin mRNA, an optimised Kozak sequence, sequences encoding a secretory signal peptide (sec2.0), transmembrane and cytoplasmic domains of a major histocompatibility complex (MHC) Class I molecule (MITD), a 3'-untranslated region (FI) and a poly(A)-tail; sequences encoding patient specific antigens are inserted between the secretory signal peptide and the transmembrane/cytoplasmic encoding domains. immunological agent for active immunization (antineoplastic) (INN Proposed List #122)

SEQUENCE

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SEQ      1 ggggcgaacu  aguauucuuu  ugguccccac  agacucagag  agaaccgccc
      51 accaugagag  ugauggcccc  cagaaccucg  auccugcugc  ugucuggcgc
     101 ccuggccucg  acagagacau  gggccggaag  cnaucguggg  aauuguggca
     151 ggacuggcag  ugcuggccgu  gguggugauc  ggagccgugg  uggcuaccgu
     201 gaugugcaga  cggaagucca  gcggaggcaa  gggcggcagc  uacagccagg
     251 ccgccagcuc  ugauagcgcc  cagggcagcg  acgugucacu  gacagccuag
     301 uaacucgagc  ugguacugca  ugcacgcaau  gcuagcugcc  ccuuucccgu
     351 ccuggguacc  ccgagucucc  cccgaccucg  ggucccaggu  augcucccac
     401 cuccaccugc  cccacucacc  accucugcua  guuccagaca  ccucccaagc
     451 acgcagcaau  gcagcucaa  acgcuuagcc  uagccacacc  cccacgggaa
     501 acagcaguga  uuaaccuuua  gcaauaaacg  aaaguuuuac  uaagcuauac
     551 uaaccccagg  guuggucaau  uucgugccag  ccacaccgag  accuggucca
     601 gagucgcuag  ccgcgucgcu  aaaaaaaaaa  aaaaaaaaaa  aaaaaaaaaa
     651 aaaaaaaaaa  aaaaaaaaaa  aaaaaaaaaa  aaaaaaaaaa  aaaaaaaaaa
     701 aaaaaaaaaa  aaaaaaaaaa  aaaaaaaaaa  aaaaaaaaaa  aaaaaaaaaa
    
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Feature		Position
Capping structure :	phosphorothioate stabilized cap analogue	1-2
hAG-Kozak	human ct-globin - Kozak region	3-53
sec 2.0 :	secretion signal sequence	54-131
N:	indicates location of insertion of patient-specific sequences	132
MITD:	transmembrane and cytoplasmic domains of MHC class I molecule	133-303
FI:	sequence element derived of the amino	:304-620

AI 20: terminal enhancer of split RNA (F) and from
the mitochondrially encoded I2S RNA (I)
poly(A) tail of 120 nucleotides 621-740

TRADEMARK	None as yet
SPONSOR	Genentech
CODE DESIGNATION	RO7198457
<u>CAS</u> REGISTRY NUMBER	2365453-34-3
UNII	L8HDH9IB06
WHO NUMBER	11177

SCS