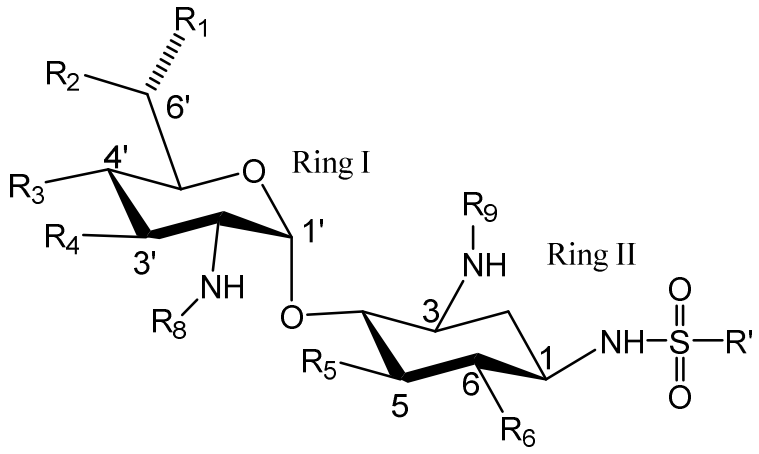
WHAT IS CLAIMED IS:

1. A compound represented by general formula Ia:



Formula Ia

or a pharmaceutically acceptable salt thereof,

wherein:

the dashed line indicates a stereo-configuration of position 6' being an *R* configuration or an *S* configuration;

R_1 is selected from hydrogen, a substituted or unsubstituted alkyl, a substituted or unsubstituted cycloalkyl, and a substituted or unsubstituted aryl;

R_2 is selected from hydrogen, a substituted or unsubstituted alkyl and ORx, wherein Rx is selected from hydrogen, a substituted or unsubstituted alkyl, a substituted or unsubstituted cycloalkyl, a substituted or unsubstituted aryl, a substituted or unsubstituted heteroaryl, a substituted or unsubstituted alkaryl, and an acyl, or, alternatively, R_2 is said ORx and forms together with R_3 a dioxane;

R_3 is selected from hydrogen, a substituted or unsubstituted alkyl and ORy, wherein Ry is selected from hydrogen, a substituted or unsubstituted alkyl, a substituted or unsubstituted cycloalkyl, a substituted or unsubstituted aryl, a substituted or unsubstituted heteroaryl, a substituted or unsubstituted alkaryl, and an acyl, or, alternatively, R_3 is said ORy and formed together with R_2 a dioxane;

R_4-R_6 are each independently selected from hydrogen, a substituted or unsubstituted alkyl, and ORz, wherein Rz is selected from hydrogen, a monosaccharide moiety, an oligosaccharide

moiety, a substituted or unsubstituted alkyl, a substituted or unsubstituted cycloalkyl, a substituted or unsubstituted aryl, a substituted or unsubstituted heteroaryl, a substituted or unsubstituted alkaryl, and an acyl; and

R_8-R_9 are each independently selected from hydrogen, acyl, an amino-substituted alpha-hydroxy acyl, a substituted or unsubstituted alkyl, a substituted or unsubstituted cycloalkyl, a substituted or unsubstituted aryl, a substituted or unsubstituted alkaryl and a sulfonyl

and wherein R' is selected from a substituted or unsubstituted alkyl, a substituted or unsubstituted cycloalkyl, a substituted or unsubstituted alkaryl, and a substituted or unsubstituted aryl.

2. The compound of claim 1, wherein R' is methyl.

3. The compound of any one of claims 1 or 2, wherein R_8 and R_9 are each hydrogen.

4. The compound of any one of claims 1 to 3, wherein R_2 is ORx, and Rx is selected from hydrogen and a substituted or unsubstituted alkyl.

5. The compound of any one of claims 1 to 4, wherein R_3 is ORy, and Ry is selected from hydrogen and a substituted or unsubstituted alkyl.

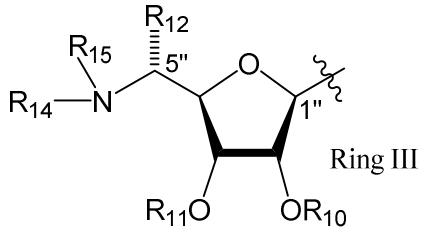
6. The compound of any one of claims 1 to 5, wherein each of R_4-R_6 is ORz.

