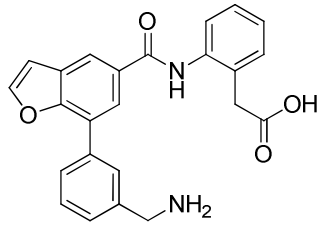
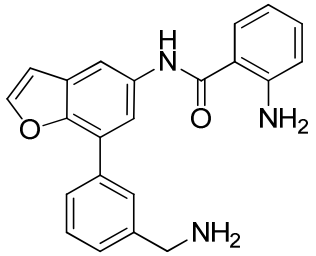
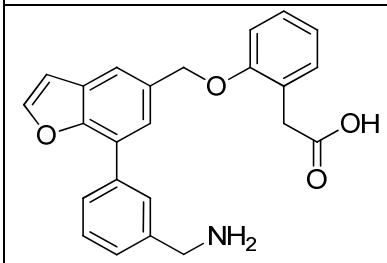
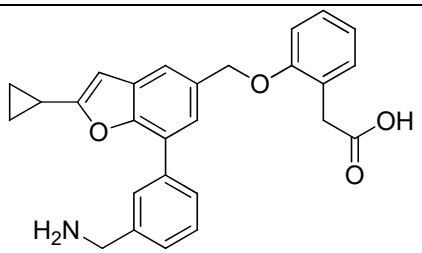
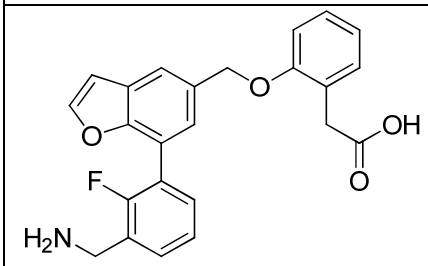
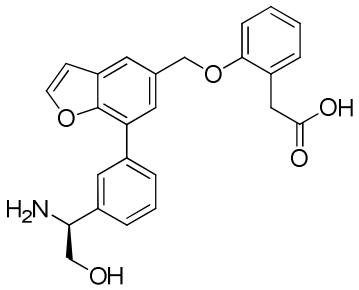
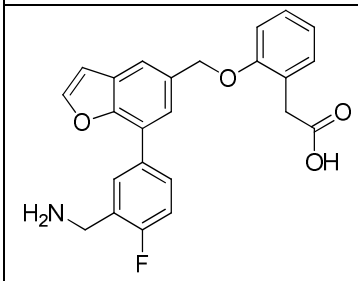
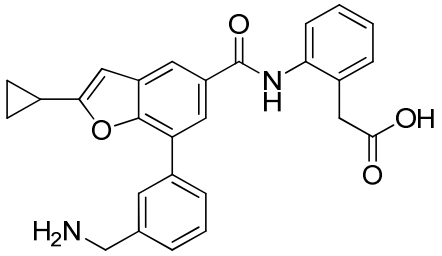
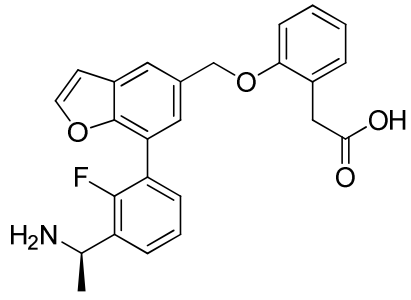
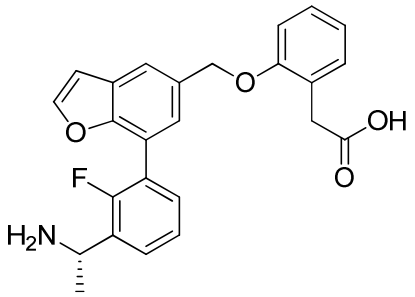
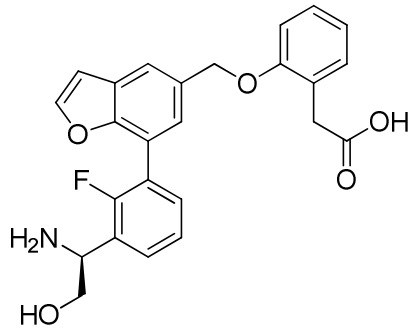
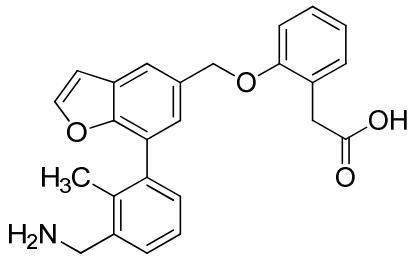
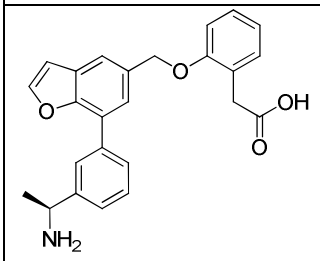
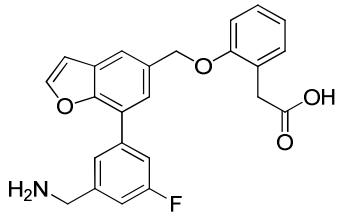
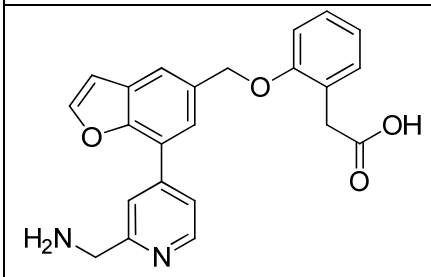
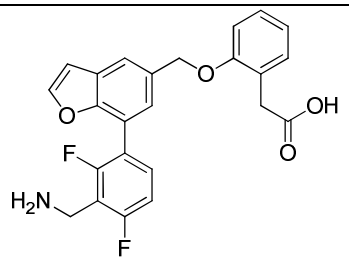
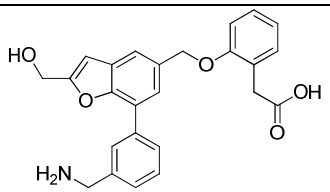
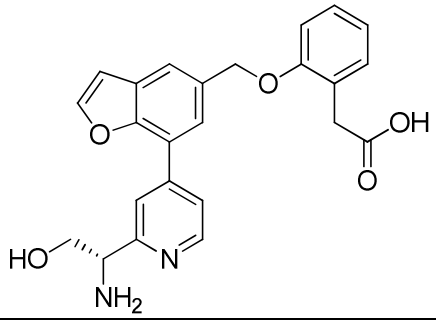
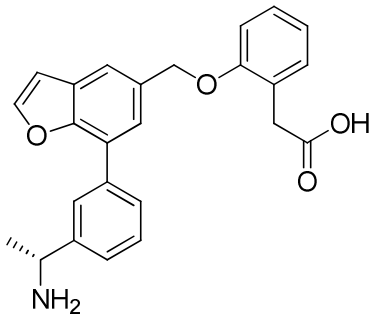
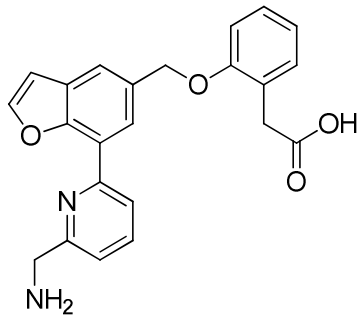
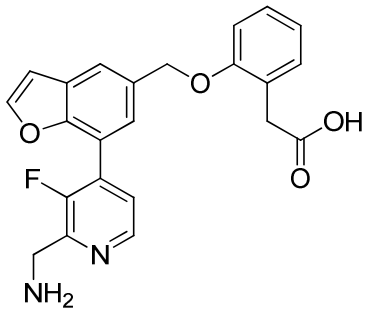
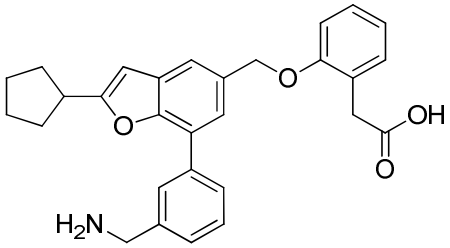
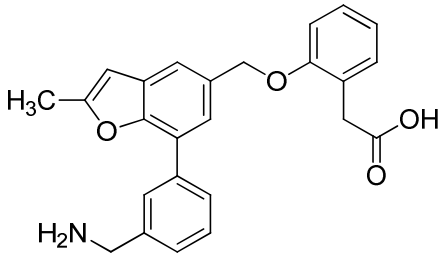
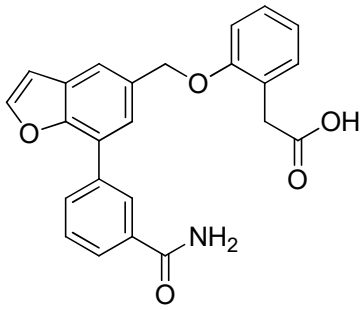
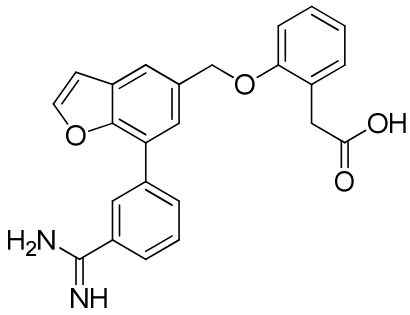
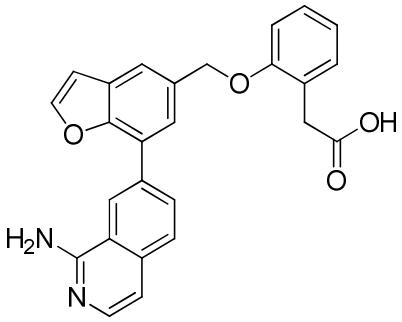
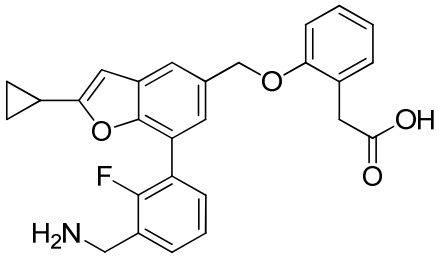
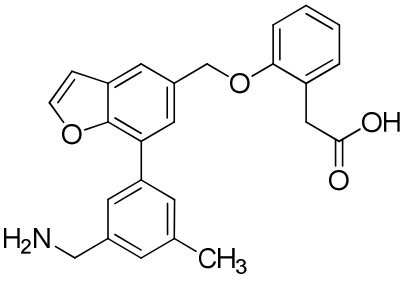
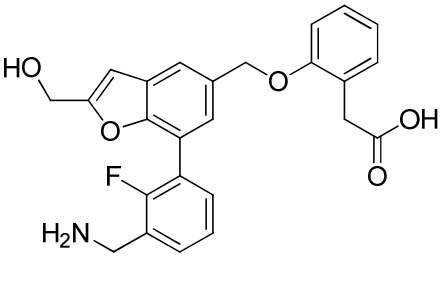
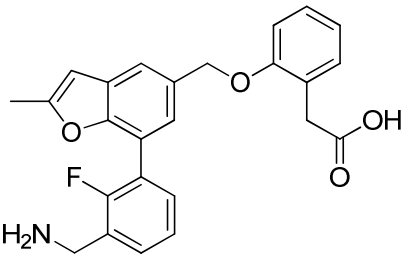
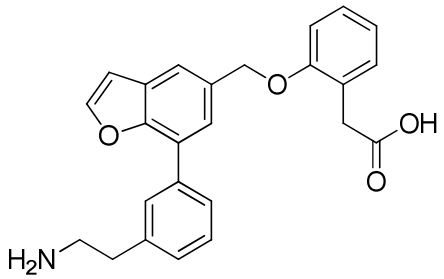
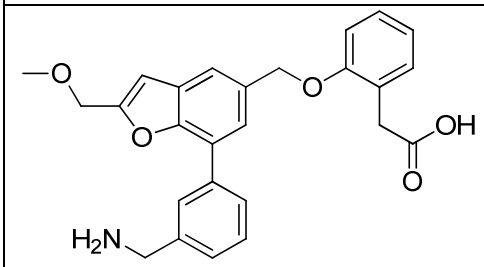
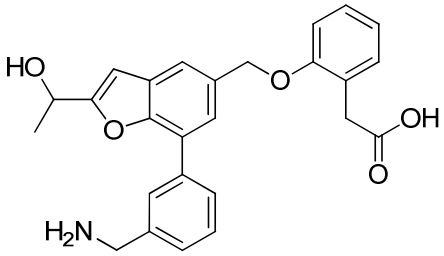
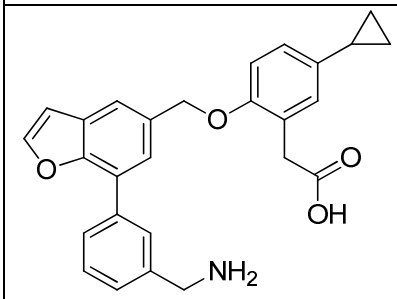
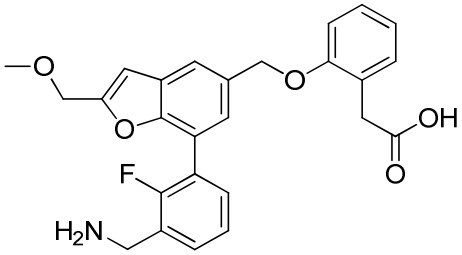
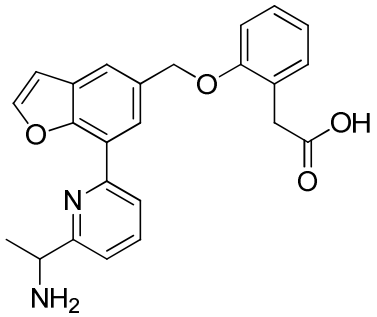
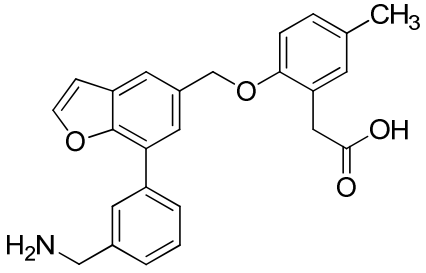
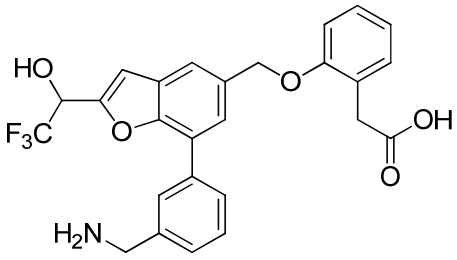
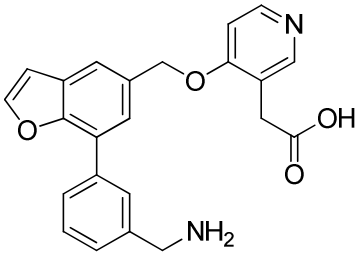
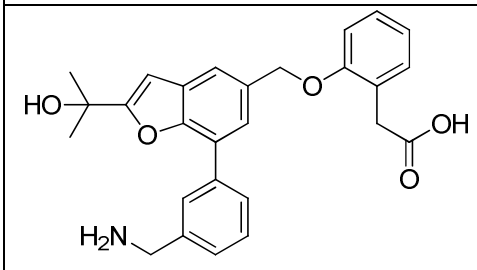
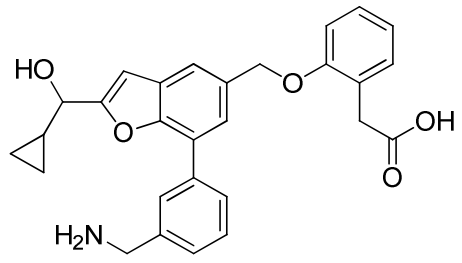
CLAIMS

