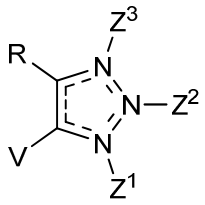
CLAIMS

1. A composition comprising (i) at least one compound of general formula (III) or a tautomer, mesomer, racemate, enantiomer, diastereomer, or mixture thereof, or an acceptable salt thereof, and (ii) an acceptable excipient, buffering agent, cell culture medium, or transfection medium:



(III)

wherein:

- **Z^1** represents H, X_1-R_3-X_2-P^+, X_1-R_3-P^+, X_1-X_2-P^+, R_3-X_2-P^+, X_1-P^+, R_3-P^+, or X_2-P^+; or **Z^1** is absent;
- **Z^2** represents H, a linear or branched, saturated or unsaturated C_1-C_{18} alkyl, C_6-C_{18} aryl, a linear or branched, saturated or unsaturated C_6-C_{18} aryl-C_1-C_{18} alkyl, a linear or branched, saturated or unsaturated C_2-C_{18} heteroalkyl, C_5-C_{10} heteroaryl, halogen, OH, a linear or branched, saturated or unsaturated C_1-C_{18} alkylamine, a C_1-C_{12} alkoxy, a linear or branched, saturated or unsaturated C_1-C_{18} alkyl-C_1-C_{12} alkoxy, X_1-R_3-X_2-P^+, X_1-R_3-P^+, X_1-X_2-P^+, R_3-X_2-P^+, X_1-P^+, R_3-P^+, or X_2-P^+; or **Z^2** is absent;
- **Z^3** represents H, a linear or branched, saturated or unsaturated C_1-C_{18} alkyl, C_6-C_{18} aryl, a linear or branched, saturated or unsaturated C_6-C_{18} aryl-C_1-C_{18} alkyl, C_5-C_{10} heteroaryl, a linear or branched, saturated or unsaturated C_2-C_{18} heteroalkyl, C_2-C_{18} alkylidene, OH, guanidine, halogen, X_1-R_3-X_2-P^+, X_1-R_3-P^+, X_1-X_2-P^+, R_3-X_2-P^+, X_1-P^+, R_3-P^+, or X_2-P^+; or **Z^3** is absent;
- **X_1** and **X_2**, which may be identical or different, represent CO or CH_2;
- **R_3** represents $(CH_2)_m$, $(CH_2)_m$-$CHCH_3$-$(CH_2)_n$-, $(CH_2)_m$-$C(CH_3)_2$-$(CH_2)_n$-, $(CH_2)_m$-O-$(CH_2)_n$-, $(CH_2)_m$-S-$(CH_2)_n$-, $(CH_2)_m$-CH_2-O-, with m representing an integer between 1 and 3 and n representing an integer between 1 and 3;
- **P^+** represents a graft cationic polymer, which is a polyamine comprising secondary amines, tertiary amines, a mixture of primary and secondary amines, a mixture of primary and tertiary amines, a mixture of secondary and tertiary amines, or a mixture of primary, secondary and tertiary amines;
- **R** or **V** represents H, a linear or branched, saturated or unsaturated C_1-C_{18} alkyl or cycloalkyl, a C_6-C_{18} aryl, a linear or branched, saturated or unsaturated C_6-C_{18} aryl-C_1-

C_{18} alkyl, a linear or branched, saturated or unsaturated C_2-C_{18} heteroalkyl, a linear or branched, saturated or unsaturated C_1-C_{24} ester, a C_5-C_{10} heterocyclyl, a C_5-C_{10} heteroaryl, a linear or branched, saturated or unsaturated C_1-C_{18} alkyl-C_5-C_{10} heteroaryl, X_1-R_3-X_2-P^+, X_1-R_3-P^+, X_1-X_2-P^+, R_3-X_2-P^+, X_1-P^+, R_3-P^+, or X_2-P^+;

with the provisos that:

- at least one of $\mathbf{Z^1}$, $\mathbf{Z^2}$ or $\mathbf{Z^3}$ is present; and
- only one of $\mathbf{Z^1}$, $\mathbf{Z^2}$, $\mathbf{Z^3}$, **R** or **V** represents X_1-R_3-X_2-P^+, X_1-R_3-P^+, X_1-X_2-P^+, R_3-X_2-P^+, X_1-P^+, R_3-P^+, or X_2-P^+.

