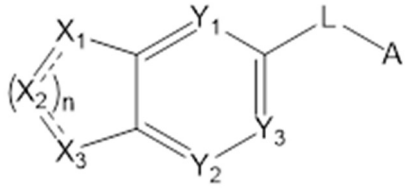
THAT WHICH IS CLAIMED:

1. A compound with a structure of formula (I):



Formula (I)

wherein

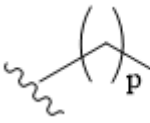
X_1, X_2, and X_3 are, in each instance, independently selected from CR_1, CHR_1, N, NH, O and S, wherein adjacent X_1, X_2, and X_3 are not simultaneously selected to be O or S;

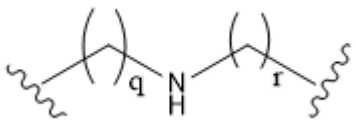
the dashed lines represent optional double bonds;

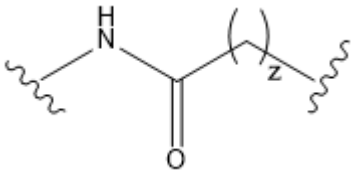
Y_1, Y_2, and Y_3 are, in each instance, independently selected from CR_2 and N;

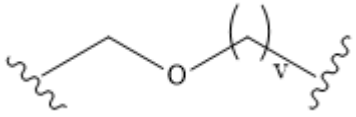
n is 1 or 2, wherein when n is 1, only one of the dashed lines is a double bond;

L is selected from



,


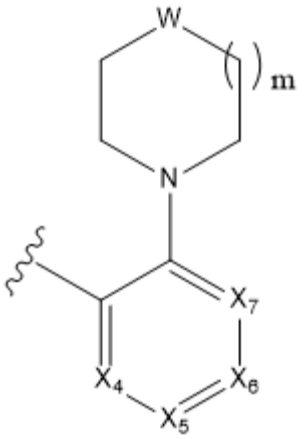
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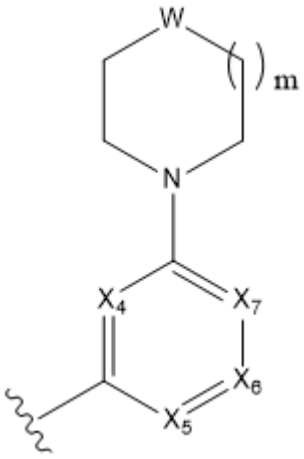
, and


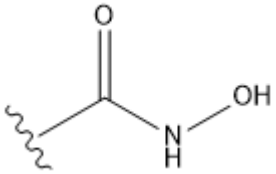
,

wherein p, q, r, and v are independently selected from integers 0, 1, 2, 3, 4, 5, 6, 7, 8, 9, and 10, and z is selected from integers 1, 2, 3, 4, and 5; and

A is selected from



, 

, and 

,

wherein X_4, X_5, X_6, and X_7, are independently selected from CR_3 and N;

wherein R_1, R_2, and R_3 are independently selected from -H, -Cl, -Br, -I, -F, $-CF_3$, -OH, -CN, $-NO_2$, $-NH_2$, $-NH(C_1\text{-}C_6$ alkyl), $-N(C_1\text{-}C_6$ alkyl)$_2$, -COOH, $-COO(C_1\text{-}C_6$ alkyl), $-CO(C_1\text{-}C_6$ alkyl), $-O(C_1\text{-}C_6$ alkyl), $-OCO(C_1\text{-}C_6$ alkyl), $-NCO(C_1\text{-}C_6$ alkyl), $-CONH(C_1\text{-}C_6$ alkyl), and substituted or unsubstituted $C_1\text{-}C_6$ alkyl;

m is 1 or 2;

W is –O or $–NR_4$, wherein R_4 is selected from selected from -H, $-CO(C_1\text{-}C_6$ alkyl), substituted or unsubstituted $C_1\text{-}C_6$ alkyl, substituted or unsubstituted aryl, substituted or unsubstituted heteroaryl, substituted or unsubstituted cycloalkyl, -CO(aryl), -CO(heteroaryl), and -CO(cycloalkyl); and

provided that at least two of X_1, X_2, X_3, X_4, X_5, X_6, and X_7 are N;

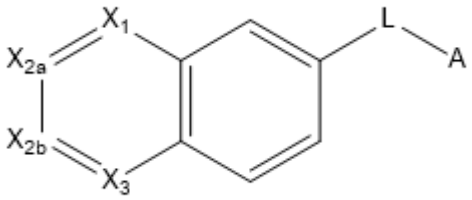
or a pharmaceutically acceptable salt thereof.