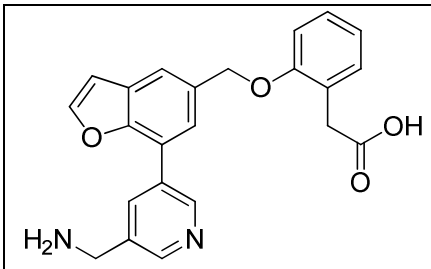
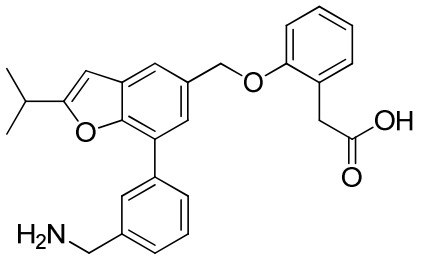
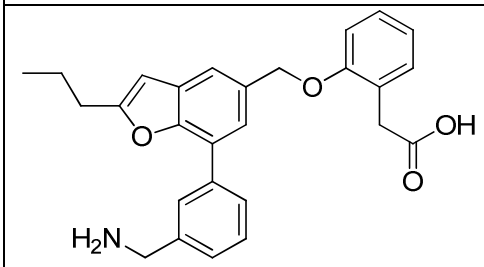
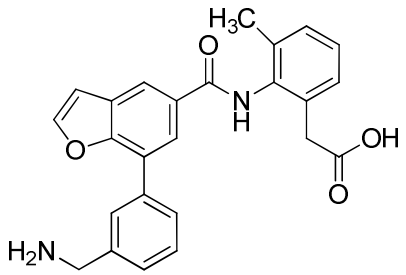
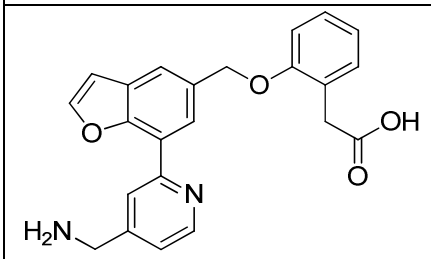
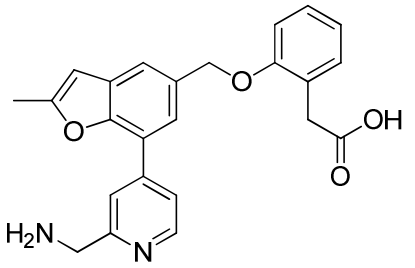
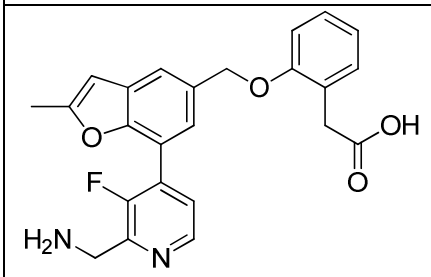
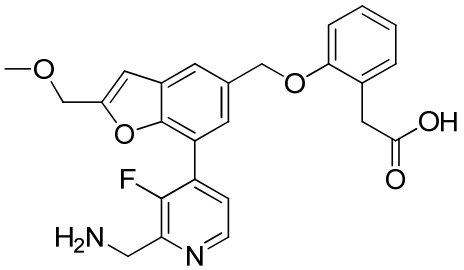
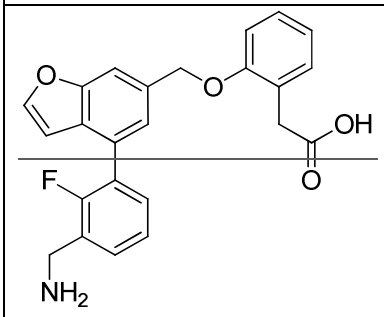
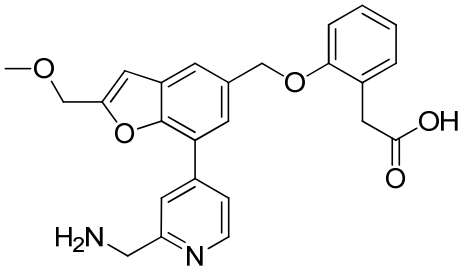
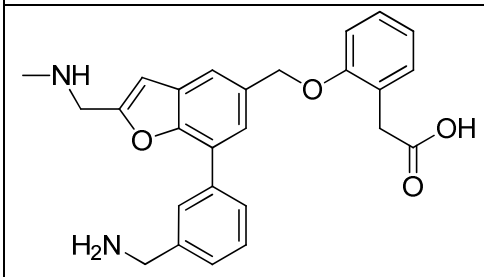
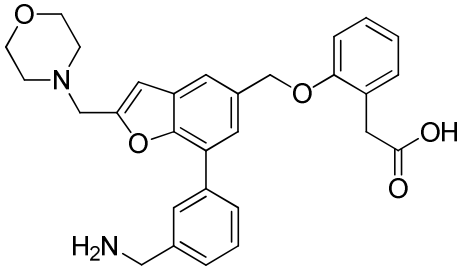
1. An oral dosage form comprising a compound selected from:

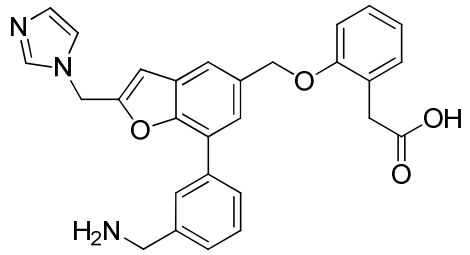
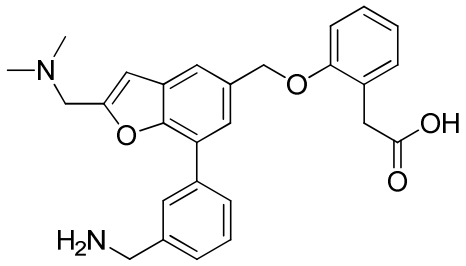
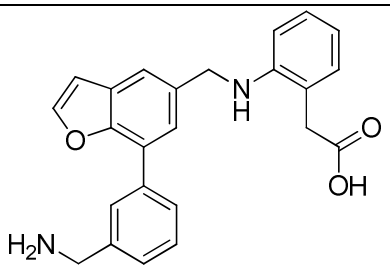
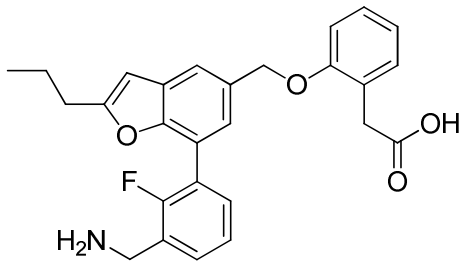
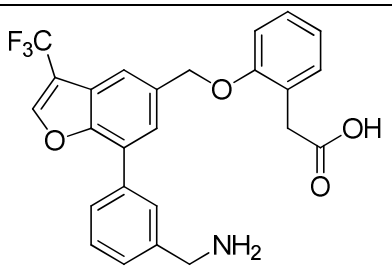
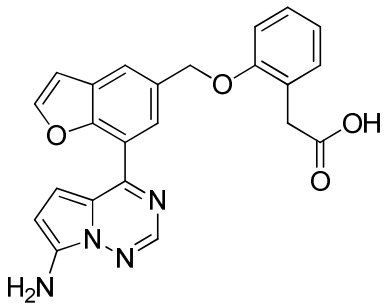
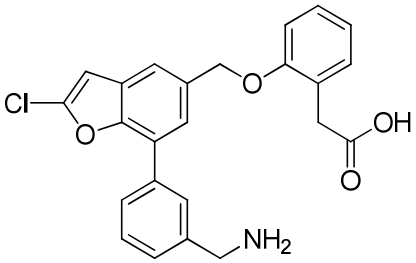
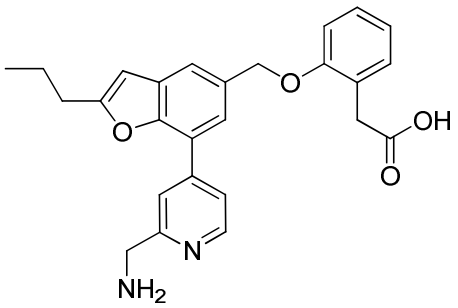
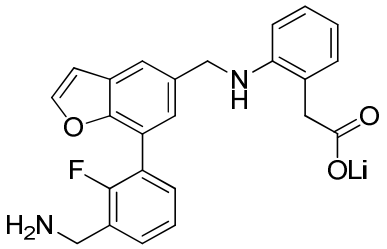
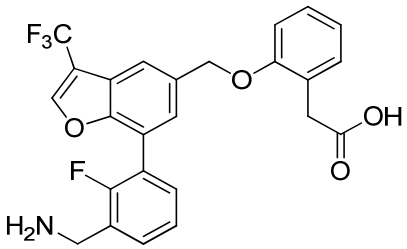
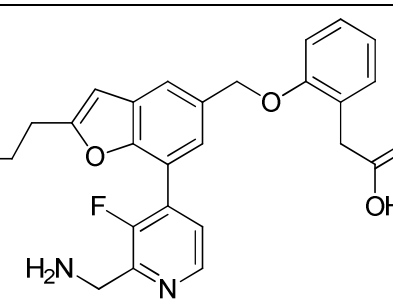
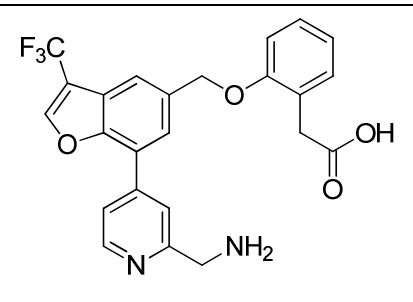
	