2. The composition according to claim 1, further comprising at least one nucleic acid molecule to be transfected in a cell, preferably a nucleic acid molecule selected from the group consisting of a deoxyribonucleic acid (DNA), a ribonucleic acid (RNA), a DNA/RNA hybrid, a short interfering RNA (siRNA), a microRNA (miRNA), a short hairpin RNA (shRNA), a messenger RNA (mRNA), a CRISPR guide RNA, more preferably a DNA, and an expression vector encoding said nucleic acid molecule, in particular a plasmid encoding said nucleic acid molecule or a plasmid expressing said nucleic acid molecule.

3. The composition according to claim 1 or 2, wherein **R** or **V** represents H, methyl, ethyl, propyl, cyclopropyl, isopropyl, sec-butyl, cyclopentyl, phenyl, fluorophenyl, benzyl, pyridine, 2-pyridine, 3-pyridine, fluorobenzyl, substituted morpholinyl, substituted piperazinyl, 4-hydroxybenzyl, or 4-hydroxyphenethyl; more preferably **R** or **V** represents methyl, ethyl, propyl, cyclopropyl, isopropyl, sec-butyl, cyclopentyl, phenyl, benzyl, fluorobenzyl, 4-hydroxyphenethyl, 2-pyridine or 3-pyridine.

4. The composition according to any one of claims 1 to 3, wherein:

(i) only one of $\mathbf{Z^1}$, $\mathbf{Z^2}$ or $\mathbf{Z^3}$ represents X_1-R_3-X_2-P^+, X_1-R_3-P^+, X_1-X_2-P^+, R_3-X_2-P^+, X_1-P^+, R_3-P^+, or X_2-P^+, wherein $\mathbf{X_1}$, $\mathbf{X_2}$, $\mathbf{R_3}$ and $\mathbf{P^+}$ are as defined in claim 1; preferably only one of $\mathbf{Z^1}$, $\mathbf{Z^2}$ or $\mathbf{Z^3}$ represents X_1-R_3-X_2-P^+, wherein $\mathbf{X_1}$ represents CH_2, $\mathbf{X_2}$ represents CO, and $\mathbf{R_3}$ represents $(CH_2)_m$, with m representing an integer between 1 and 3, preferably m is equal to 2; and/or

(ii) $\mathbf{Z^1}$ represents H; and/or

(iii) $\mathbf{Z^2}$ represents H, a C_1-C_{12} alkoxy, or a linear or branched, saturated or unsaturated C_1-C_{18} alkyl, preferably a linear or branched, saturated or unsaturated C_1-C_6 alkyl; more preferably $\mathbf{Z^2}$ represents H, CH_3, CF_3 or OCH_3; and/or

(iv) $\mathbf{Z^3}$ represents H, or a linear or branched, saturated or unsaturated C_1-C_{18} alkyl, preferably a linear or branched, saturated or unsaturated C_1-C_6 alkyl.