2. The compound of claim 1, wherein at least one of X_1, X_2, or X_3 is N.

3. The compound of claim ~~1 or~~ 2, wherein n is 2.

~~4. The compound of any one of claims 1 to 3, wherein, in each instance, two of X_1, X_2, and X_3 are N.~~

4~~5~~. The compound of ~~any one of~~ claim~~s~~ 1 ~~to 4~~, having the structure of formula (II):



,

Formula (II)

wherein

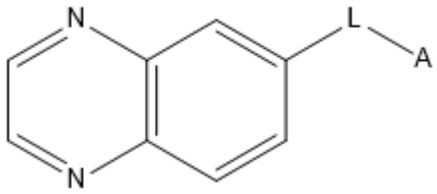
X_{2a} and X_{2b} are independently selected from CR_1 and N;

X_1 and X_3 are independently selected from CR_1 and N;

L and A are as provided for Formula (I); and

two of X_1, X_{2a}, X_{2b}, and X_3 are N.

56. The compound of ~~any one of claims 1 to~~claim 4 ~~5~~, having the structure of formula (III):



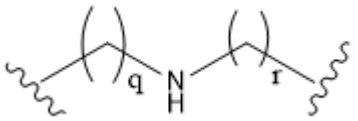
Formula (III)

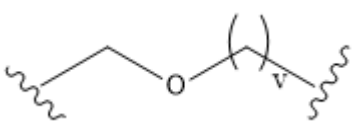
wherein

L and A are as provided for Formula (I).

~~7. The compound of any one of claims 1 to 6, wherein p, q, r, and v are independently selected from integers 0, 1, 2, and 3.~~

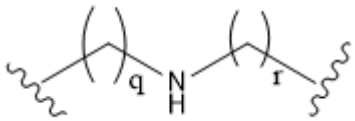
68. The compound of claim 5~~any one of claims 1 to 7~~, wherein L is selected from



~~,~~and 

, wherein q, r and v are independently selected from integers 0, 1, 2, and 3.~~.~~

79. The compound of claim 6~~any one of claims 1 to 8~~, wherein L is



, wherein q and r are 0 or 1.

~~10. The compound of any one of claims 1 to 9, wherein q and r are 0 or 1.~~

~~11. The compound of any one of claims 1 to 10, wherein q and r are 1.~~

~~12. The compound of any one of claims 1 to 10, wherein q is 1 and r is 0.~~

8~~13~~. The compound of ~~any one of claims 1 to 12~~claim 7, wherein m is 1 and~~.~~

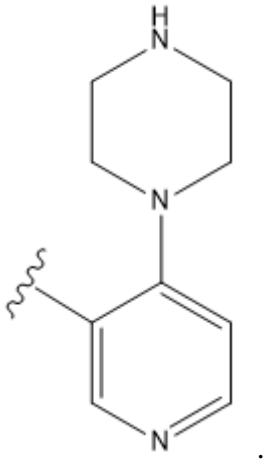
~~14. The compound of any one of claims 1 to 13, wherein~~ W is selected from -NH, -O, and -N(C_1-C_6 alkyl)$_2$.

~~15. The compound of any one of claims 1 to 14, wherein W is –NH.~~

9~~16~~. The compound of claim 8~~any one of claims 1 to 15~~, wherein at least one of X_4, X_5, X_6, and X_7 is N.

~~17. The compound of any one of claims 1 to 16, wherein X_5 or X_6 are N, and both X_4 and X_7 are independently CR_2.~~