	
	
	
	

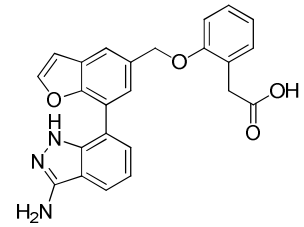
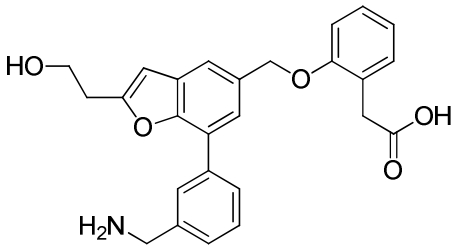
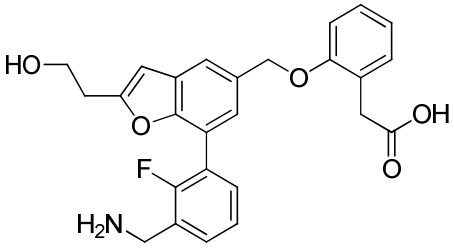
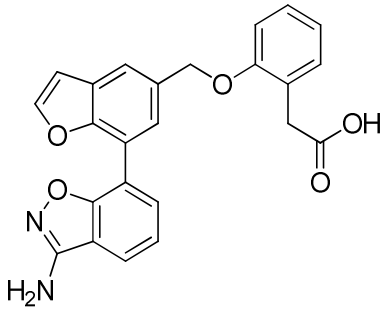
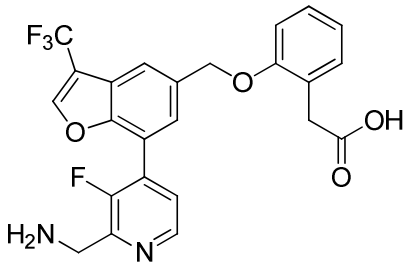
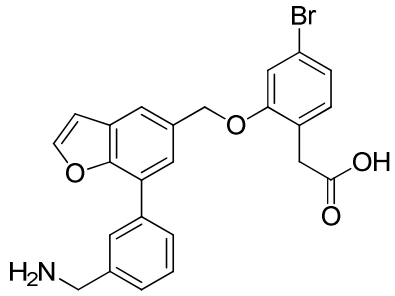
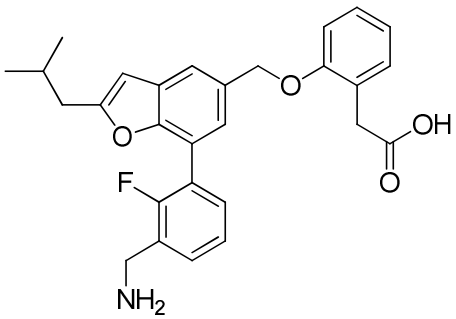
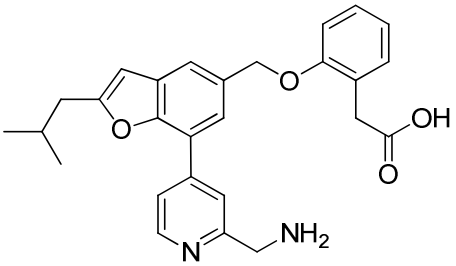
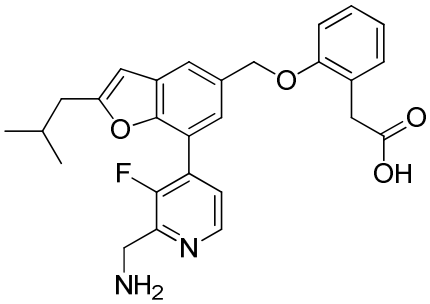
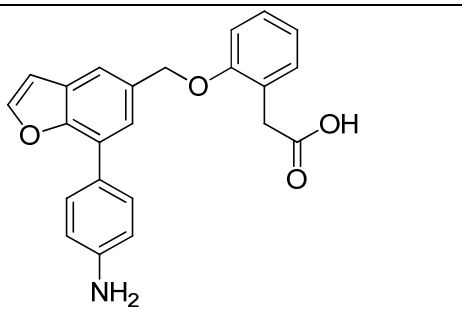
	
	
	
	
	

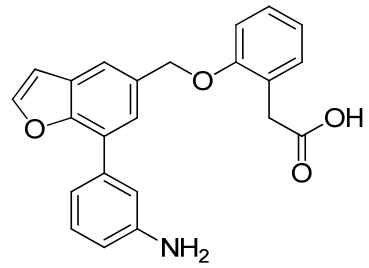
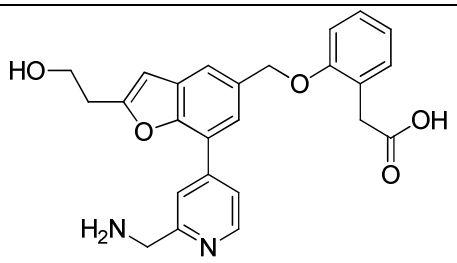
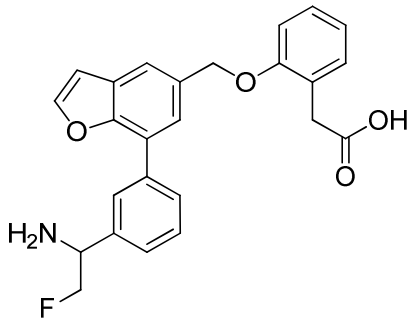
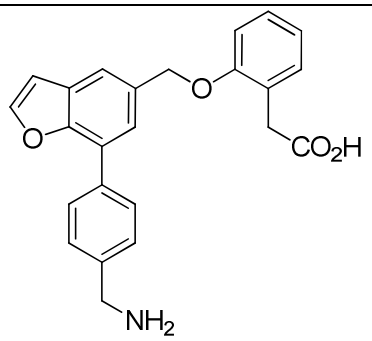
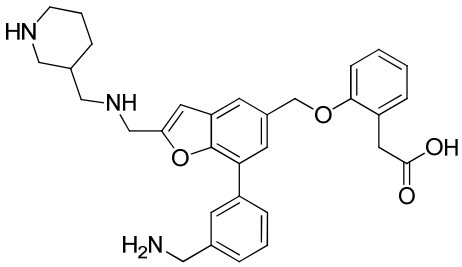
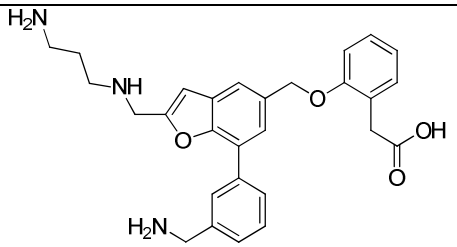
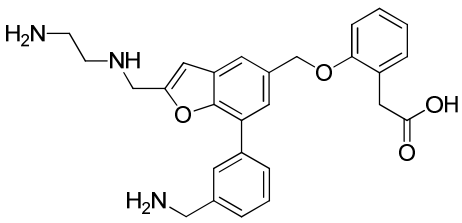
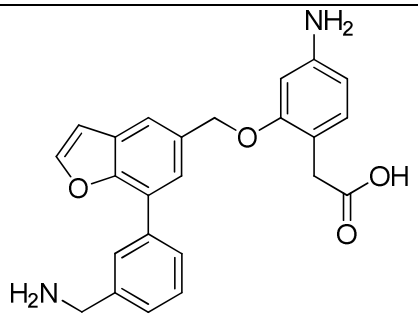
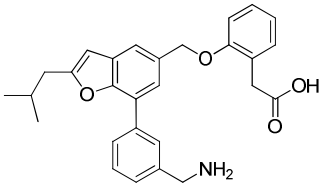
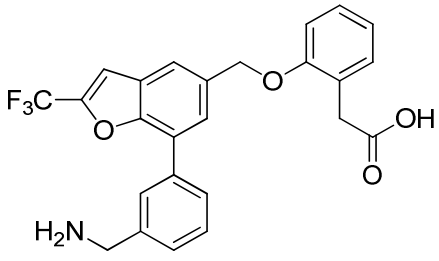
	
	
	
	
	

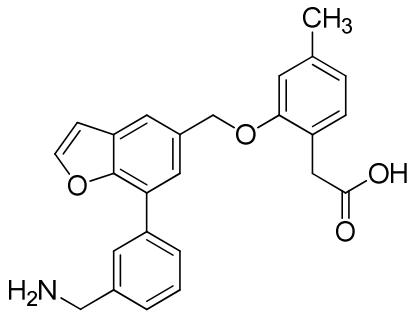
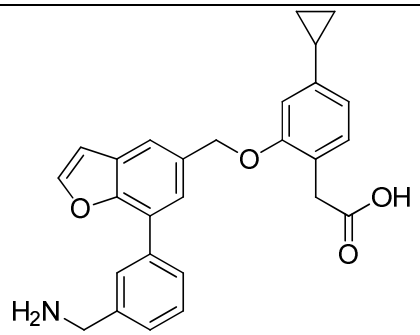
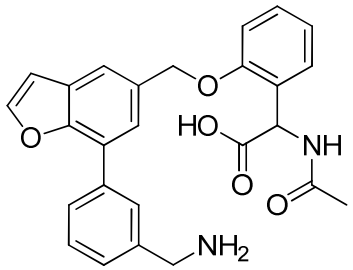
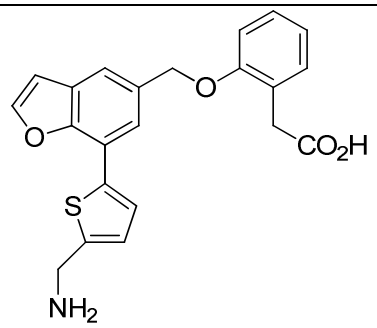
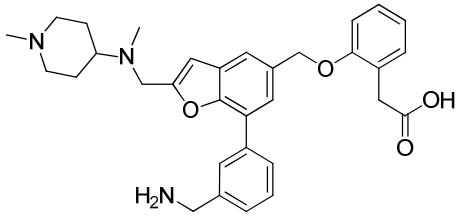
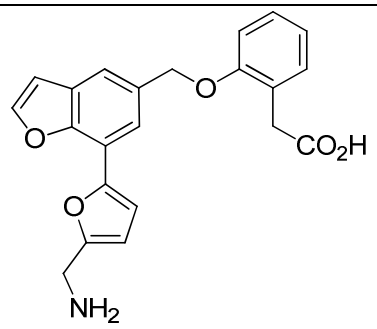
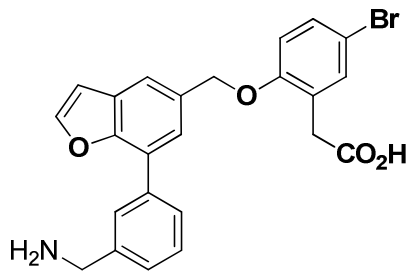
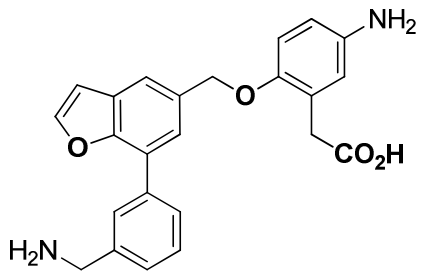
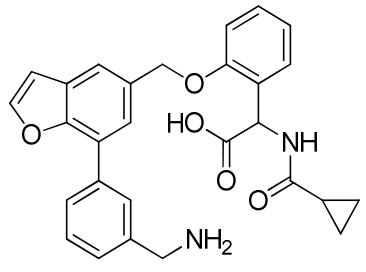
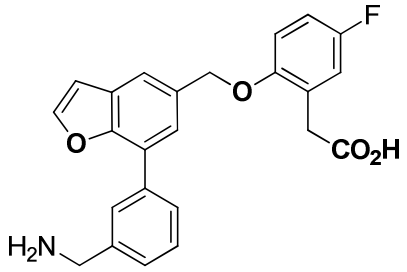
	