5. The composition according to any one of claims 1 to 4, wherein:
if (i) $\mathbf{Z^1}$ represents X_1-R_3-X_2-P^+, X_1-R_3-P^+, X_1-X_2-P^+, R_3-X_2-P^+, X_1-P^+, R_3-P^+, or X_2-P^+, preferably X_1-R_3-X_2-P^+, wherein $\mathbf{X_1}$, $\mathbf{X_2}$, $\mathbf{R_3}$ and $\mathbf{P^+}$ are as defined herein; more preferably $\mathbf{Z^1}$ represents X_1-R_3-X_2-P^+, wherein $\mathbf{X_1}$ represents CH_2, $\mathbf{X_2}$ represents CO, and $\mathbf{R_3}$ represents $(CH_2)_m$, with m representing an integer between 1 and 3, preferably m is equal to 2 then
(ii) $\mathbf{Z^2}$ represents H, a C_1-C_{12} alkoxy, or a linear or branched, saturated or unsaturated C_1-C_{18} alkyl, preferably a linear or branched, saturated or unsaturated C_1-C_6 alkyl; more preferably $\mathbf{Z^2}$ represents H, CH_3, CF_3 or OCH_3; and/or
(iii) $\mathbf{Z^3}$ represents H, a linear or branched, saturated or unsaturated C_1-C_{18} alkyl, preferably a linear or branched, saturated or unsaturated C_1-C_6 alkyl, or a linear or branched, saturated or unsaturated C_6-C_{18} aryl-C_1-C_{18} alkyl, preferably fluorobenzyl or 4-hydroxyphenethyl; and/or
(iv) **R** or **V** represents H, a linear or branched, saturated or unsaturated C_1-C_{18} alkyl or cycloalkyl, a C_6-C_{18} aryl, a linear or branched, saturated or unsaturated C_6-C_{18} aryl-C_1-C_{18} alkyl, a linear or branched, saturated or unsaturated C_2-C_{18} heteroalkyl, a linear or branched, saturated or unsaturated C_1-C_{24} ester, a C_5-C_{10} heterocyclyl, a C_5-C_{10} heteroaryl, or a linear or branched, saturated or unsaturated C_1-C_{18} alkyl-C_5-C_{10} heteroaryl.

6. The composition according to any one of claims 1 to 5, wherein:
if (i) $\mathbf{Z^2}$ represents X_1-R_3-X_2-P^+, X_1-R_3-P^+, X_1-X_2-P^+, R_3-X_2-P^+, X_1-P^+, R_3-P^+, or X_2-P^+, preferably X_1-R_3-X_2-P^+, wherein $\mathbf{X_1}$, $\mathbf{X_2}$, $\mathbf{R_3}$ and $\mathbf{P^+}$ are as defined herein; more preferably $\mathbf{Z^2}$ represents X_1-R_3-X_2-P^+, wherein $\mathbf{X_1}$ represents CH_2, $\mathbf{X_2}$ represents CO, and $\mathbf{R_3}$ represents $(CH_2)_m$, with m representing an integer between 1 and 3, preferably m is equal to 2 then
(ii) $\mathbf{Z^1}$ represents H; and/or
(iii) $\mathbf{Z^3}$ represents H, a linear or branched, saturated or unsaturated C_1-C_{18} alkyl, preferably a linear or branched, saturated or unsaturated C_1-C_6 alkyl, or a linear or branched, saturated or unsaturated C_6-C_{18} aryl-C_1-C_{18} alkyl, preferably fluorobenzyl or 4-hydroxyphenethyl; and/or
(iv) **R** or **V** represents H, a linear or branched, saturated or unsaturated C_1-C_{18} alkyl or cycloalkyl, a C_6-C_{18} aryl, a linear or branched, saturated or unsaturated C_6-C_{18} aryl-C_1-C_{18} alkyl, a linear or branched, saturated or unsaturated C_2-C_{18} heteroalkyl, a linear or branched, saturated or unsaturated C_1-C_{24} ester, a C_5-C_{10} heterocyclyl, a C_5-C_{10} heteroaryl, or a linear or branched, saturated or unsaturated C_1-C_{18} alkyl-C_5-C_{10} heteroaryl.

