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- (54) LIPID NANOPARTICLE BASED COMPOSITIONS AND METHODS FOR THE DELIVERY OF BIOLOGICALLY ACTIVE **MOLECULES**
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(57)ABSTRACT

The present invention relates to novel cationic lipids, transfection agents, microparticles, nanoparticles, and short interfering nucleic acid (siNA) molecules. The invention also features compositions, and methods of use for the study, diagnosis, and treatment of traits, diseases and conditions that respond to the modulation of gene expression and/or activity in a subject or organism. Specifically, the invention relates to novel cationic lipids, microparticles, nanoparticles and transfection agents that effectively transfect or deliver biologically active molecules, such as antibodies (e.g., monoclonal, chimeric, humanized etc.), cholesterol, hormones, antivirals, peptides, proteins, chemotherapeutics, small molecules, vitamins, co-factors, nucleosides, nucleotides, oligonucleotides, enzymatic nucleic acids, antisense nucleic acids, triplex forming oligonucleotides, 2,5-A chimeras, dsRNA, allozymes, aptamers, decoys and analogs thereof, and small nucleic acid molecules, such as short interfering nucleic acid (siNA), short interfering RNA (siRNA), double-stranded RNA (dsRNA), micro-RNA (miRNA), and short hairpin RNA (shRNA) molecules, to relevant cells and/or tissues, such as in a subject or organism. Such novel cationic lipids, microparticles, nanoparticles and transfection agents are useful, for example, in providing compositions to prevent, inhibit, or treat diseases, conditions, or traits in a cell, subject or organism. The compositions described herein are generally referred to as formulated molecular compositions (FMC) or lipid nanoparticles (LNP).



Figure 1

A
$$\bigcap_{n} O = \bigcap_{n} O = \bigcap_$$

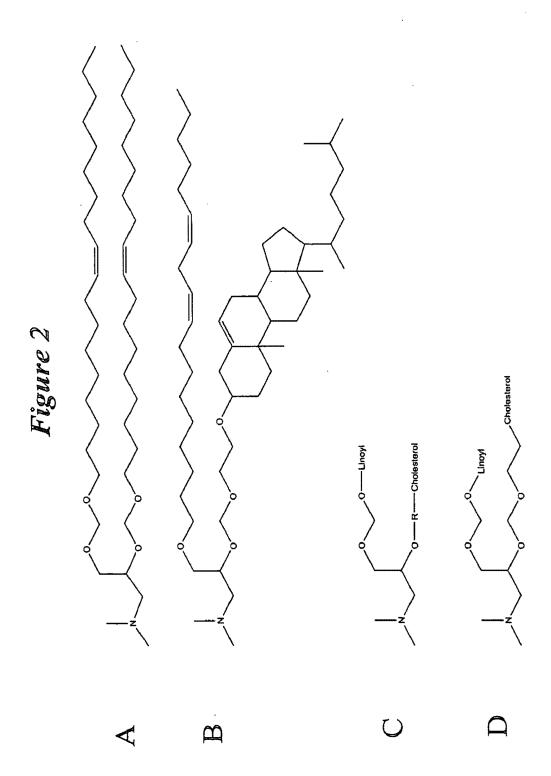


Figure 3

$$C$$
 H_2N

Figure 4

A
$$C_{8}H_{17} \longrightarrow C_{H}$$

$$C_{8}H_{17} \longrightarrow C_{H}$$

$$C_{8}H_{17} \longrightarrow C_{H}$$

$$C_{14}H_{29} \longrightarrow C_{14}H_{29} \longrightarrow C_{H}$$

$$C_{18}H_{37} \longrightarrow C_{18}H_{37} \longrightarrow C_{$$

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