7. The compound of any one of claims 1 to 5, wherein each of R_4-R_6 is ORz, and in each of said R_4-R_6 Rz is hydrogen.

8. The compound of any one of claims 1 to 7, wherein at least one of R_4-R_6 is ORz and Rz is said monosaccharide moiety or said oligosaccharide moiety.

9. The compound of any one of claims 1 to 7, wherein R_5 is ORz and Rz is said monosaccharide moiety.

10. The compound of any one of claims 1 to 5, 8 and 9, wherein said monosaccharide moiety is represented by Formula II:



Formula II

wherein the curved line denotes a position of attachment;

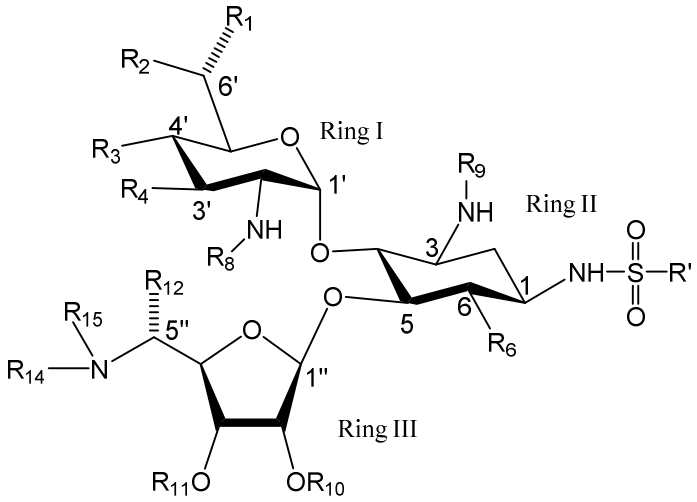
the dashed line indicates a stereo-configuration of position 5'' being an *R* configuration or an *S* configuration;

R_{10} and R_{11} are each independently selected from hydrogen, a substituted or unsubstituted alkyl, a substituted or unsubstituted cycloalkyl, a substituted or unsubstituted aryl, a substituted or unsubstituted alkaryl, and an acyl;

R_{12} is selected from the group consisting of hydrogen, a substituted or unsubstituted alkyl, a substituted or unsubstituted cycloalkyl, and a substituted or unsubstituted aryl; and

each of R_{14} and R_{15} is independently selected from hydrogen, acyl, an amino-substituted alpha-hydroxy acyl, a substituted or unsubstituted alkyl, a substituted or unsubstituted cycloalkyl, a substituted or unsubstituted aryl, a substituted or unsubstituted alkaryl, a sulfonyl and a cell-permealizable group, or, alternatively, R_{14} and R_{15} form together a heterocyclic ring.

11. The compound of any one of claims 1 to 5, the compound being represented by Formula IIIa:



Formula IIIa

wherein:

R_1-R_4, R_6, R_8 and R_9 are as defined for Formula Ia; and

R_{10}, R_{11}, R_{12}, R_{14} and R_{15} are as defined for Formula II in claim 10.

12. The compound of claim 11, wherein R_2 is ORx, and Rx is selected from hydrogen and a substituted or unsubstituted alkyl.

13. The compound of claim 11 or 12, wherein R_3 is ORy, and Ry is selected from hydrogen and a substituted or unsubstituted alkyl.

14. The compound of any one of claims 11 to 13, wherein R_4 and R_6 are each independently ORz.

15. The compound of any one of claims 11 to 13, wherein R_4 and R_6 are each ORz and Rz is hydrogen.

16. The compound of any one of claims 11 to 15, wherein R_8 and R_9 are each hydrogen.

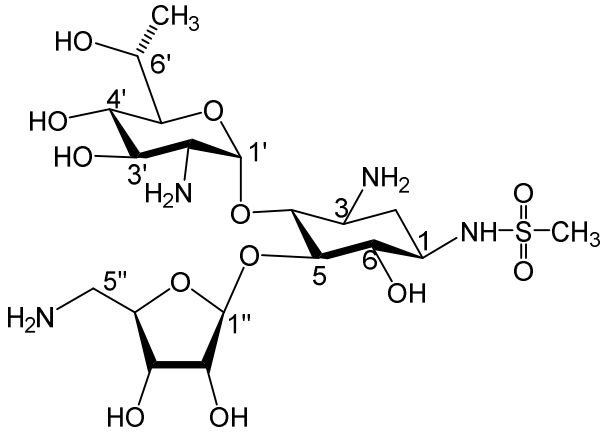
17. The compound of any one of claims 11 to 16, wherein R_{10}, R_{11}, R_{12}, R_{14} and R_{15} are each hydrogen.