7. The composition according to any one of claims 1 to 6, wherein:
if (i) $\mathbf{Z^3}$ represents X_1-R_3-X_2-P^+, X_1-R_3-P^+, X_1-X_2-P^+, R_3-X_2-P^+, X_1-P^+, R_3-P^+, or X_2-P^+, preferably X_1-R_3-X_2-P^+, wherein $\mathbf{X_1}$, $\mathbf{X_2}$, $\mathbf{R_3}$ and $\mathbf{P^+}$ are as defined herein; more preferably $\mathbf{Z^3}$ represents X_1-R_3-X_2-P^+, wherein $\mathbf{X_1}$ represents CH_2, $\mathbf{X_2}$ represents CO, and $\mathbf{R_3}$ represents $(CH_2)_m$, with m representing an integer between 1 and 3, preferably m is equal to 2 then
(ii) $\mathbf{Z^1}$ represents H; and/or
(iii) $\mathbf{Z^2}$ represents H, a C_1-C_{12} alkoxy, or a linear or branched, saturated or unsaturated C_1-C_{18} alkyl, preferably a linear or branched, saturated or unsaturated C_1-C_6 alkyl; more preferably $\mathbf{Z^2}$ represents H, CH_3, CF_3 or OCH_3; and/or
(iv) **R** or **V** represents H, a linear or branched, saturated or unsaturated C_1-C_{18} alkyl or cycloalkyl, a C_6-C_{18} aryl, a linear or branched, saturated or unsaturated C_6-C_{18} aryl-C_1-C_{18} alkyl, a linear or branched, saturated or unsaturated C_2-C_{18} heteroalkyl, a linear or branched, saturated or unsaturated C_1-C_{24} ester, a C_5-C_{10} heterocyclyl, a C_5-C_{10} heteroaryl, or a linear or branched, saturated or unsaturated C_1-C_{18} alkyl-C_5-C_{10} heteroaryl.

8. The composition according to any one of claims 1 to 7, wherein:
if (i) **R** or **V** represents X_1-R_3-X_2-P^+, X_1-R_3-P^+, X_1-X_2-P^+, R_3-X_2-P^+, X_1-P^+, R_3-P^+, or X_2-P^+, preferably X_1-R_3-X_2-P^+, wherein $\mathbf{X_1}$, $\mathbf{X_2}$, $\mathbf{R_3}$ and $\mathbf{P^+}$ are as defined herein; more preferably $\mathbf{Z^3}$ represents X_1-R_3-X_2-P^+, wherein $\mathbf{X_1}$ represents CH_2, $\mathbf{X_2}$ represents CO, and $\mathbf{R_3}$ represents $(CH_2)_m$, with m representing an integer between 1 and 3, preferably m is equal to 2 then
(ii) $\mathbf{Z^1}$ represents H; and/or
(iii) $\mathbf{Z^2}$ represents H, a C_1-C_{12} alkoxy, or a linear or branched, saturated or unsaturated C_1-C_{18} alkyl, preferably a linear or branched, saturated or unsaturated C_1-C_6 alkyl; and/or
(iv) $\mathbf{Z^3}$ represents H, a linear or branched, saturated or unsaturated C_1-C_{18} alkyl, preferably a linear or branched, saturated or unsaturated C_1-C_6 alkyl, or a linear or branched, saturated or unsaturated C_6-C_{18} aryl-C_1-C_{18} alkyl, preferably fluorobenzyl or 4-hydroxyphenethyl.