	
	
	
	
	

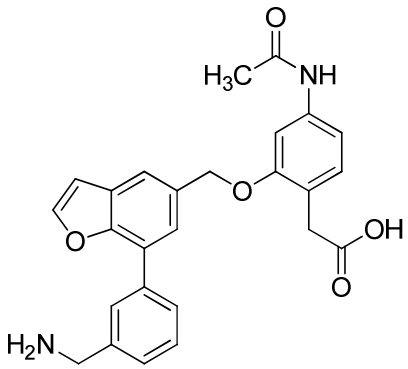
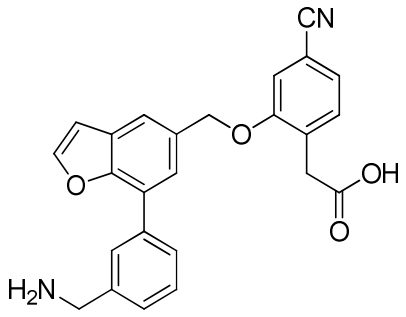
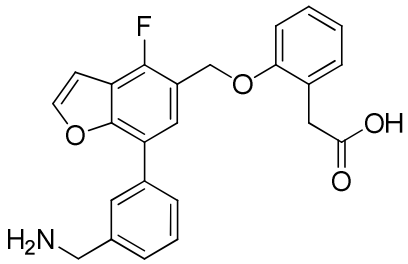
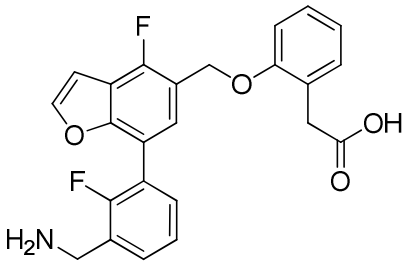
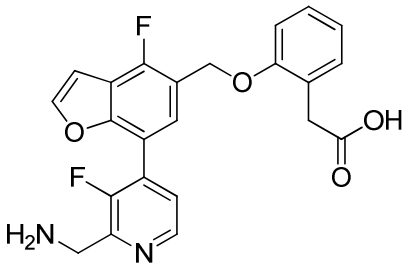
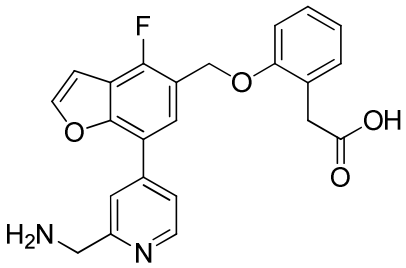
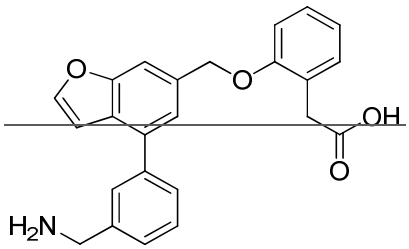
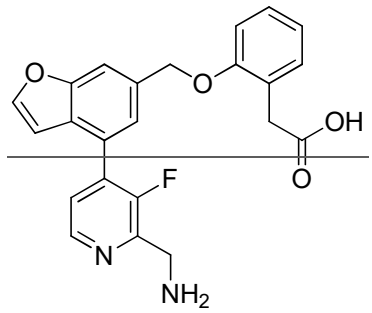
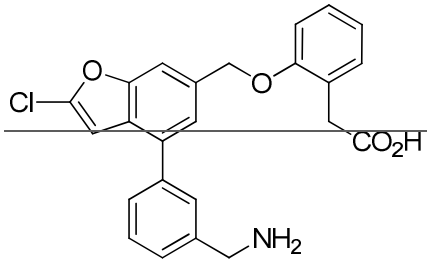
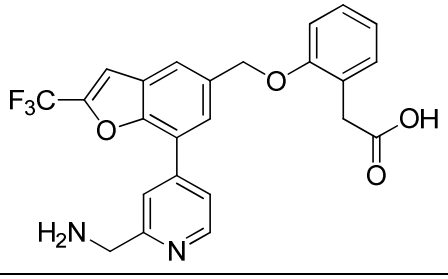
	
	
	
	
	
	

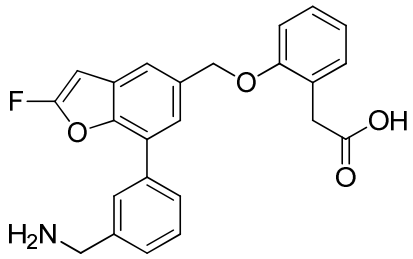
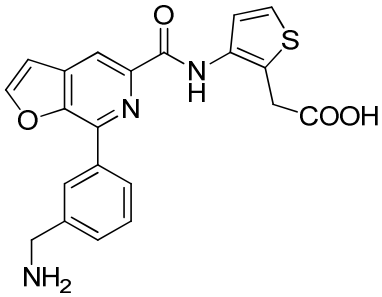
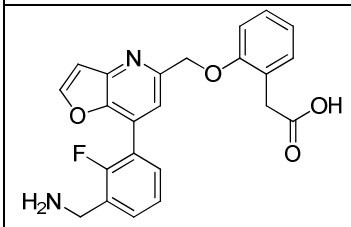
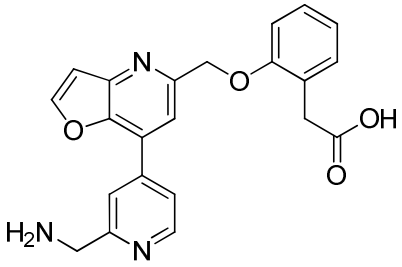
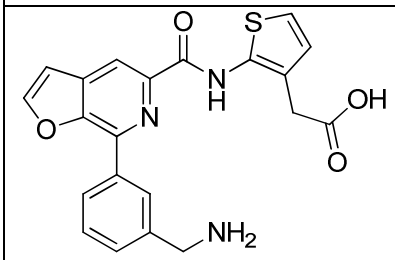
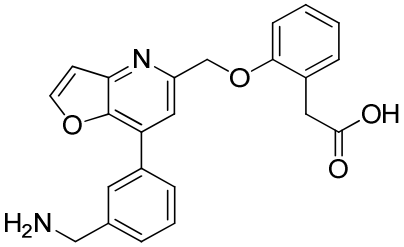
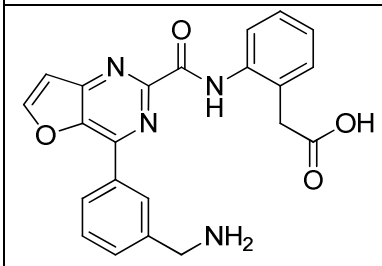
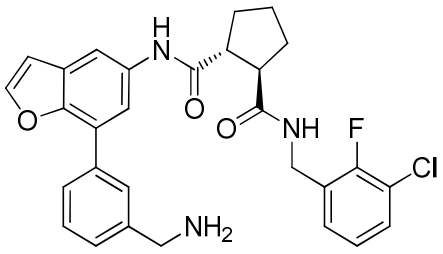
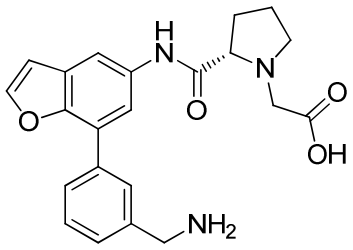
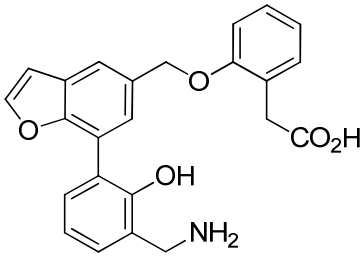
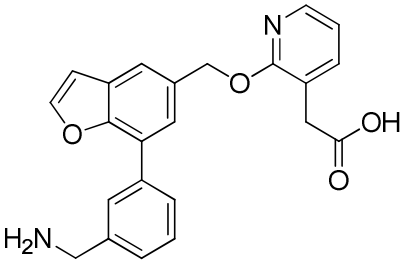
	
	
	
	
	
	

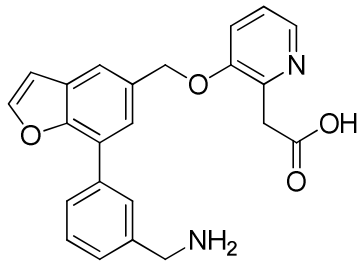
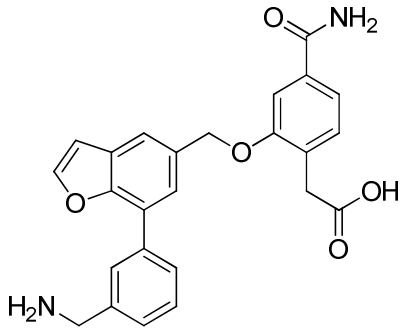
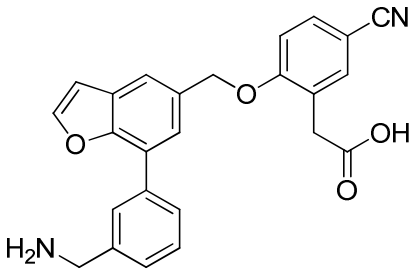
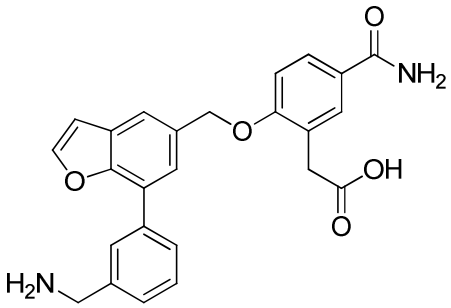
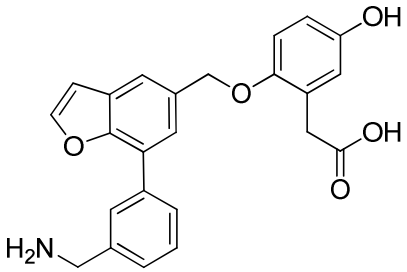
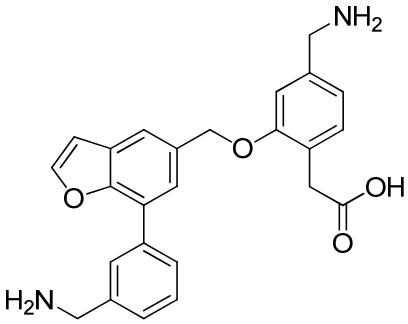
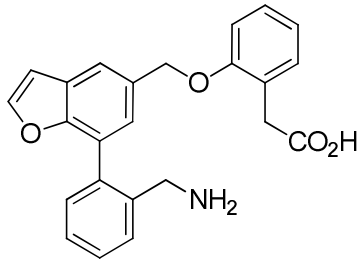
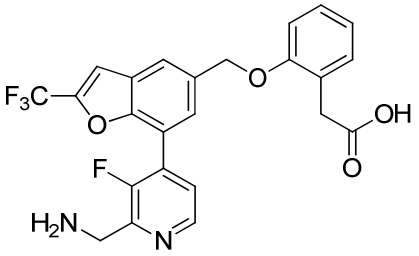
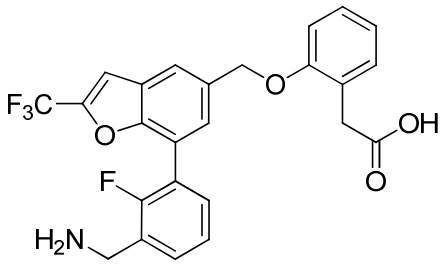
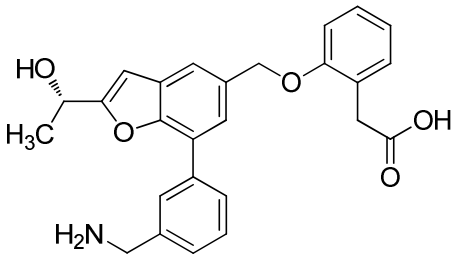
	