10~~8~~. The compound of claim 9~~any one of claims 1 to 17~~, wherein A is



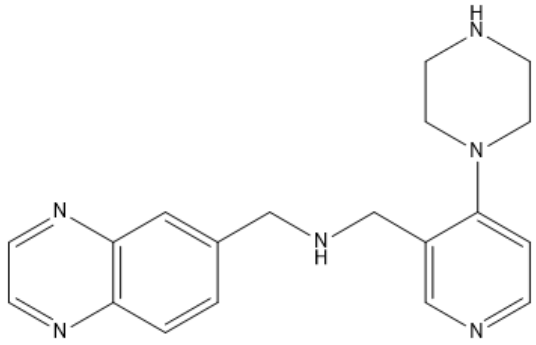
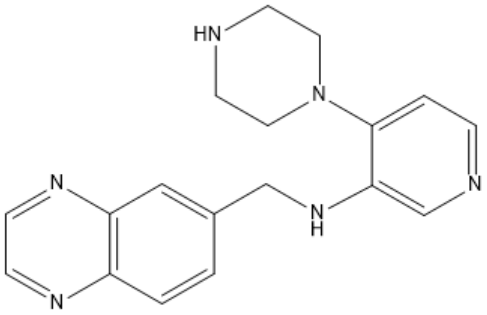
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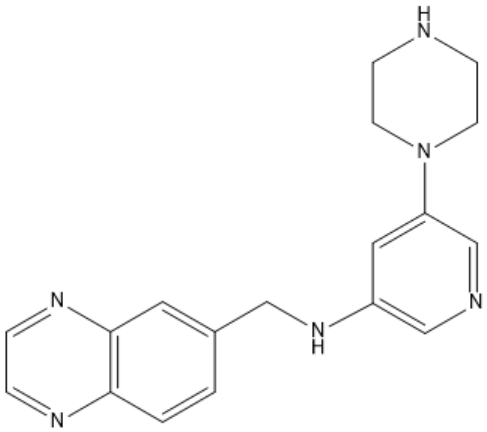
~~19. The compound of any one of claims 1 to 18, wherein Y_1, Y_2, and Y_3 are, in each instance, independently selected from CR_2 and N, wherein R_1 is selected from -H, -Cl, -Br, -I, -F, -OH, -and -NH_2.~~

~~20. The compound of any one of claims 1 to 19, wherein Y_2 is N.~~

1~~2~~1. The compound of claim 10~~any one of claims 1 to 19~~, wherein Y_2 is CR_2 and R_1 is selected from -H, -F, -OH, -and -NH_2.

1~~2~~2. The compound of claim 11, wherein said compound has the structure:



, , or


, or a pharmaceutically acceptable salt thereof.

1~~2~~3. The compound of ~~any one of claims 1 to 22~~claim 1, wherein the compound binds to a region of an RNA molecule.

1~~2~~4. The compound of claim 13~~23~~, wherein the RNA molecule is a non-coding RNA molecule~~.~~

~~25. The compound of claim 24, wherein the non-encoding RNA molecule is~~ selected from rRNA, microRNAs, siRNAs, piRNAs, snoRNAs, snRNAs, exRNAs, and scaRNAs.

15~~26~~. The compound of claim 13~~23~~, wherein the RNA molecule is a coding RNA molecule selected from~~.~~

~~27. The compound of claim 26, wherein the coding RNA molecule is~~ mRNA.

16~~28~~. The compound of claim 1~~2~~5~~3~~, wherein the region of the mRNA is a ~~riboswitch.~~

~~29. The compound of claim 28, wherein the riboswitch is a~~ TPP riboswitch.

~~30. The compound of any one of claims 23 to 29 wherein the compound exhibits a K_d binding affinity of from about 50 nM to about 100 μM.~~

17~~31~~. A composition comprising a therapeutically effective amount of the compound of ~~any one of~~ claim~~s~~ 1~~ to 30~~ in a pharmaceutically acceptable carrier, diluent, or excipient.

18~~32~~. A compound of claim 1 for use in the treatment of ~~A method of treating~~ a disease or disorder associated with a dysfunction in RNA expression, wherein the ~~method comprising~~compound is to be administer~~ed~~~~ing~~ to a subject in need thereof at a dose of a therapeutically effective amount of ~~a~~ the compound ~~of any one of claims 1-30 or a composition of claim 31~~claim 1.

19~~33~~. The ~~method~~ compound for use of claim 18~~32~~, wherein ~~administering~~ the compound ~~or composition~~is to be administered to lower~~s~~ protein expression due to binding of the compound to RNA ~~(e.g., mRNA)~~.

20~~34~~. The ~~method~~ compound for use of claim 18~~32 or 33~~, wherein said disease or disorder is selected from genetic diseases, degenerative disorders, cancer, diabetes, autoimmune disorders, cardiovascular disorders, clotting disorders, diseases of the eye, infectious disease, and diseases caused by mutations in one or more gene.

21~~35~~. The ~~method~~ compound for use of claim 18~~34~~, wherein the disease or disorder is a genetic disease and ~~administering~~ the compound ~~or the composition~~ is to be administered to switch~~es~~ expression of a therapeutic gene on or off.

~~36. A method of making a compound of any one of claims 1 to 30, the method comprising:~~

~~a) contacting a fragment of formula IV with a fragment of formulae V-1 or V-2 in the presence of a Pd-catalyst; or~~

~~b) contacting a fragment of formula IV with a fragment of formulae VI-1 or VI-2 in the presence of a reducing agent; or~~

~~c) contacting a fragment of formula IV with a fragment of formulae VII-1 or VII-2 in the presence of base.~~