

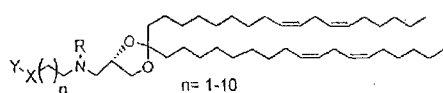


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(54) Title: NOVEL LIPIDS AND COMPOSITIONS FOR THE DELIVERY OF THERAPEUTICS

Figure 1.



X = NH(CO), (CO)NH, NH(CO)O
O(CO)NH, O, NH(CO)NH, S-S

R = CH₃, -(CH₂)₁₋₁₀,

Y = (CH₂)_n- GalNAc, Mannose, Lactose, Glucose, Fucose

Y = (CH₂)_n- (GalNAc)₂, (Mannose)₂, (Lactose)₂, (Glucose)₂, (Fucose)₂

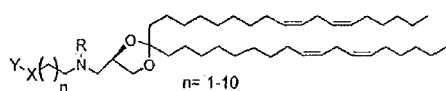
Y = (CH₂)_n- (GalNAc)₃, (Mannose)₃, (Lactose)₃, (Glucose)₃, (Fucose)₃

Y = (CH₂-CH₂-O)_a- GalNAc, Mannose, Lactose, Glucose, Fucose

Y = (CH₂-CH₂-O)_a- (GalNAc)₂, (Mannose)₂, (Lactose)₂, (Glucose)₂, (Fucose)₂

Y = (CH₂-CH₂-O)_a- (GalNAc)₃, (Mannose)₃, (Lactose)₃, (Glucose)₃, (Fucose)₃

a = 1-100



X = NH(CO), (CO)NH, NH(CO)O
O(CO)NH, O, NH(CO)NH, S-S

R = CH₃, -(CH₂)₁₋₁₀,

Y = (CH₂)_n- GalNAc, Mannose, Lactose, Glucose, Fucose

Y = (CH₂)_n- (GalNAc)₂, (Mannose)₂, (Lactose)₂, (Glucose)₂, (Fucose)₂

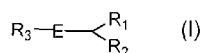
Y = (CH₂)_n- (GalNAc)₃, (Mannose)₃, (Lactose)₃, (Glucose)₃, (Fucose)₃

Y = (CH₂-CH₂-O)_a- GalNAc, Mannose, Lactose, Glucose, Fucose

Y = (CH₂-CH₂-O)_a- (GalNAc)₂, (Mannose)₂, (Lactose)₂, (Glucose)₂, (Fucose)₂

Y = (CH₂-CH₂-O)_a- (GalNAc)₃, (Mannose)₃, (Lactose)₃, (Glucose)₃, (Fucose)₃

a = 1-100



(57) Abstract: The present invention provides lipids that are advantageously used in lipid particles for the in vivo delivery of therapeutic agents to cells. In particular, the invention provides lipids having the following structure (I) wherein R₁ and R₂ are each independently for each occurrence optionally substituted C₁₀-C₃₀ alkyl, optionally substituted C₁₀-C₃₀ alkenyl, optionally substituted C₁₀-C₃₀ alkynyl, optionally substituted C₁₀-C₃₀ acyl, or -linker-ligand; R₃ is H, optionally substituted C₁-C₁₀ alkyl, optionally substituted C₂-C₁₀ alkenyl, optionally substituted C₂-C₁₀ alkynyl, alkyl-heterocycle, alkylphosphate, alkylphosphorothioate, alkylphosphorodithioate, alkylphosphonates, alkylamines, hydroxyalkyls, ω-aminoalkyls, ω-(substituted)aminoalkyls, ω-phosphoalkyls, ω-thiophosphoalkyls, optionally substituted polyethylene glycol (PEG, mw 100-40K), optionally substituted mPEG (mw 120-40K), heteroaryl,

[Continued on next page]



YARAMAN, Muthusamy [IN/US]; Alnylam Pharmaceuticals, Inc., 300 Third Street, Cambridge, MA 02142 (US). **BUTLER, David** [GB/US]; Alnylam Pharmaceuticals, Inc., 300 Third Street, Cambridge, MA 02142 (US). **NARAYANANNAIR, Jayaprakash, K.** [IN/US]; Alnylam Pharmaceuticals, Inc., 300 Third Street, Cambridge, MA 02142 (US). **MAIER, Martin** [—/US]; Alnylam Pharmaceuticals, Inc., 300 Third Street, Cambridge, MA 02142 (US). **ELTEPU, Laxman**; Alnylam Pharmaceuticals, Inc., 300 Third Street, Cambridge, MA 02142 (US).

(74) **Agent: MCCARTY, Catherine, M.**; Lando & Anastasi, LLP, Riverfront Office Park, One Main Street - 11th Floor, Cambridge, MA 02142 (US).

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- *as to the applicant's entitlement to claim the priority of the earlier application (Rule 4.17(iii))*

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