

# STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (HI-177)

BELCESIRAN

PRONUNCIATION

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THERAPEUTIC CLAIM

Treatment of alpha-1 antitrypsin deficiency

CHEMICAL NAME

1. RNA, (Am-s<sup>p</sup>-Am-(2'-deoxy-2'-fluoro)A-Cm-Cm-Cm-Um-(2'-deoxy-2'-fluoro)U-(2'-deoxy-2'-fluoro)U-(2'-deoxy-2'-fluoro)G-Um-(2'-deoxy-2'-fluoro)C-(2'-deoxy-2'-fluoro)U-Um-Cm-Um-(2'-deoxy-2'-fluoro)U-Am-Am-Am-Gm-Cm-Am-Gm-Cm-Cm-[2'-O-[[2-[2-[[5-[[2-(acetylamino)-2-deoxy-β-D-galactopyranosyl]oxy]-1-oxopentyl]amino]ethoxy]ethoxy]methyl]]G-[2'-O-[[2-[2-[[5-[[2-(acetylamino)-2-deoxy-β-D-galactopyranosyl]oxy]-1-oxopentyl]amino]ethoxy]ethoxy]methyl]]A-[2'-O-[[2-[2-[[5-[[2-(acetylamino)-2-deoxy-β-D-galactopyranosyl]oxy]-1-oxopentyl]amino]ethoxy]ethoxy]methyl]]A-[2'-O-[[2-[2-[[5-[[2-(acetylamino)-2-deoxy-β-D-galactopyranosyl]oxy]-1-oxopentyl]amino]ethoxy]ethoxy]methyl]]A-Gm-Gm-Cm-Um-Gm-Cm), complex with RNA ([1-de-β-D-ribofuranosyl-1-[(2*R*,3*R*,4*S*,5*S*)-tetrahydro-4-hydroxy-5-[(hydroxymethoxyphosphinyl)methoxy]-3-methoxy-2-furanyl]]U-(4'→5')-s<sup>p</sup>-(2'-deoxy-2'-fluoro)U-s<sup>p</sup>-(2'-deoxy-2'-fluoro)U-s<sup>p</sup>-Am-(2'-deoxy-2'-fluoro)A-Gm-(2'-deoxy-2'-fluoro)A-Am-Gm-Am-Cm-(2'-deoxy-2'-fluoro)A-Am-(2'-deoxy-2'-fluoro)A-Gm-(2'-deoxy-2'-fluoro)G-Gm-Um-(2'-deoxy-2'-fluoro)U-Um-s<sup>p</sup>-Gm-sp-Gm) (1:1) (CA INDEX NAME)

## STRUCTURAL FORMULA

### Sense Strand:

5' mA-S-mA-fA-mC-mC-mC-mU-fIJ-fIJ-fG-mU-fC-fIJ-mU-mC-mU-fIJ-mA-mA-mA-mG-mC-mA-mG-mC-mC-[ademG-GalNAc]-[ademA-GalNAc]-[ademA-GalNAc]-[ademA-GalNAc]-mG-mG-mC-mU-mG-mC 3'

Hybridized to:

### Antisense Strand:

5' [MePhosphonate-4*O*-mU]-S-fIJ-S-fIJ-S-mA-fA-mG-fA-mA-mG-mA-mC-fA-mA-fA-mG-fG-mG-mU-fU-mU-S-mG-S-mG 3'

### Legend:

mX: 2'-0-methyl ribonucleotide

fX: 2'-fluoro-deoxyribonucleotide [ademA-GalNAc]:

2'-0-GalNAc-

modified adenosine [ademG-GalNAc]:

2'-0-GalNAc-

modified guanosine

[MePhosphonate-4*O*-mU]: 4'-0-monomethylphosphonate-2'-0-

methyl uridine Linkages: "-" denotes phosphodiester

"-S-" denotes phosphorothioate

SEQ 1 aaaccuuug ucuucuuaaa gcagccgaaa ggcugc

SEQ 1 uuaagaaga caaaggguuu gg

MOLECULAR FORMULA	C <sub>666</sub> H <sub>877</sub> O <sub>417</sub> N <sub>231</sub> F <sub>15</sub> P <sub>57</sub> S <sub>6</sub>
MOLECULAR WEIGHT	21033.3 Da
TRADEMARK	None as of yet
SPONSOR	Dicerna Pharmaceuticals, Inc.
CODE DESIGNATIONS	DCR-S1459; DCR-A1AT
<u>CAS</u> REGISTRY NUMBER	2375562-54-0
UNII	9ET5DMZ3AW
WHO NUMBER	11754

SCS