

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (HI-158)

NEDOSIRAN

PRONUNCIATION

ned" oh sir' an

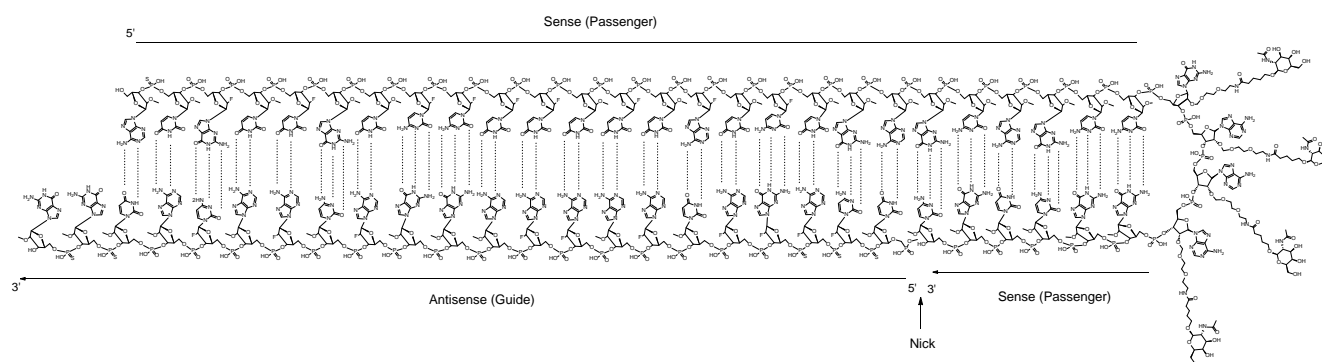
THERAPEUTIC CLAIM

Treatment of primary hyperoxaluria

CHEMICAL NAME

RNA, (Am-sp-Um-(2'-deoxy-2'-fluoro)G-Um-(2'-deoxy-2'-fluoro)U-Gm-Um-(2'-deoxy-2'-fluoro)C-(2'-deoxy-2'-fluoro)C-(2'-deoxy-2'-fluoro)U-(2'-deoxy-2'-fluoro)U-Um-(2'-deoxy-2'-fluoro)U-Um-(2'-deoxy-2'-fluoro)A-Um-(2'-deoxy-2'-fluoro)C-Um-Gm-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-[(2R,3R,4S,5S)-tetrahydro-4-hydroxy-5-[(hydroxymethoxyphosphinyl)methoxy]-3-methoxy-2-furanyl]]U-(4'→5')- sp-(2'-deoxy-2'-fluoro)C-sp-(2'-deoxy-2'-fluoro)A-sp-(2'-deoxy-2'-fluoro)G- (2'-deoxy-2'-fluoro)A-Um-(2'-deoxy-2'-fluoro)A-Am-(2'-deoxy-2'-fluoro)A- (2'-deoxy-2'-fluoro)A-Am-Gm-Gm-(2'-deoxy-2'-fluoro)A-Cm-(2'-deoxy-2'-fluoro)A-Am-(2'-deoxy-2'-fluoro)C-Am-Um-sp-Gm-sp-Gm) (1:1)

STRUCTURAL FORMULA



MOLECULAR FORMULA

$C_{662}H_{865}F_{19}N_{231}O_{413}P_{57}S_6$

MOLECULAR WEIGHT

21.0 kDa

TRADEMARK

None as yet

SPONSOR

Dicerna Pharmaceuticals Inc.

CODE DESIGNATIONS

None

<u>CAS</u> REGISTRY NUMBER	224 7026-22-6
UNII	13U9R5J3WL
WHO NUMBER	11373

gbk