CLAIMS

1. A modulator of the function of a toxic disease-inducing RNA, wherein the toxic disease-inducing RNA comprises expanded repeat sequences $r(CUG)^{exp}$ or $r(CCUG)^{exp}$, the modulator comprising an RNA-binding oligomer that is formed *in situ* within a living cell from:

(a) a condensation reaction of a cell-permeable RNA expanded repeat sequence binding module, wherein the cell-permeable RNA expanded repeat sequence binding module comprises both an alkyne and an azide moiety, and is (i) N_3-K-Ak, denoting a dimeric 6'-*N*-acylated kamamycin A compound that displays both a 6" azide and a 6' alkyne, or is (ii) N_3-K-AaK, denoting a dimeric 6'-*N*-acylated kanamycin A compound that displays both a 6" azide and a 6' activated, electron deficient alkyne, or is (iii) N_3-2H-K4NMeS-Aak, denoting a bis-benzimidazole moiety that contains a H RNA binding module displayed on a *N*-methyl peptide backbone, that comprises both an azide group and an activated, electron deficient alkyne group; or

(b) a condensation reaction of a pair of cell-permeable RNA expanded repeat sequence binding modules, wherein a first module comprises an alkyne moiety and is K-Ak, denoting a dimeric 6'-*N*-acylated kanamycin A compound with an alkyne group at 6'-position, and a second module comprises an azide moiety and is N_3-K, denoting a dimeric 6'-N-acylated kanamycin A compound with an azido group at 6" position;

wherein the condensation reaction takes place between the RNA expanded repeat bound modules via a 1,3 Huisgen dipolar cyclo-addition reaction between the alkyne and azide moieties.

2. The modulator of claim 1, wherein the toxic RNA comprises adjacent internal loops formed by RNA expanded repeat sequences $r(CCUG)^{exp}$.

3. The modulator of claim 1 or 2, wherein the cell-permeable module is N_3-K-Ak, or N_3-K-AaK, or a pair of cell-permeable modules used together are N_3-K and K-Ak.

4. The modulator of claim 2 or 3 for use in the treatment of myotonic dystrophy type 2 (DM2).

5. The modulator of claim 1, wherein the toxic RNA comprises adjacent internal loops formed by RNA expanded repeat $r(CUG)^{exp}$.

6. The modulator of claim 5, wherein the cell permeable module is N_3-2H-K4NMeS-Aak.

7. The modulator of claim 5 or 6, for use in the treatment of myotonic dystrophy type 1 (DM1).

8. The modulator of any one of claims 1 to 3, 5 or 6, for use as a medicament.

9. The modulator of any one of claims 1 to 3, 5 or 6, for use in treating a disease in a patient wherein the disease is induced by the presence of a toxic RNA having expanded repeat sequences.

10. The modulator of any one of claims 1 to 3, 5 or 6, for use in the treatment of myotonic dystrophy type 2 (DM2) or myotronic dystrophy type 1 (DM1).

11. The modulator comprising a modulator of any one of claims 1 to 3, 5 or 6 further comprising a nucleic acid cleaving moiety for use in cleaving a toxic disease-inducing RNA, wherein the RNA comprises expanded repeat sequences.