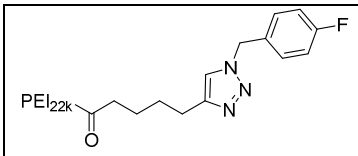
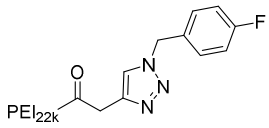
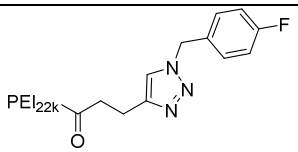
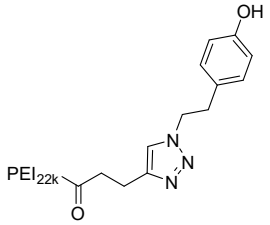
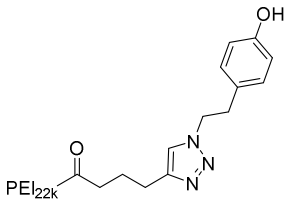
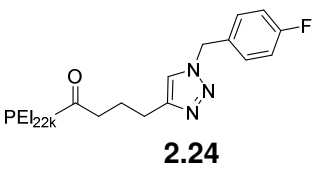
9. The composition according to any one of claims 1 to 8, wherein:
if (i) **R** or **V** represents X_1-R_3-X_2-P^+, X_1-R_3-P^+, X_1-X_2-P^+, R_3-X_2-P^+, X_1-P^+, R_3-P^+, or X_2-P^+, preferably X_1-R_3-X_2-P^+, wherein $\mathbf{X_1}$, $\mathbf{X_2}$, $\mathbf{R_3}$ and $\mathbf{P^+}$ are as defined herein; more preferably $\mathbf{Z^3}$ represents X_1-R_3-X_2-P^+, wherein $\mathbf{X_1}$ represents CH_2, $\mathbf{X_2}$ represents CO, and $\mathbf{R_3}$ represents $(CH_2)_m$, with m representing an integer between 1 and 3, preferably m is equal to 2 then

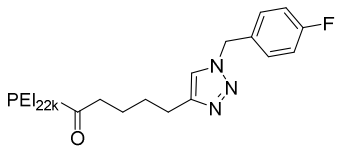
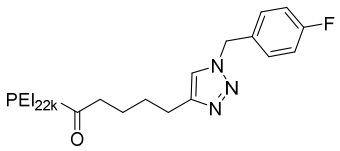
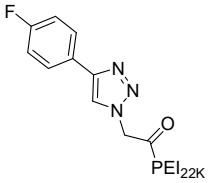
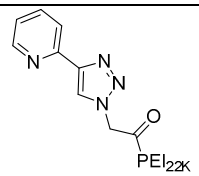
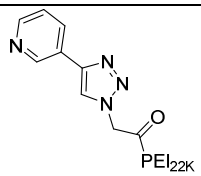
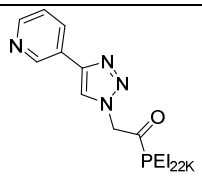
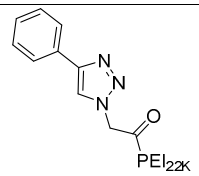
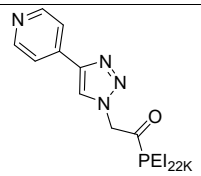
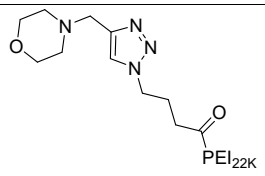
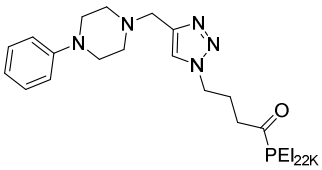
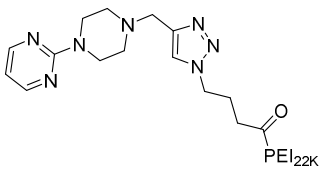
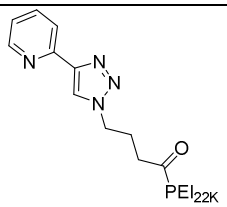
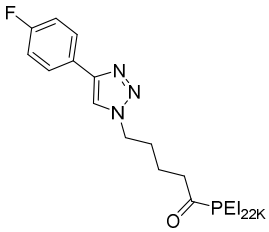
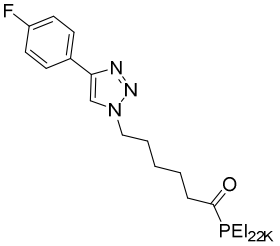
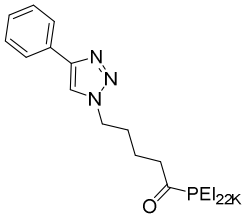
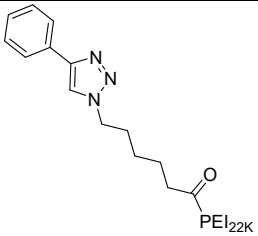
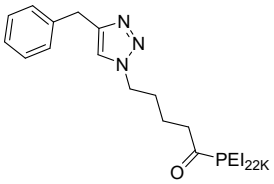
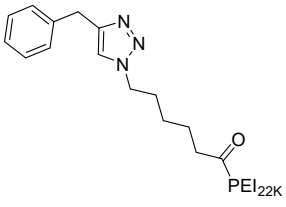
(ii) **Z^3** is present and **Z^3** represents H, a linear or branched, saturated or unsaturated C_1-C_{18} alkyl, preferably a linear or branched, saturated or unsaturated C_1-C_6 alkyl, or a linear or branched, saturated or unsaturated C_6-C_{18} aryl-C_1-C_{18} alkyl, preferably fluorobenzyl or 4-hydroxyphenethyl.

