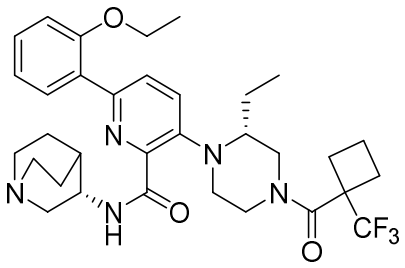
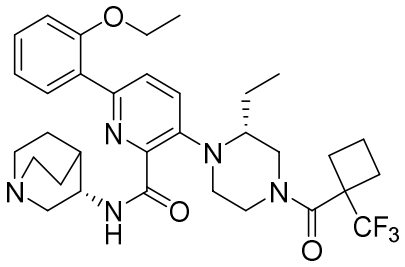
CLAIMS

1. Use of a compound of Formula (I), or a pharmaceutically acceptable salt thereof in the manufacture of a medicament for treating congenital adrenal hyperplasia (CAH) in a subject:



Formula (I).

2. Use of a compound of Formula (I), or a pharmaceutically acceptable salt thereof in the manufacture of a medicament for treating hyperandrogenism in a subject with CAH:



Formula (I).

3. The use of any one of claims 1-2, wherein the compound of Formula (I), or the pharmaceutically acceptable salt thereof, is to be administered in a therapeutically effective amount.

4. The use of any one of claims 1-3, wherein the compound of Formula (I), or the pharmaceutically acceptable salt thereof, is to be administered in an amount equivalent to about 40 mg to about 160 mg of Formula (I).

5. The use of any one of claims 1-3, wherein the compound of Formula (I), or the pharmaceutically acceptable salt thereof, is to be administered in an amount equivalent to about 80 mg to about 160 mg of Formula (I).

6. The use of any one of claims 1-3, wherein the compound of Formula (I), or the pharmaceutically acceptable salt thereof, is to be administered in an amount equivalent to about 80 mg to about 120 mg of Formula (I).

7. The use of any one of claims 1-3, wherein the compound of Formula (I), or the pharmaceutically acceptable salt thereof, is to be administered in an amount equivalent to about 40 mg of Formula (I).

8. The use of any one of claims 1-3, wherein the compound of Formula (I), or the pharmaceutically acceptable salt thereof, is to be administered in an amount equivalent to about 80 mg of Formula (I).

9. The use of any one of claims 1-3, wherein the compound of Formula (I), or the pharmaceutically acceptable salt thereof, is to be administered in an amount equivalent to about 120 mg of Formula (I).

10. The use of any one of claims 1-3, wherein the compound of Formula (I), or the pharmaceutically acceptable salt thereof, is to be administered in an amount equivalent to about 160 mg of Formula (I).

11. The use of any one of claims 1-10, wherein the compound of Formula (I), or the pharmaceutically acceptable salt thereof, is to be administered in one or more unit dosage forms.

12. The use of claim 11, wherein the unit dosage form comprises the compound of Formula (I), or the pharmaceutically acceptable salt thereof, in an amount of equivalent to about 40 mg of Formula (I).

13. The use of any one of claims 11-12, wherein the unit dosage form is an oral unit dosage form.

14. The use of any one of claims 1-13, wherein the compound of Formula (I), or the pharmaceutically acceptable salt thereof, is to be administered daily.

15. The use of any one of claims 1-13, wherein the compound of Formula (I), or the pharmaceutically acceptable salt thereof, is to be administered once daily.

16. The use of any one of claims 1-15, wherein the CAH is caused by 21-hydroxylase deficiency.

17. The use of any one of claims 1-16 wherein the subject is concurrently on glucocorticoid replacement therapy.

18. The use of any one of claims 1-16, wherein the subject is concurrently on hydrocortisone, prednisolone, prednisone, or methylprednisolone therapy.

19. The use of any one of claims 1-16, wherein the subject is concurrently on mineralocorticoid replacement therapy.

20. The use of any one of claims 1-16, wherein the subject is concurrently on fludrocortisone therapy.

21. The use of any one of claims 1-20, wherein treating comprises reducing the subject's serum levels of androstenedione (A4) from baseline.

22. The use of claim 21, wherein the subject's serum levels of A4 are reduced by >75% from baseline.

23. The use of claim 21, wherein the subject's serum levels of A4 are reduced by >80% from baseline.

24. The use of claim 21, wherein the subject's serum levels of A4 are reduced by >85% from baseline.

25. The use of claim 21, wherein the subject's serum levels of A4 are reduced by >90% from baseline.

26. The use of any one of claims 1-20, wherein treating comprises reducing the subject's serum levels of 17-hydroxyprogesterone (17-OHP) from baseline.

27. The use of claim 26, wherein the subject's serum levels of 17-OHP are reduced by >75% from baseline.

28. The use of claim 26, wherein the subject's serum levels of 17-OHP are reduced by >80% from baseline.

29. The use of claim 26, wherein the subject's serum levels of 17-OHP are reduced by >85% from baseline.

30. The use of claim 26, wherein the subject's serum levels of 17-OHP are reduced by >90% from baseline.

31. The use of claim 26, wherein the subject's serum levels of 17-OHP are reduced by >95% from baseline.

32. The use of any one of claims 1-20, wherein treating comprises reducing the subject's serum levels of testosterone from baseline.

33. The use of claim 32, wherein the subject's serum levels of testosterone are reduced by ›50% from baseline.

34. The use of claim 32, wherein the subject's serum levels of testosterone are reduced by >60% from baseline.

35. The use of claim 32, wherein the subject's serum levels of testosterone are reduced by >70% from baseline.

36. The use of claim 32, wherein the subject's serum levels of testosterone are reduced by >80% from baseline.

37. The use of any one of claims 32-36, wherein the subject is a female.

38. The use of any one of claims 1-37, wherein treating comprises reducing the subject's serum levels of one or more of 11-deoxycorticosterone (11-DOC), 11β-

hydroxyandrostenedione (11-OHA4), 11-ketoandrostenedione (11-ketoA4), 11β-hydroxytestosterone (11-OHT), and 11-ketotestosterone (11-ketoT) from baseline.

39. The use of any one of claims 21-36, wherein the baseline is measured in the morning.

40. The use of any one of claims 1-39, wherein treating comprises reducing adrenal gland size.

41. The use of any one of claims 1-39, wherein treating comprises reducing waist circumference.

42. The use of any one of claims 1-39, wherein treating comprises reducing levels of hemoglobin A1C (HbA1C).

43. The use of any one of claims 1 and 3-39, wherein the subject has hyperandrogenism.

44. The use of any one of claims 1-39, wherein the subject has acne.

45. The use of claim 44, wherein the subject reports their acne has improved following administration of the compound of Formula (I), or a pharmaceutically acceptable salt thereof.

46. The use of any one of claims 1-39, wherein the subject has hirsutism.

47. The use of claim 46, wherein the subject reports their hirsutism has improved following administration of the compound of Formula (I), or a pharmaceutically acceptable salt thereof.

48. The use of any one of claims 1-39, wherein the subject has menstrual dysfunction.

49. The use of claim 48, wherein the subject reports their menstrual dysfunction has improved following administration of the compound of Formula (I), or a pharmaceutically acceptable salt thereof.

50. The use of any one of claims 1-39, wherein the subject has testicular adrenal rests tumor (TART).

51. The use of claim 50, wherein the subject’s TART has improved following administration of the compound of Formula (I), or a pharmaceutically acceptable salt thereof.