	
	
	
	

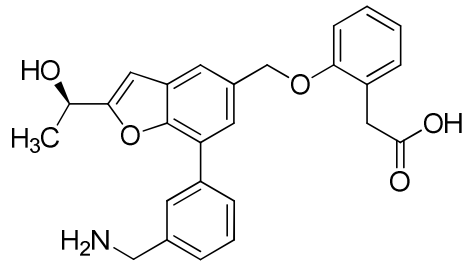
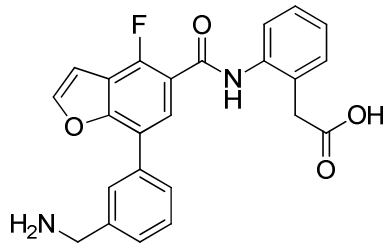
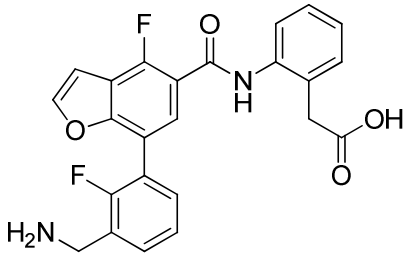
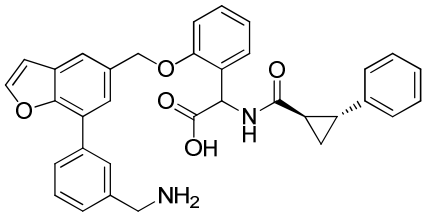
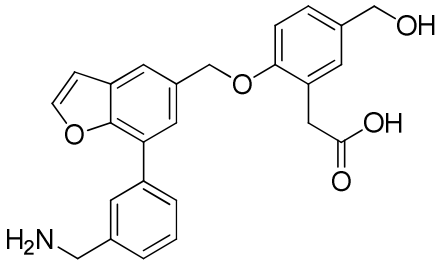
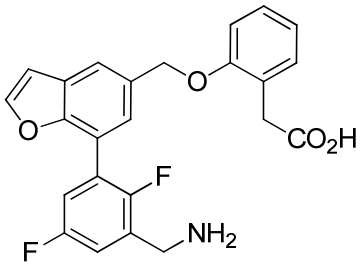
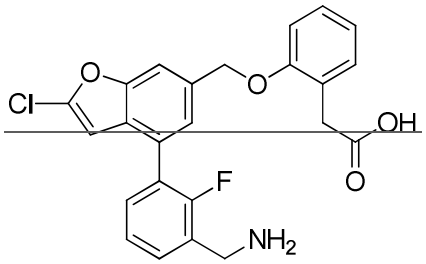
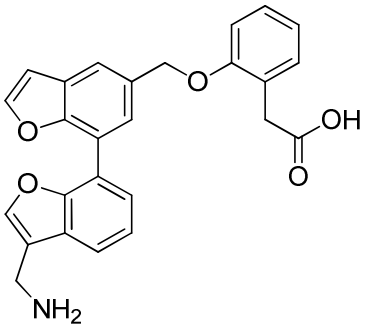
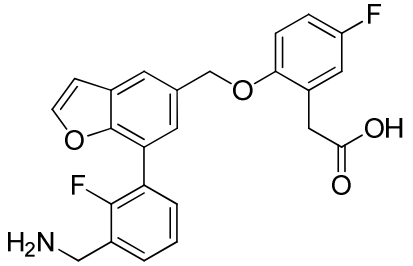
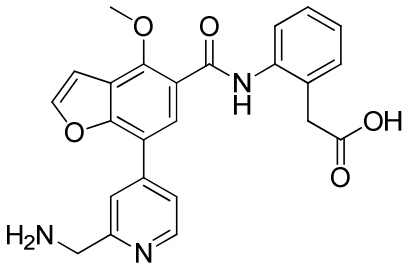
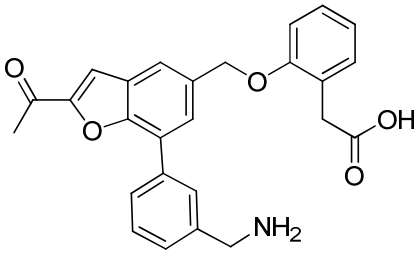
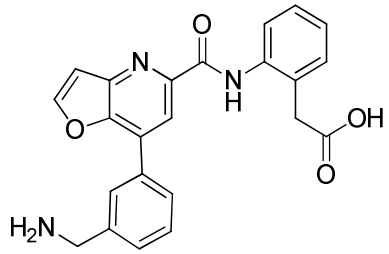
	
	
	
	
	

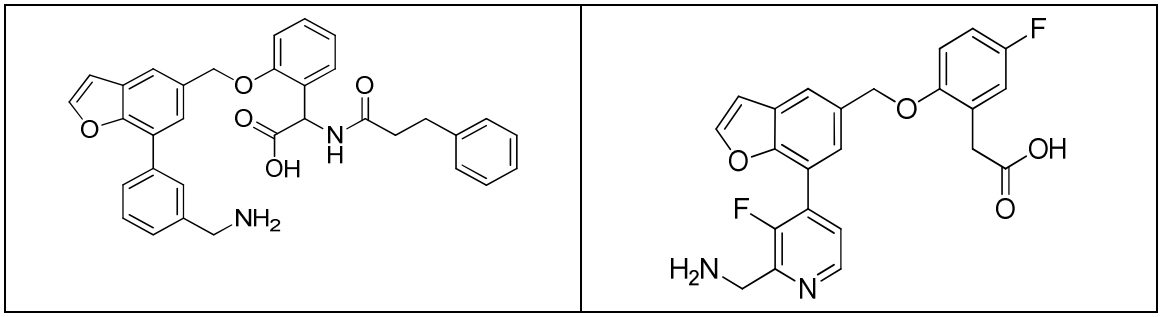
	
	
	
	
	



, or a pharmaceutically acceptable salt ~~or prodrug~~ thereof; and
a pharmaceutically acceptable carrier.

2. The oral dosage form of claim 1, comprising ~~about~~ 1 mg to ~~about~~ 1500 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

3. The oral dosage form of claim 1, comprising ~~about~~ 1 mg to ~~about~~ 1200 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

4. The oral dosage form of claim 1, comprising ~~about~~ 1 mg to ~~about~~ 1000 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

5. The oral dosage form of claim 1, comprising ~~about~~ 1 mg to ~~about~~ 800 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

6. The oral dosage form of claim 1, comprising ~~about~~ 1 mg to ~~about~~ 600 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

7. The oral dosage form of claim 1, comprising ~~about~~ 1 mg to ~~about~~ 400 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

8. The oral dosage form of claim 1, comprising ~~about~~ 1 mg to ~~about~~ 300 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

9. The oral dosage form of claim 1, comprising ~~about~~ 1 mg to ~~about~~ 200 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

10. The oral dosage form of claim 1, comprising ~~about~~ 1 mg to ~~about~~ 100 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

11. The oral dosage form of claim 1, comprising ~~about~~ 10 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

12. The oral dosage form of claim 1, comprising ~~about~~ 30 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

13. The oral dosage form of claim 1, comprising ~~about~~ 100 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

14. The oral dosage form of claim 1, comprising ~~about~~ 200 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

15. The oral dosage form of claim 1, comprising ~~about~~ 300 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

16. The oral dosage form of claim 1, comprising ~~about~~ 400 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

17. The oral dosage form of claim 1, comprising ~~about~~ 600 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

18. The oral dosage form of claim 1, comprising ~~about~~ 800 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

19. The oral dosage form of claim 1, comprising ~~about~~ 1000 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

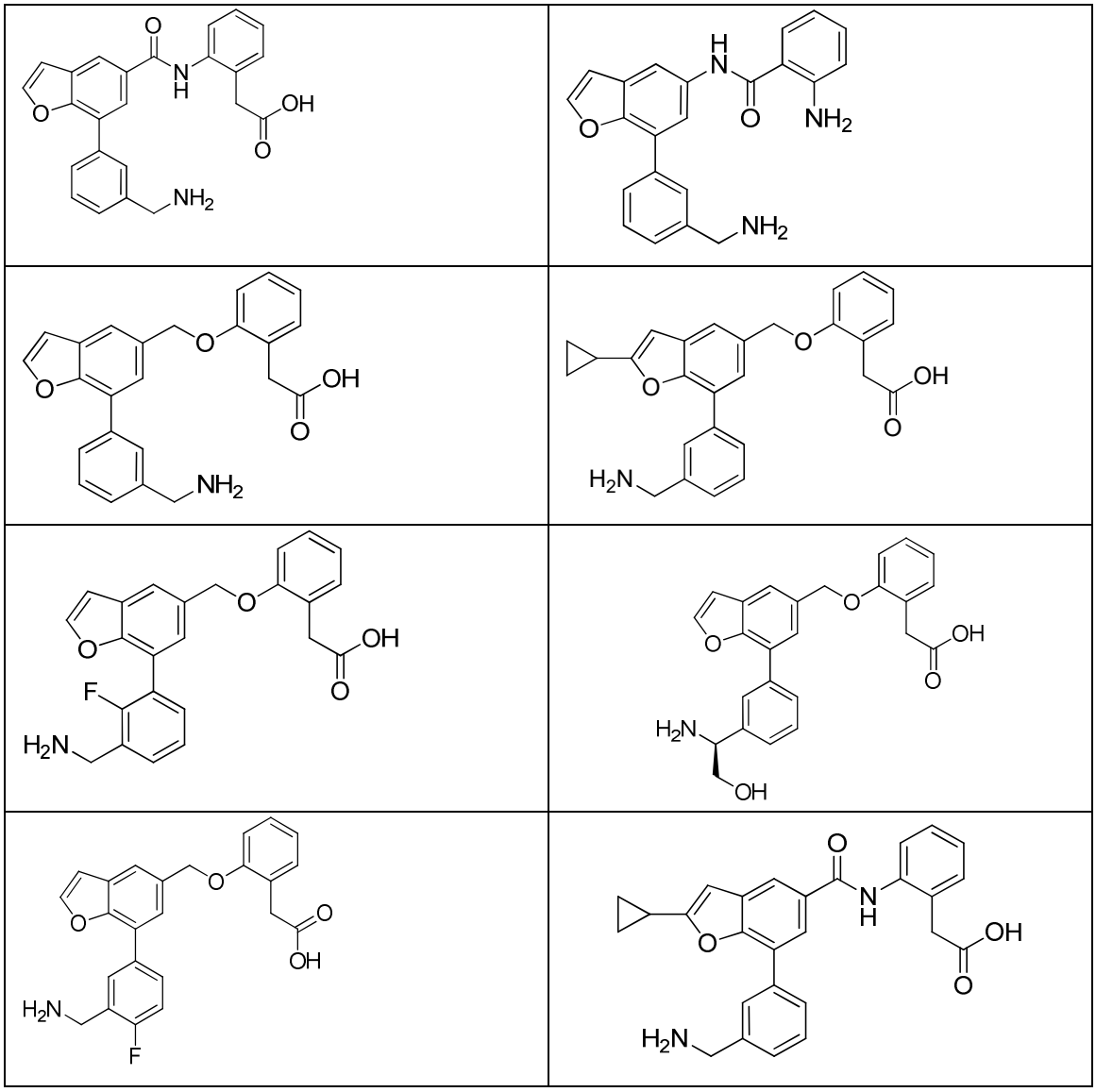
20. The oral dosage form of claim 1, comprising ~~about~~ 1200 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