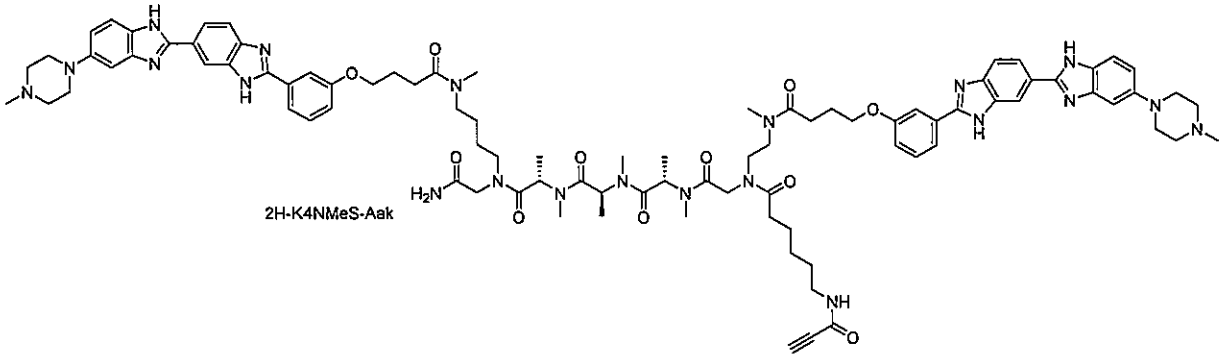
12. The modulator of claim 11, wherein the nucleic acid cleaving moiety comprises a bleomycin.

13. Use of a modulator of claim 1 in the manufacture of a medicament for the treatment of a disease in a patient wherein the disease is induced by the presence of a toxic RNA having expanded repeat sequences, wherein the disease is myotonic dystrophy type 2 (DM2), the toxic RNA comprises $r(CCUG)^{exp}$ expanded repeat sequences, and the modulator is formed *in situ* from one cell-permeable module comprising a kanamycin bearing an alkyne and an azide group, or a pair of cell-permeable modules, each comprising a kanamycin, a first module bearing an alkyne group and a second module bearing an azide group.

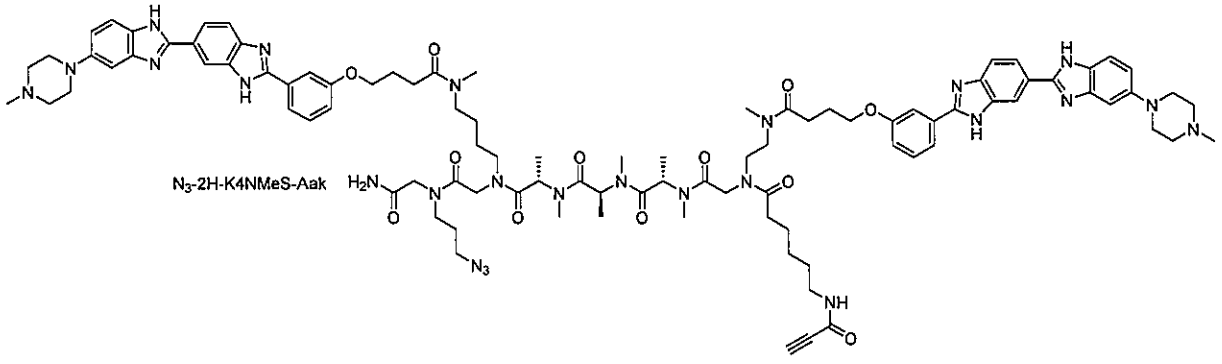
14. Use of a modulator of claim 1 in the manufacture of a medicament for the treatment of a disease in a patient wherein the disease is induced by the presence of a toxic RNA having expanded repeat sequences, wherein the disease is myotonic dystrophy type 1 (DM1), the toxic RNA comprises r(CUG)^{exp} expanded repeat sequences, and the modulator is formed *in situ* from one cell-permeable module bearing an alkyne and an azide group, or a pair of cell-permeable modules each comprising a bis-benzimidazole, a first module bearing an alkyne group and a second module bearing an azide group.