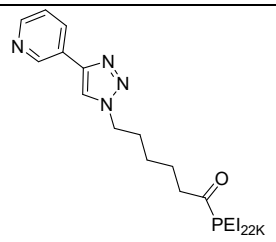
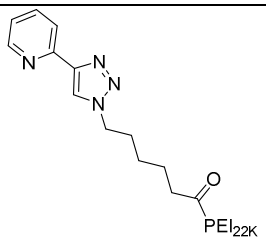
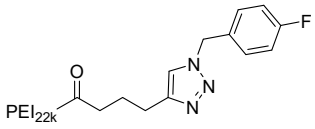
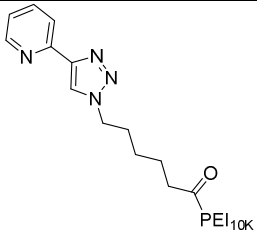
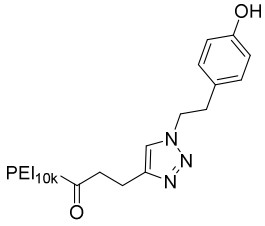
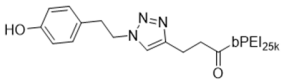
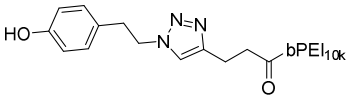
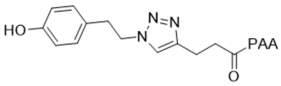
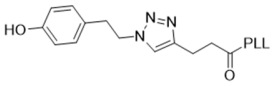
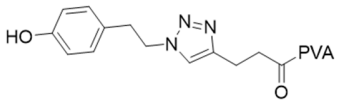
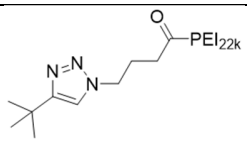
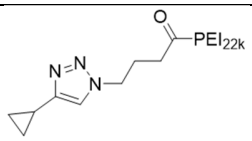
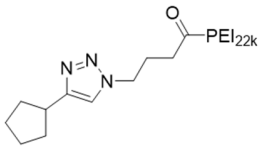
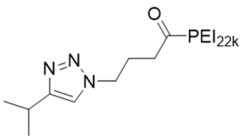
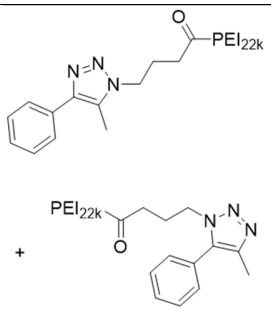
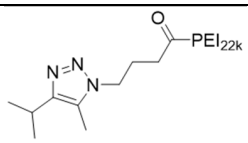
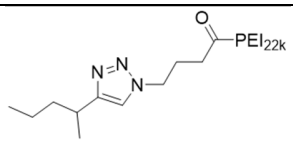
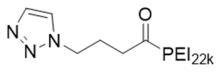
10. The composition according to any one of claims 1 to 9, wherein the graft cationic polymer is selected from the group consisting of a linear or branched polyethyleneimine (PEI), PEI dendrimers, a polypropyleneimine (PPI), Poly(amidoamine) (PAA) and dendrimers (PAMAM), cationic cyclodextrin, polyalkylamine, a polyhydroxyalkylamine, poly(butyleneimine) (PBI), spermine, a N-substituted polyallylamine, N-substituted chitosan, a N-substituted polyornithine, a N-substituted polylysine (PLL), a N-substituted polyvinylamine, poly(β-amino ester), hyperbranched poly(amino ester) (h-PAE), networked poly(amino ester) (n-PAE), poly(4-hydroxy-1-proline ester) (PHP-ester) and a poly-β-aminoacid, preferably is a linear or branched PEI, more preferably a linear PEI.