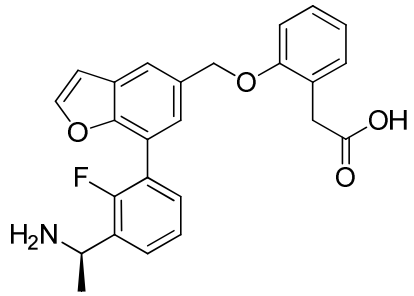
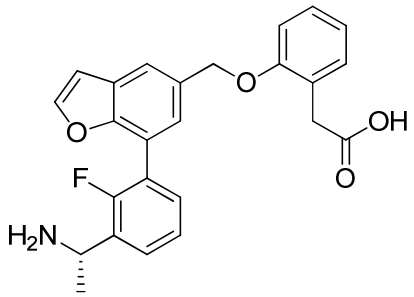
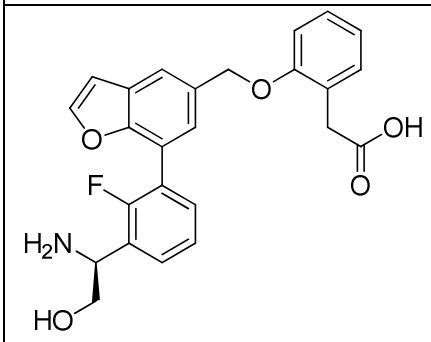
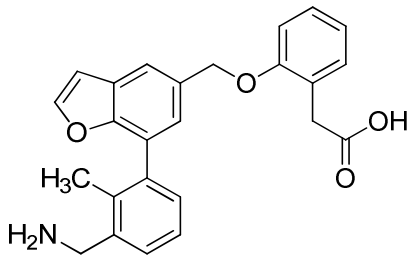
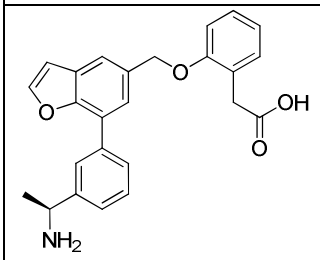
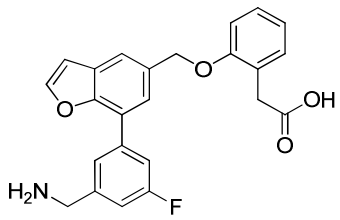
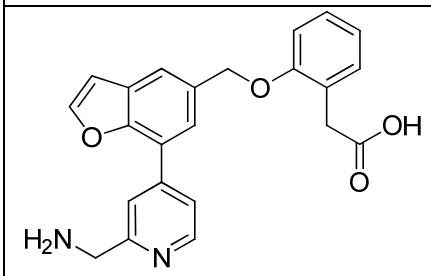
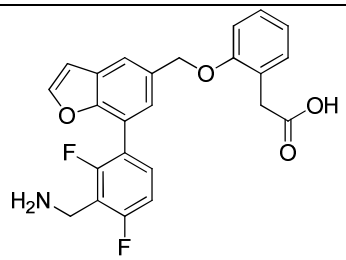
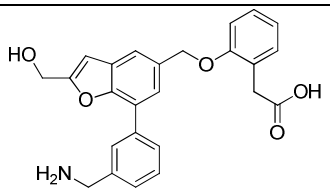
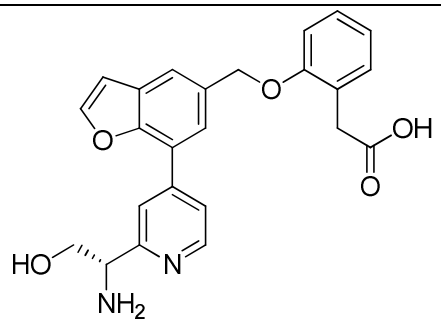
21. The oral dosage form of any one of claims 1-20, wherein the oral dosage form is a capsule.

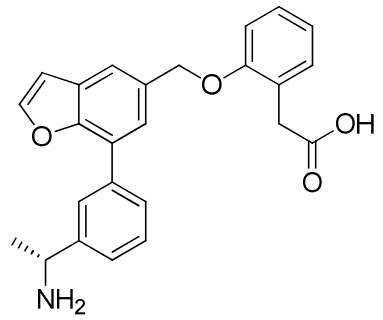
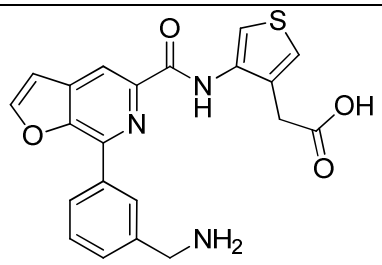
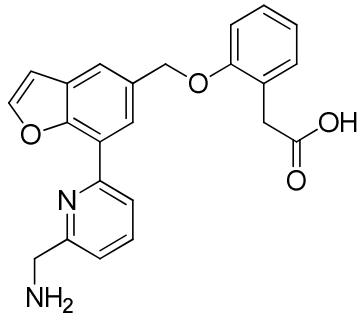
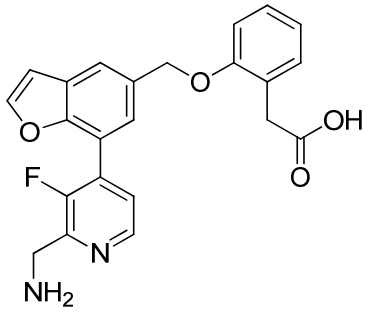
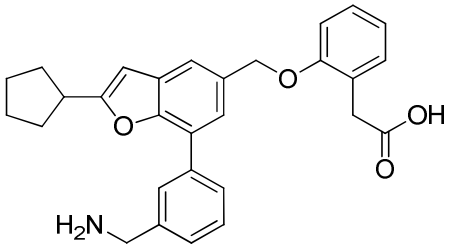
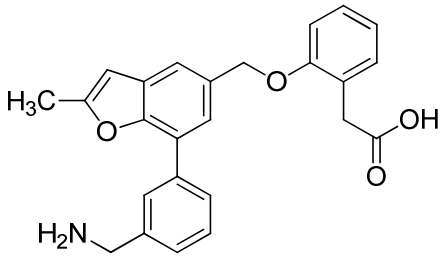
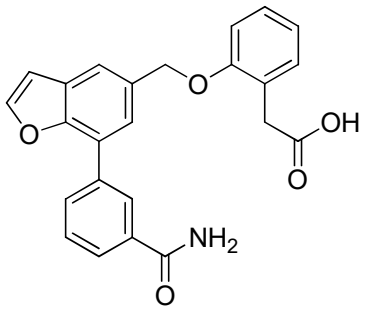
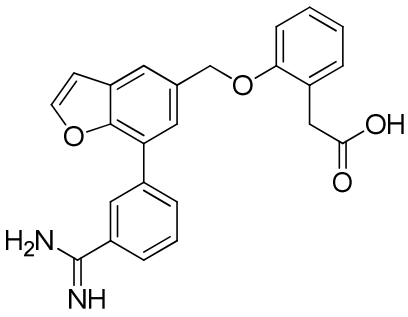
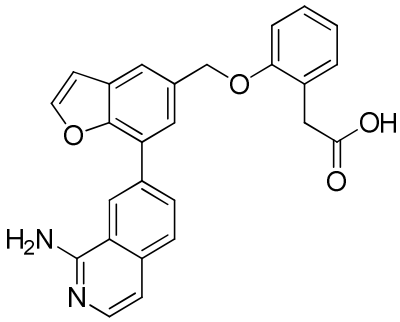
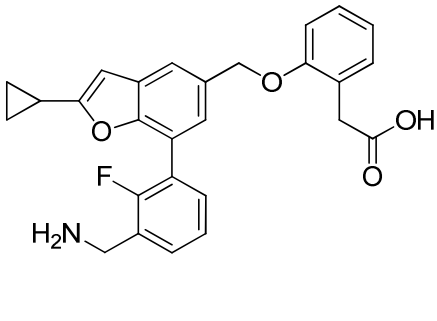
22. The oral dosage form of any one of claims 1-20, wherein the oral dosage form is a tablet.

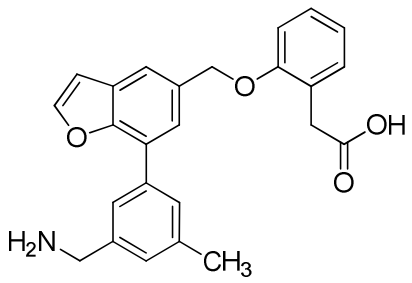
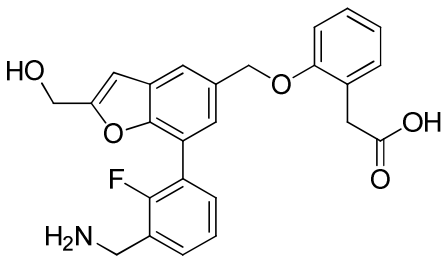
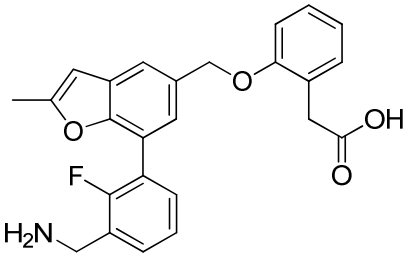
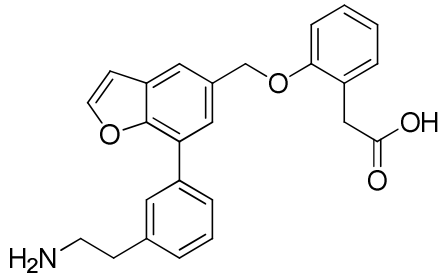
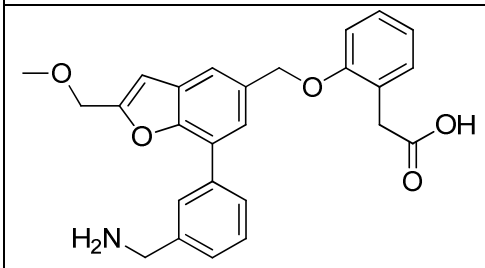
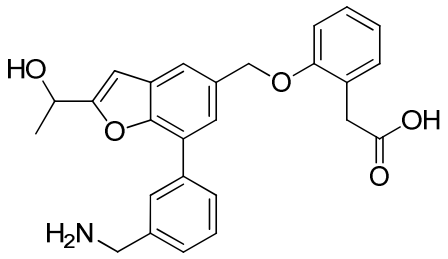
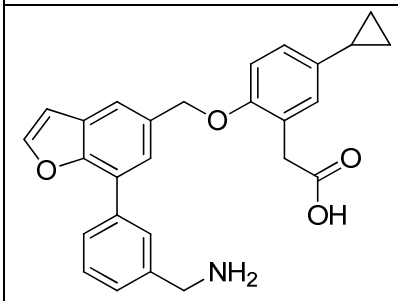
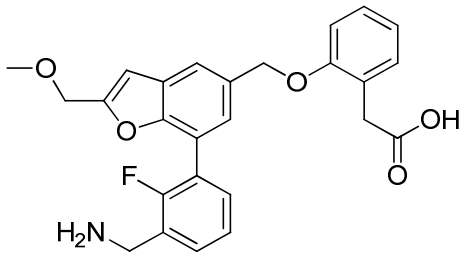
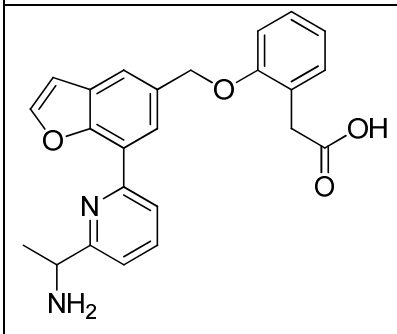
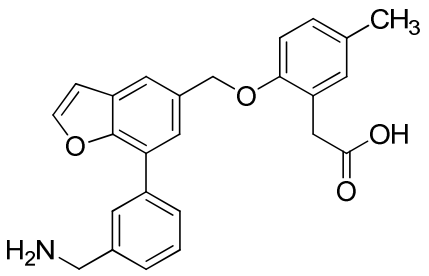
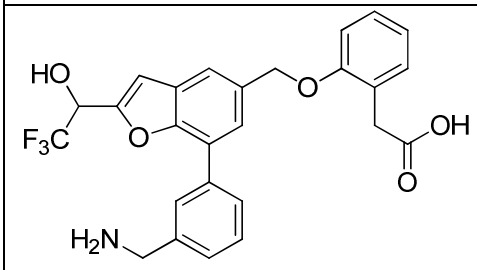
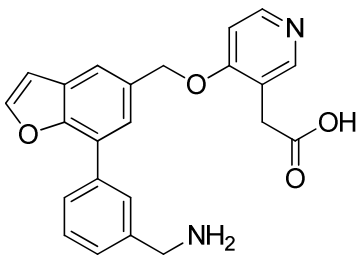
23. The oral dosage form of claim 22, wherein the tablet is a coated tablet.