15. The use of claim 13 or claim 14, wherein the modulator further comprises a nucleic acid cleaving moiety.

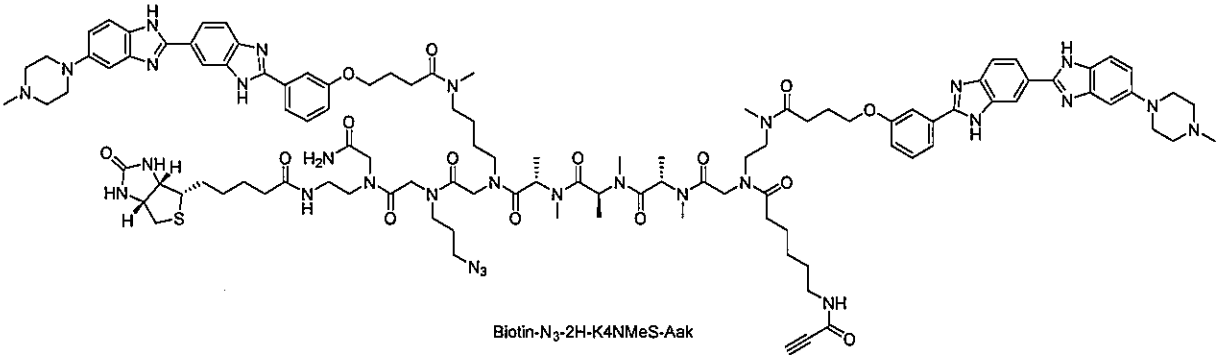
16. A compound having a structure of 2H-K4NMeS-Aak, N_3-2H-K4NMeS-Aak, Biotin-N_3-2H-K4NMeS-Aak, FAM-2H-K4NMe-S-Aak, or N_3-2H-K4NMe-S-TAMRA:



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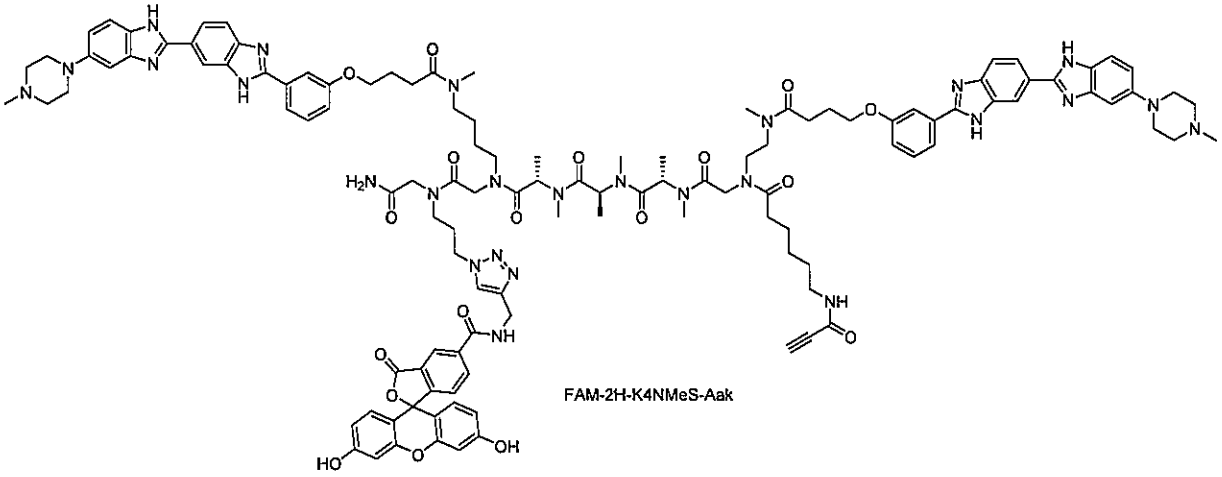