18. The compound of any one of claims 11 to 17, wherein R_{10}, R_{11}, R_{14} and R_{15} are each hydrogen and R_{12} is selected from the group consisting of a substituted or unsubstituted alkyl, a substituted or unsubstituted cycloalkyl, and a substituted or unsubstituted aryl.

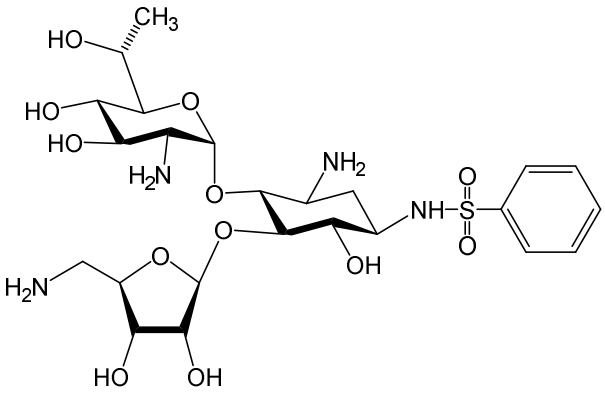
19. The compound of claim 18, wherein R_{12} is a substituted or unsubstituted alkyl.

20. The compound of claim 19, wherein R_{12} is methyl.

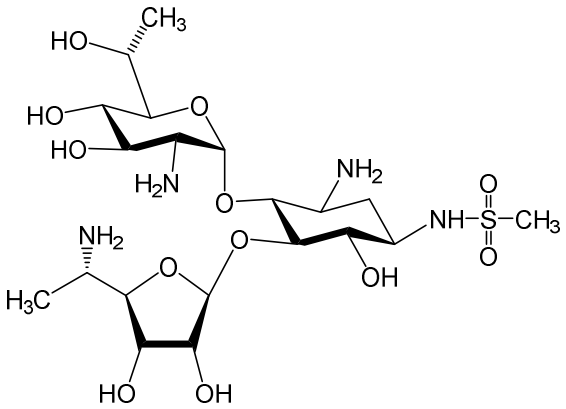
21. The compound of any one of claims 11 to 16, being selected from:



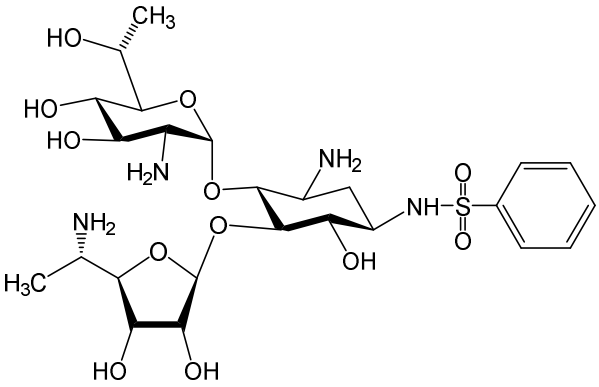
NB74-MeS;



NB74-PhS;



NB124-MeS; and



NB124-PhS.

22. The compound of any one of claims 1 to 20, wherein R_1 is a substituted or unsubstituted alkyl.

23. A pharmaceutical composition comprising the compound of any one of claims 1 to 22 and a pharmaceutically acceptable carrier.

24. The compound of any one of claims 1 to 22 or the composition of claim 23, for use in the treatment of a genetic disorder.

25. The compound or composition of claim 25, wherein the genetic disorder is associated with a premature stop codon mutation and/or a protein truncation phenotype.

26. The compound of any one of claims 1 to 22 or the composition of claim 23, for use in increasing an expression level of a gene having a stop codon mutation.