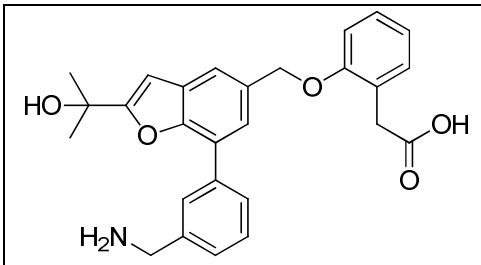
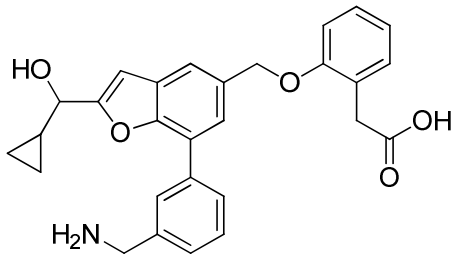
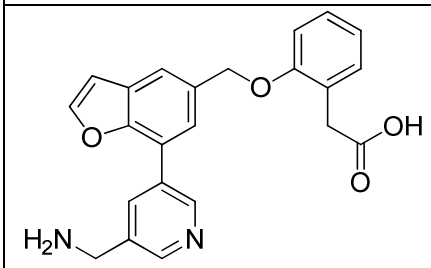
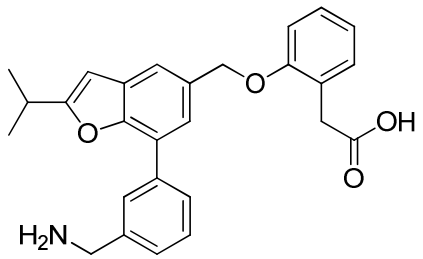
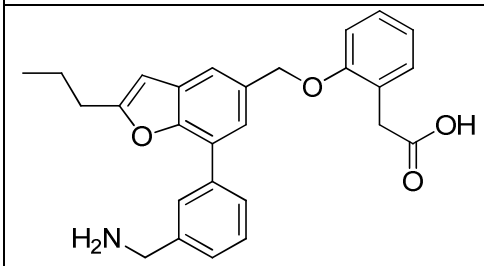
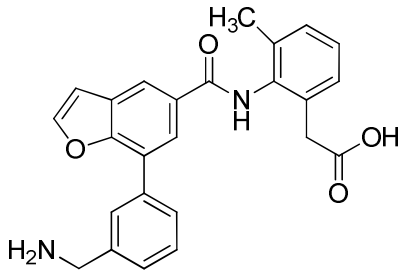
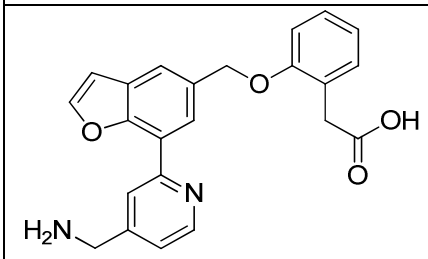
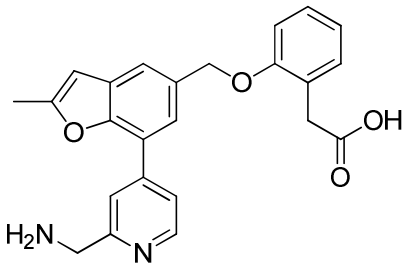
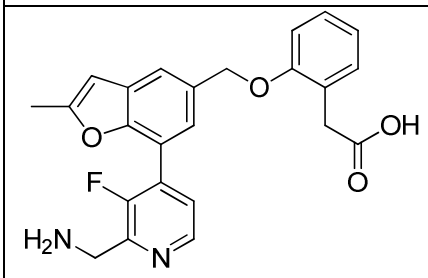
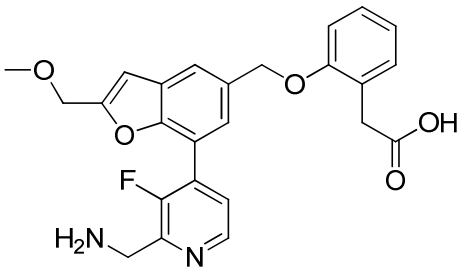
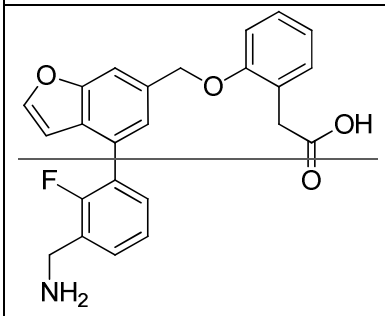
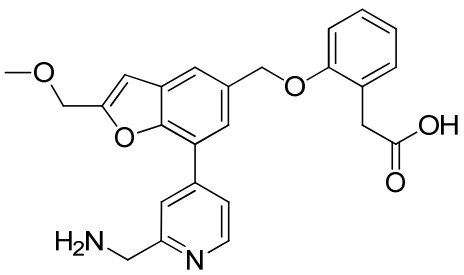
24. Use of a therapeutically effective amount of a compound selected from:

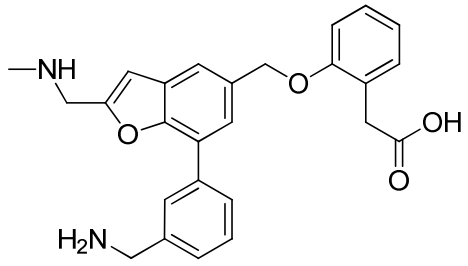
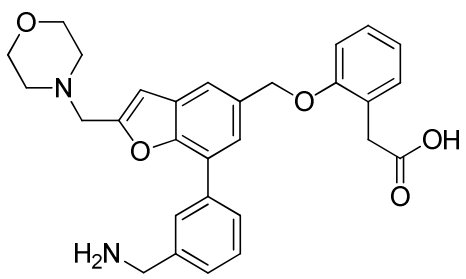
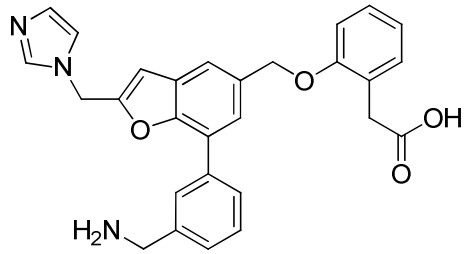
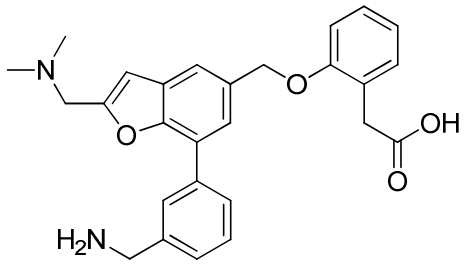
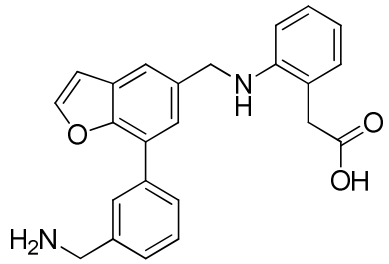
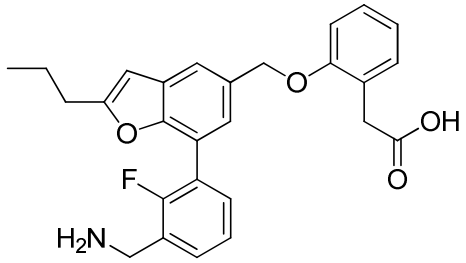
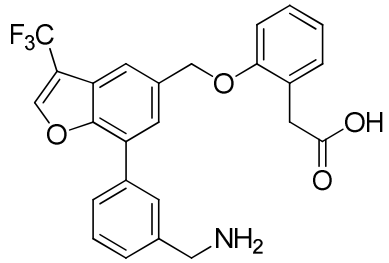
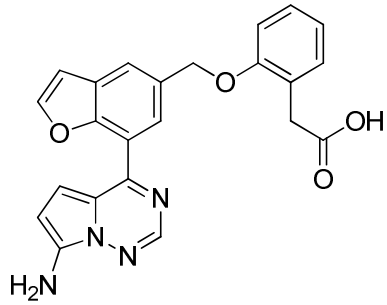
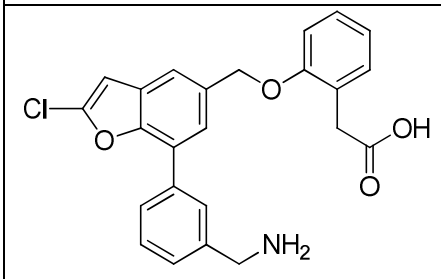
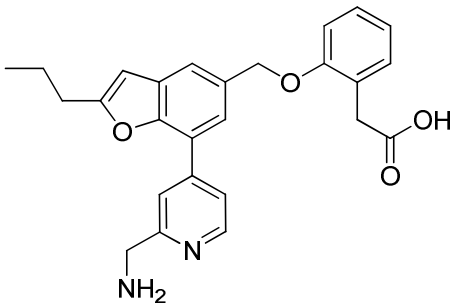
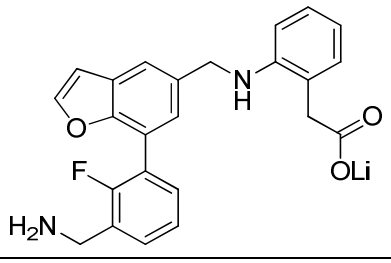
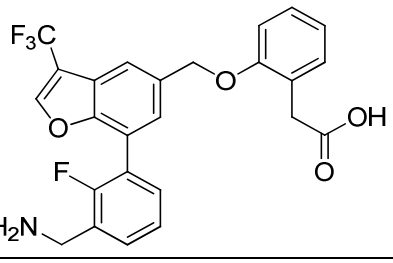