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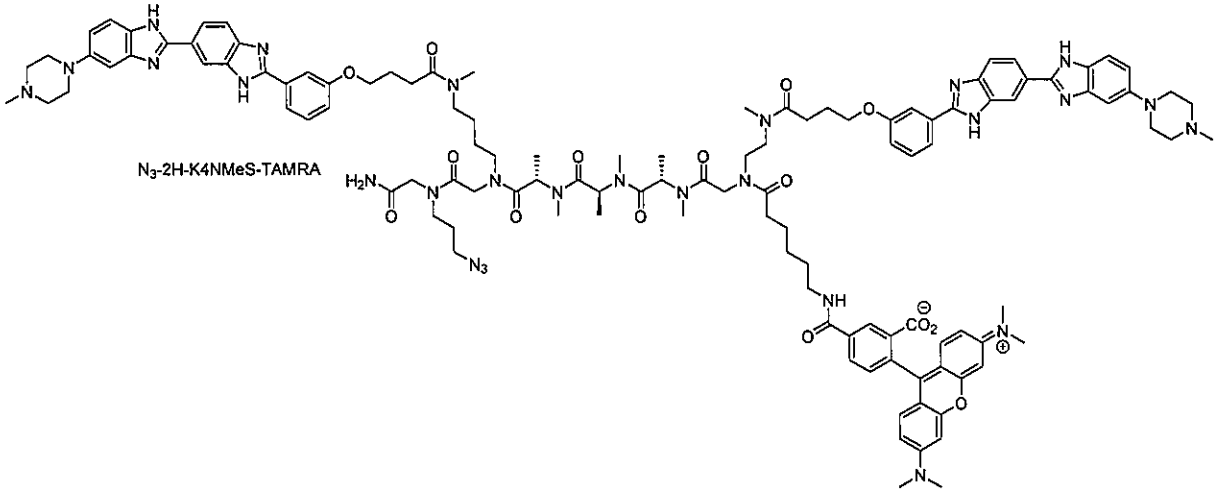
Biotin-N_3-2H-K4NMeS-Aak

;



FAM-2H-K4NMeS-Aak

; or



N_3-2H-K4NMeS-TAMRA

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17. A product comprising a condensation reaction of the compound of claim 16, wherein either the condensation reaction is (1) from a plurality of the compound of claim 16 having each of an alkyne moiety and an azide moiety or (2) from a pair of compounds of claim 16, the first compound having an alkyne moiety and the second

compound having an azide moiety, or a combination thereof, the condensation reaction taking place via a 1,3 Huisgen dipolar cycloaddition reaction between the alkyne and azide moieties.

18. The product of claim 17, comprising a condensation reaction between a plurality of N_3-2H-K4NMeS-Aak.

19. The product of claim 17, comprising a condensation reaction between FAM-2H-K4NMeS-Aak and N_3-2H-K4NMeS-TAMRA.

20. A composition comprising the product of claim 17 bound to RNA expanded repeat sequences $r(CCUG)^{exp}$ or $r(CUG)^{exp}$.

21. The composition of claim 20, wherein the RNA expanded repeat sequence is $r(CUG)^{exp}$.

22. The composition of claim 20, wherein the RNA expanded repeat sequence is $r(CCUG)^{exp}$.

23. A method of making the product of claim 17, comprising contacting a living cell having RNA expanded repeat sequences $r(CCUG)^{exp}$ or $r(CUG)^{exp}$ with (1) the plurality of the compound of claim 1, (2) the pair of compounds of claim 1, or a combination thereof, such that the compounds of claim 1 bind to the RNA expanded repeat sequences $r(CCUG)^{exp}$ or $r(CUG)^{exp}$ and react via the 1,3 Huisgen dipolar cycloaddition reaction between the alkyne and azide moieties to form the product.

24. A method of monitoring formation of the product of claim 19 comprising contacting RNA expanded repeat sequences $r(CCUG)^{exp}$ or $r(CUG)^{exp}$ with FAM-2H-K4NMeS-Aak and N_3-2H-K4NMeS-TAMRA and measuring changes in FRET, wherein a change in FRET occurs upon formation of the product of claim 18.

25. The method of claim 24, wherein the RNA expanded repeat sequence is r(CUG)^{exp}.

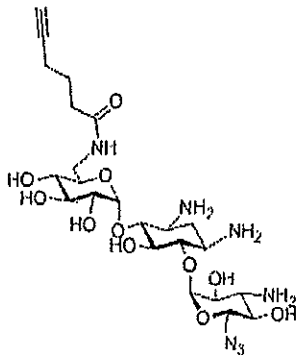
26. The method of claim 24, wherein the RNA expanded repeat sequence is r(CCUG)^{exp}.

