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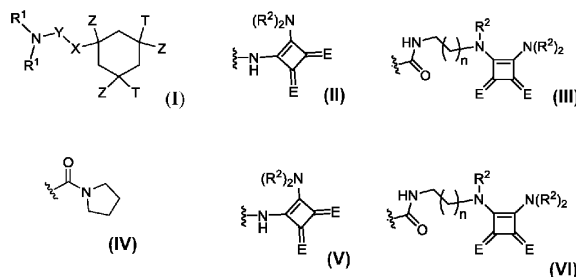
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(54) Title: CYCLOHEXANE LIPIDOIDS FOR NUCLEIC ACID TRANSFECTION AND USE THEREOF



(57) **Abstract:** The present invention relates to a lipidoid of general formula I, wherein X is selected from -C(=O)NH-, -C(=O)O-, -C(=S)O-, -C(=O)S-, -C(=S)S-, -C(=O)NHNH-, -CH₂-, -O-, -OC(=O)-, -S-, -SC(=O)-, -NH-, -NHNH-, -NHC(=O)-, -NHNHC(=O)-, -C≡C-, -CH=CH-, a five-membered heterocycle containing at least 2 nitrogen atoms, -CH₂C(=O)NH-, -CH₂C(=S)O-, -CH₂C(=S)S-, -CH₂C(=O)NHNH-, -N=CH-, -CH=N-, -NH-N=CH-, and -CH=N-NH-; Y is alkylene C₂-C₁₀ chain; R¹ is selected from alkyl C₁-C₄₆, alkenyl C₂-C₄₆, alkynyl C₂-C₄₆; Z is selected from H, -OH, -CH₃, -CH₂OH, -NH₂, -C(=O)NH₂, -CONH(CH₂)₂OH, -CON[(CH₂)₂OH]₂, -CONHCH(CH₂OH)₂, -CONHCH₂CH(OH)CH₂OH, -CONH(CH₂)₂C(=O)NH₂, -CON[CH₂C(=O)NH₂]₂, -CONH(CH₂)₂NHC(=O)NH₂, -CONH(CH₂)₃-N+(CH₃)₂-(CH₂)₂-SO₃⁻, -CONH(CH₂)₃-N+(CH₃)₂-(CH₂)₂-COO⁻, -COO(CH₂)₂-O-P(=O)(O-)-O(CH₂)₂-N+(CH₃)₃, -N+(CH₃)₂-(CH₂)₃-SO₃⁻, -N+(CH₃)₂-(CH₂)₂-COO⁻, formula (II), and formula (III), wherein R² is independently selected from hydrogen and -CH₃; E is independently selected from O and S atoms; n is an integer with in the range of from 1 to 5; and T is selected from -X-Y-N(R¹)₂, -C(=O)O(C₁-C₃ alkyl), -C(=O)OCH₂CH₂OH, formula (IV), formula (V), formula (VI), -C(=O)OH, -CONH(CH₂)₂OH, -CON[(CH₂)₂OH]₂, -CONHCH(CH₂OH)₂, CONH(CH₂)₂C(=O)NH₂, -CON[CH₂C(=O)NH₂]₂, -CONHCH[C(=O)NH₂]₂, -CONH(CH₂)₂NHC(=O)NH₂, -C(=O)NH₂, -CONH(CH₂)₃-N+(CH₃)₂-(CH₂)₂-SO₃⁻, -CONH(CH₂)₃-N+(CH₃)₂-(CH₂)₂-COO⁻, -NH₂, -NHC(=O)CH₃, -COO(CH₂)₂-O-P(=O)(O-)-O(CH₂)₂-N+(CH₃)₃, -OH, -O(C₁-C₃ alkyl), -NHC(=O)NH(CH₃), -NHC(=S)N(CH₃)₂, -NHC(=S)NH(CH₃), -NHC(=N-CN)NH₂, -NHC(=N-CN)NH(CH₃), -NHC(=N-CN)N(CH₃)₂, -NHC[=N-S(=O)₂NH₂]NH₂, -N+(CH₃)₂-(CH₂)₃-SO₃⁻, -N+(CH₃)₂-(CH₂)₂-COO⁻, wherein R², E and n are as defined above; and/or if Z is -OH or -CH₂OH, and T is -C(=O)OH, then Z together with T and three carbon atoms between them may form a cyclic lactone containing 4 to 5 carbon atoms; and pharmaceutically acceptable salts, addition salts and solvates thereof. This lipidoid is useful as a transfection agent. The

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invention further describes transfection reagents, transfection particles containing this lipidoid, and their use.