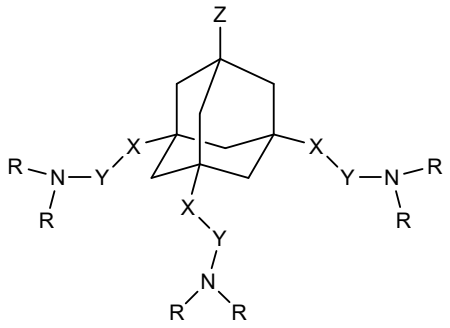
# C L A I M S

1. Lipidoids of general formula **I**



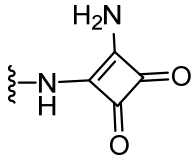
(**I**),

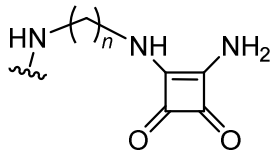
wherein X is selected from a group consisting of -C(=O)NH-, -C(=O)O-, -C(=S)O-, -C(=O)S-, -C(=S)S-, -C(=O)NHNH-, $-CH_2-$, -O-, -OC(=O)-, -S-, -SC(=O)-, -NH-, -NHNH-, -NHC(=O)-, -NHNHC(=O)-, -C≡C-, -CH=CH-, a five-membered heterocycle containing at least 2 nitrogen atoms, $-CH_2C(=O)NH-$, $-CH_2C(=O)O-$, $-CH_2C(=S)O-$, $-CH_2C(=S)S-$, $-CH_2C(=O)NHNH-$, -N=CH-, and -CH=N-;

Y is independently selected from a group consisting of alkylene chains $C_2$-$C_{10}$, wherein in the said alkylene chain, one or more $-CH_2-$ groups may optionally be replaced with one or more O or S atoms;

Z is selected from the group consisting of hydrogen, -OH, $-CH_2OH$, $-NH_2$, $-N^+(CH_3)_2-(CH_2)_3-SO_3^-$, $-N^+(CH_3)_2-(CH_2)_2-COO^-$, and $-NHCH_3$, $-N(CH_3)_2$, $-N^+(CH_3)_3$, $-OCH_3$, $-OCH_2CH_3$, $-C(=O)R^1$,

wherein $R^1$ is selected from $-NH_2$, $-NH(CH_2)_nOH$, $-N[(CH_2)_nOH]_2$, $-NHCH(CH_2OH)_2$, $-NHCH_2CH(-OH)CH_2OH$, $-NH(CH_2)_nC(=O)NH_2$, $-N[CH_2C(=O)NH_2]_2$,





$-NHCH[C(=O)NH_2]_2$, $-NH(CH_2)_2NHC(=O)NH_2$, , and ,

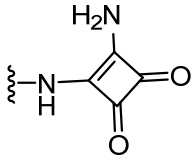
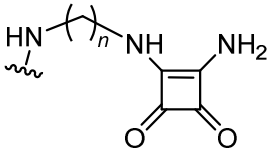
wherein *n* is an integer within the range from 2 to 5;

and R are the same or different from each other, each R being independently selected from the group consisting of alkyl $C_8$-$C_{20}$, alkenyl $C_8$-$C_{20}$, and alkynyl $C_8$-$C_{20}$, wherein in the said alkyl, alkenyl or alkynyl, one or more $-CH_2-$ groups may optionally be replaced with one or more groups selected from -CH(OH)-, -OC(=O)-, -C(=O)O-, -S-S-, -C(=O)NH-, -NHC(=O)-, -O-, and -S-;

and pharmaceutically acceptable salts, addition salts and solvates thereof.

2. The lipidoid according to claim 1, wherein Z is selected from a group consisting of hydrogen, -OH, $-CH_2OH$, $-NH_2$, $-N^+(CH_3)_2-(CH_2)_3-SO_3^-$, $-N^+(CH_3)_2-(CH_2)_2-COO^-$, and $-C(=O)R^1$,

wherein $R^1$ is selected from $-NH_2$, $-NH(CH_2)_nOH$, $-N[(CH_2)_nOH]_2$, $-NHCH(CH_2OH)_2$,

$-NHCH_2CH(OH)CH_2OH$, $-NH(CH_2)_nC(=O)NH_2$, $-N[CH_2C(=O)NH_2]_2$, $-NHCH[C(=O)NH_2]_2$,  

$-NH(CH_2)_2NHC(=O)NH_2$, , and , wherein *n* is an integer within the range from 2 to 5.

3. The lipidoid according to claim 1 or 2, wherein X is selected from -C(=O)NH-, a five membered heterocycle containing at least 2 nitrogen atoms, or -C(=O)O-.

4. The lipidoid according to any one of the preceding claims, wherein R is independently selected from the group consisting of $C_8$-$C_{20}$ alkyl, $C_8$-$C_{20}$ alkenyl, and $C_8$-$C_{20}$ alkynyl, wherein in the said alkyl, alkenyl or alkynyl, one or more $-CH_2-$ groups may optionally be replaced by one or more groups selected from -CH(OH)-, -OC(=O)-, and -C(=O)O-.

5. A lipidoid according to any one of the preceding claims, wherein all R in the molecule are the same, or all nitrogen atoms in the molecule are substituted identically by two identical R or two different R.

6. A transfection agent comprising at least one lipidoid of general formula I according to any one of claims 1 to 5 in an amount of 10 to 50 mol. %, and at least one helper lipid in a total amount of 50 to 90 mol. %.

7. A transfection agent according to claim 6, comprising at least one lipidoid of general formula I according to any one of claims 1 to 5 in an amount of 15 to 30 mol. %, cholesterol in an amount of 30 to 55 mol. %, and at least one further helper lipid in an amount of 20 to 50 mol. %.

8. A transfection particle comprising at least one lipidoid of general formula I according to any one of claims 1 to 5, at least one nucleic acid and/or a part thereof and/or nucleic acid derivative, and preferably also at least one helper lipid.

9. Use of the lipidoid of general formula I according to any one of claims 1 to 5 or the transfection agent according to claim 6 or 7, or the transfection particle according to claim 8 for *in vitro* transfection of cells or tissues with nucleic acid and/or a part thereof and/or nucleic acid derivative.

10. Use according to claim 9 for silencing or activating chromosomal gene(s), silencing or activating immunogens, inhibiting or activating signaling pathways, editing genome or transcriptome, or enabling the expression of the protein(s) encoded by the nucleic acid.

11. The lipidoid of formula I according to any one of claims 1 to 5 or the transfection agent according to claim 6 or 7, or the transfection particle according to claim 8 for use in transfecting cells or tissues with nucleic acid and/or a part thereof and/or nucleic acid derivative *in vivo*, except for the transfection of human embryos for use in industrial or commercial purposes and except for the modification of a human germ line.

12. The lipidoid of formula I according to any one of claims 1 to 5 or the transfection agent according to claim 6 or 7, or the transfection particle according to claim 8 for use according to claim 11 for silencing or activating chromosomal genes(s), silencing or activating immunogens, inhibiting or activating signaling pathways, editing genome or transcriptome, or enabling the expression of the protein(s) encoded by the nucleic acid.

13. The lipidoid of formula I according to any one of claims 1 to 5 or the transfection agent according to claim 6 or 7, or the transfection particle according to claim 8 for use as a medicament, in particular for gene therapy, preferably for the treatment of malignancies and/or genetic disorders.

14. Use of the lipidoid of general formula I according to any one of claims 1 to 5 or the transfection agent according to claim 6 or 7, or the transfection particle according to claim 8 in cosmetic preparations for delivering the active ingredient to the site of action.