27. Use of a plurality of a compound of claim 16, when the compound comprises each of an alkyne moiety and an azide moiety or use of a pair of compounds of claim 16, when a first compound of the pair comprises an alkyne moiety and a second compound of the pair comprises an azide moiety, or use of a combination thereof, in the manufacture of a medicament for the treatment of a patient suffering from myotonic dystrophy 1 (DM1).

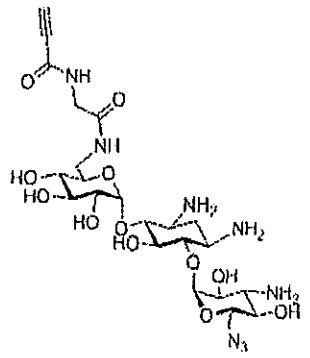
28. Use according to claim 27, wherein the medicament is in the form of a condensation product, wherein the plurality of compounds or the pair of compounds bind to an RNA expanded repeat sequence r(CUG)^{exp} and upon binding react via a 1,3 Huisgen dipolar cycloaddition reaction between the alkyne and azide moieties.

29. A pharmaceutical composition comprising a cell-permeable RNA expanded repeat sequence binding module or a pair of cell-permeable RNA expanded repeat sequence binding modules, which forms within the patient's cells a modulator of the function of a toxic disease-inducing RNA, wherein the disease is myotonic dystrophy type 2 (DM2) or myotonic dystrophy type 1 (DM1), wherein the RNA comprises expanded repeat sequences comprising adjacent internal loops formed by RNA expanded repeat sequence r(CCUG)^{exp} or RNA expanded repeat sequence r(CUG)^{exp}, respectively, the modulator comprising an RNA-binding oligomer that is formed *in situ* within a living cell from a condensation reaction of the cell-permeable RNA expanded repeat sequence binding module, wherein the cell-permeable module comprises both an alkyne and an azide moiety, or from a condensation of the pair of cell-permeable RNA expanded repeat sequence binding modules, wherein a first module comprises an alkyne moiety and a second module comprises an azide moiety, the condensation

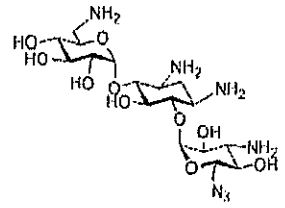
reaction taking place between the RNA expanded repeat bound modules via an a 1,3 Huisgen dipolar cycloaddition reaction between the alkyne and azide moieties, wherein the cell-permeable module is N_3-K-Ak as shown below,



or is N_3-K-AaK as shown below,



or a pair of cell-permeable modules used together are
N_3-K as shown below and



K-Ak as shown below.

30. The pharmaceutical composition of claim 29 for forming the modulator of claim 29 bound to the toxic RNA, by exposing the living cell comprising the toxic RNA to the

cell-permeable RNA expanded repeat sequence binding module of claim 29, or to the pair of cell-permeable RNA expanded repeat sequence binding modules of claim 29.

31. The pharmaceutical composition according to claim 29 for cleaving a toxic disease-inducing RNA, wherein the RNA comprises expanded repeat sequences, the modulator further comprising a nucleic acid cleaving moiety.

32. The pharmaceutical composition of claim 31 wherein the nucleic acid cleaving moiety is a derivative of bleomycin.

33. A pharmaceutical composition according to claim 29 for treating myotonic dystrophy type 2 (DM2) in a patient wherein the disease is induced by the presence of a toxic RNA wherein the toxic RNA comprises r(CCUG)^{exp} expanded repeat sequences, or myotonic dystrophy type 1 (DM1) in a patient wherein the disease is induced by the presence of a toxic RNA wherein the toxic RNA comprises r(CUG)^{exp} expanded repeat sequences.

34. The pharmaceutical composition of claim 33, wherein the modulator further comprises a nucleic acid cleaving moiety