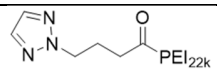
11. The composition according to any one of claims 1 to 10, wherein
(i) the graft cationic polymer has a grafting ratio ranging from 1 to 50%, preferably from 5 to 30%, more preferably is 20%; and/or
(ii) the graft cationic polymer has an average molecular weight (Mw) ranging from 1 kDa to 500 kDa, preferably from 1 kDa to 50 kDa, more preferably from 5 kDa to 50 kDa or from 1 kDa to 15 kDa; in particular the graft cationic polymer has an average molecular weight (Mw) of 6, 8, 10, 15, 22 or 30 kDa, preferably of 6, 8, 10, 15 or 30 kDa.

12. The composition according to any one of claims 1 to 11, wherein the at least one compound of general formula (III) is selected from the group consisting of the following compounds:

 **2.19**	 **2.20**	 **2.21**
 **2.22**	 **2.23**	 **2.24**

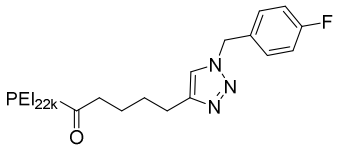
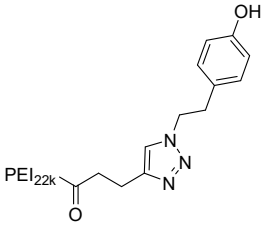
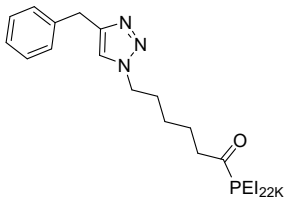
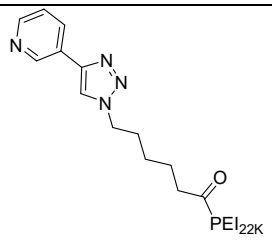
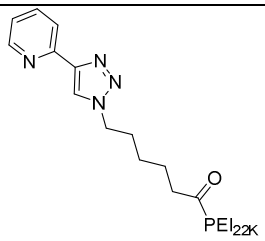
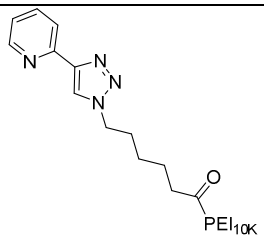
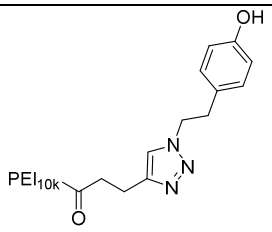
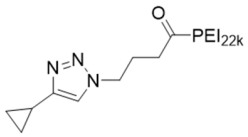
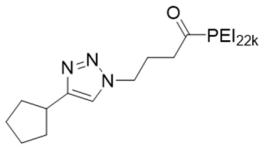
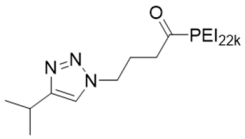
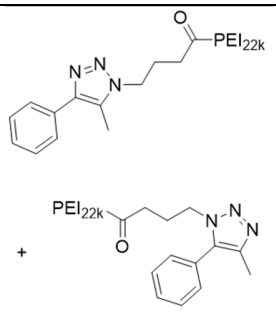
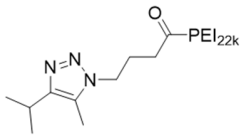
 **2.25**	 **2.26**	 **2.27**
 **2.28**	 **2.29**	 **2.30**
 **2.31**	 **2.32**	 **2.33**
 **2.34**	 **2.35**	 **2.36**
 **2.37**	 **2.38**	 **2.39**
 **2.40**	 **2.41**	 **2.42**

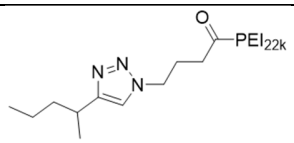
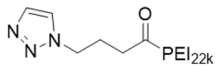
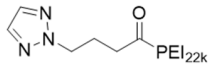
 **2.43**	 **2.44**	 **2.45**
 **2.46**	 **2.47**	 **2.48**
 **2.49**	 **2.50**	 **2.51**
 **2.52**	 **2.53**	 **2.54**
 **2.55**	 **2.56**	 **2.57**
 **2.58**	 **2.59**	 **2.60**



2.61

13. The composition according to claim 12, wherein the at least one compound of general formula (III) is selected from the group consisting of the following compounds:

 **2.19**	 **2.22**	 **2.42**
 **2.43**	 **2.44**	 **2.46**
 **2.47**	 **2.54**	 **2.55**
 **2.56**	 **2.57**	 **2.58**

 **2.59**	 **2.60**	 **2.61**

14. The composition according to claim 13, wherein the at least one compound of general formula (III) is compound **2.22**.

15. A method for *in vitro* or *ex vivo* transfection of live cells comprising introducing in the cells the composition according to any one of claims 2 to 14.

16. *In vitro* or *ex vivo* use of the composition according to any one of claims 2 to 14 to transfect at least one nucleic acid molecule into a cell, cell line or cells, preferably a cell, cell line or cells selected from the group consisting of a mammalian cell, an insect cell, a primary cell, an adherent cell, a suspension cell, a dividing cell such as a stem cell, a non-dividing cell such as a neuronal cell, and a cancer cell, said cell, cell line or cells being optionally organized into spheroids, organoids, 2D or 3D cell culture, or provided as fibre or matrix culture, and/or within a bioreactor.

17. *In vitro* or *ex vivo* use of the composition according to any one of claims 2 to 14 for genome engineering, for cell reprogramming, for differentiating cells, or for gene-editing.

18. An *in vitro* method for the production of (i) biologics, in particular biologics encoding a recombinant protein, peptide or antibody; or (ii) recombinant virus, such as adeno-associated virus (AAV), lentivirus (LV), adenovirus, oncolytic virus, or baculovirus, wherein the composition according to any one of claims 2 to 14 comprises multiple nucleic acid molecules for co-transfection; or (iii) viral or virus-like particles, wherein the composition according to any one of claims 2 to 14 comprises multiple nucleic acid molecules for co-transfection.

19. The *in vitro* method according to claim 18,
(i) for the production of AAV, said composition comprising (i) at least one compound selected from the group consisting of compounds **2.22**, **2.23**, **2.43**, **2.44, 2.47**, **2.54**, **2.57**, **2.60** and **2.61** and (ii) an acceptable excipient, buffering agent, cell culture medium, or transfection medium; or
(ii) for the production of LV, said composition comprising (i) at least the compound **2.22**, and (ii) an acceptable excipient, buffering agent, cell culture medium, or transfection medium; or

(iii) for the production of recombinant virus, said composition comprising a plurality of expression vectors such as plasmid vectors to transfect in an adherent or suspension cell, such as HEK293, HeLa, BHK-21, A549 or insect cells, wherein said vectors, in particular plasmids, are construct expressing viral structural sequences and transfer vector genome for virus or virus-like production and optionally expressing molecules of interest encoded by the transfer vector genome.

20. The *in vitro* method for the production of recombinant virus according to claim 19, wherein said recombinant virus is for use in *in vivo* applications for cell therapy or for gene therapy.