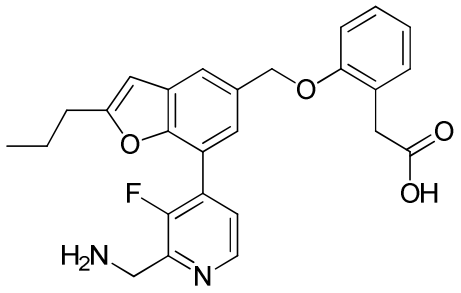
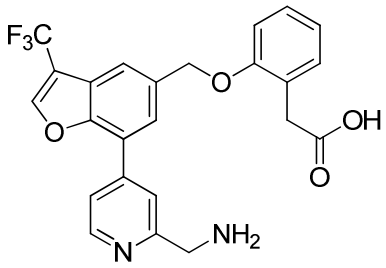
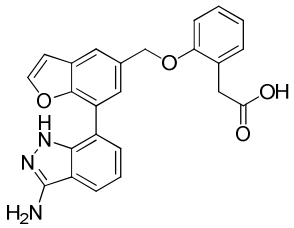
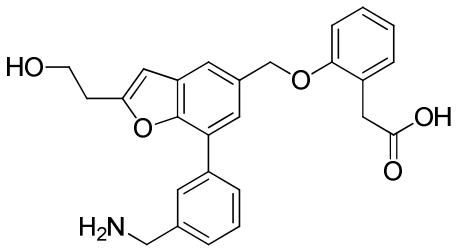
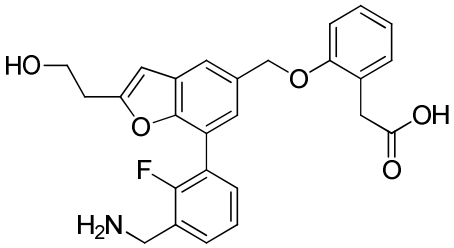
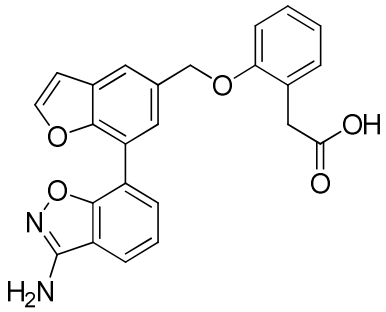
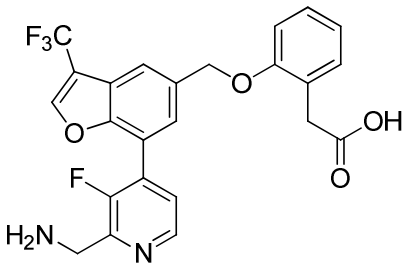
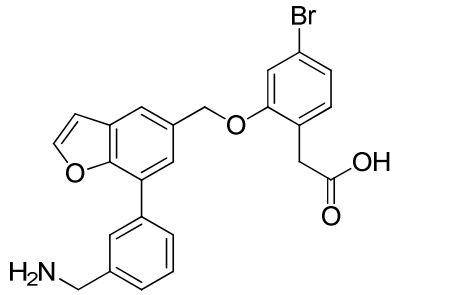
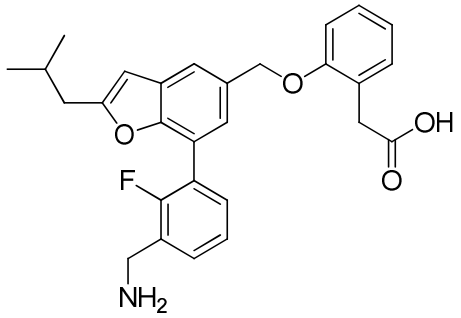
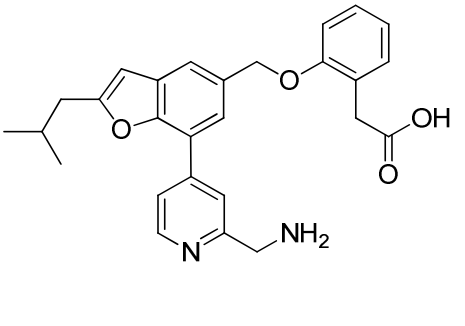
	
	
	
	
	

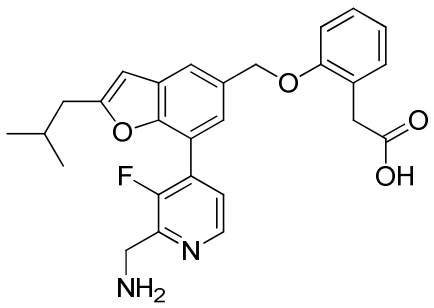
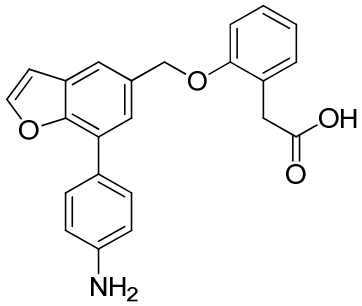
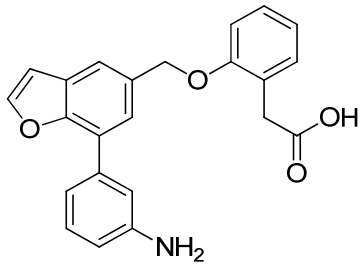
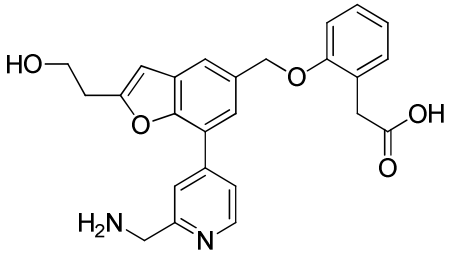
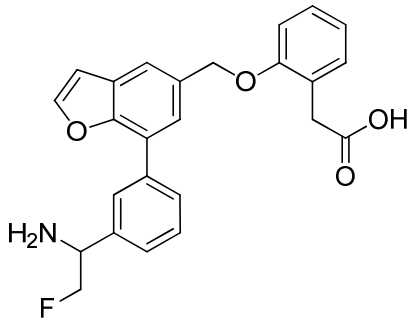
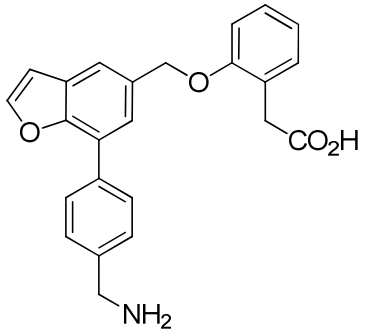
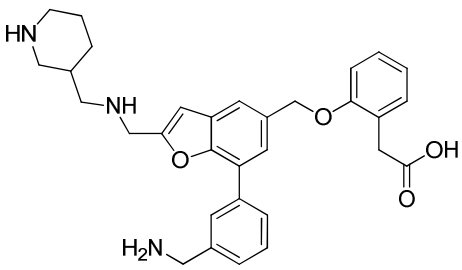
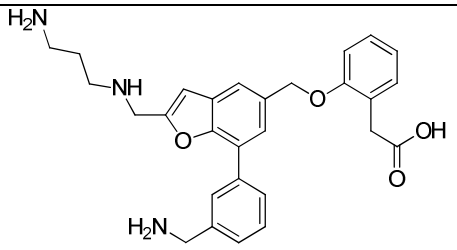
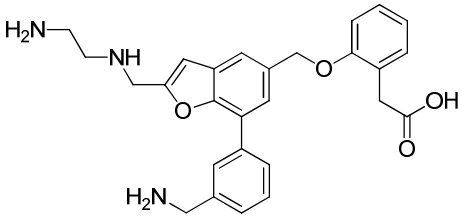
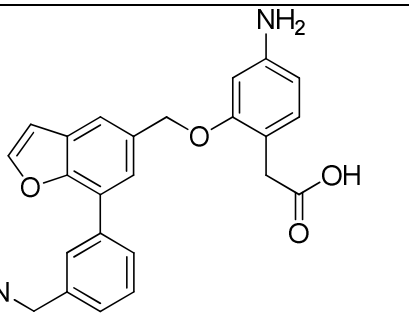
	
	
	
	
	

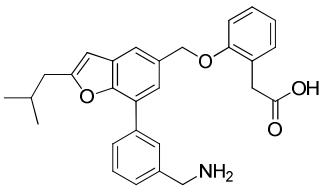
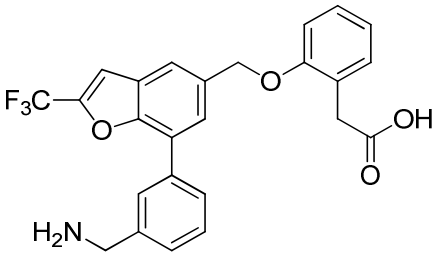
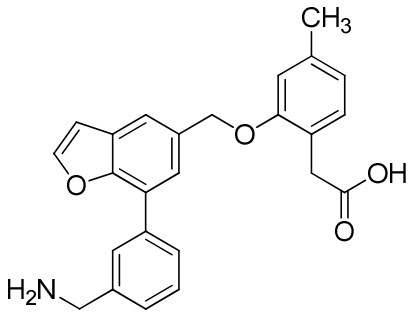
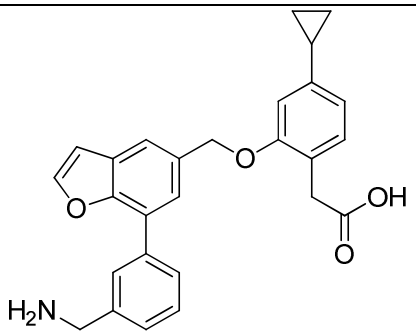
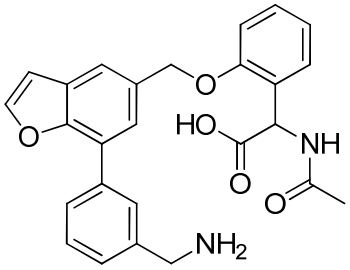
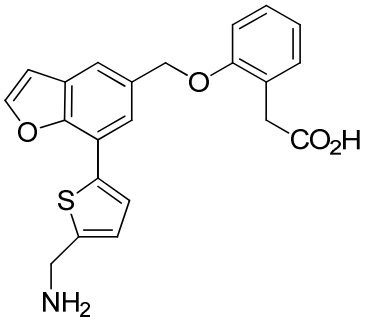
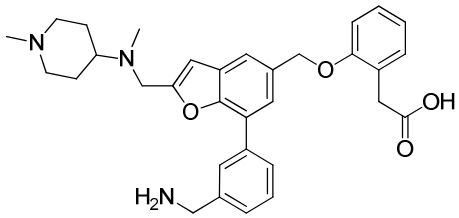
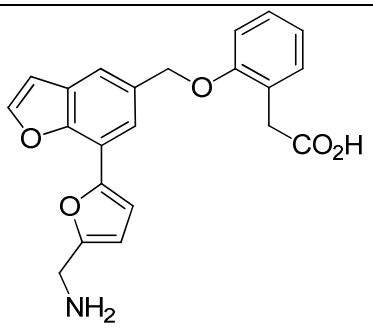
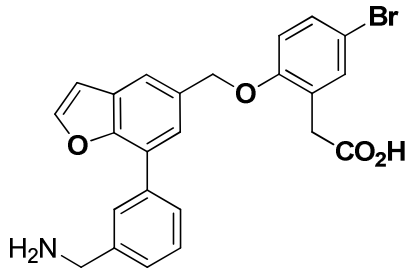
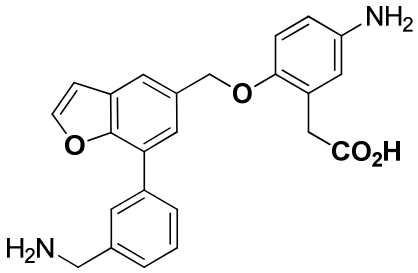
	
	
	
	
	
	

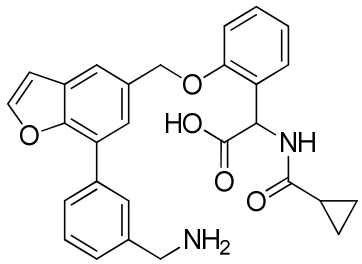
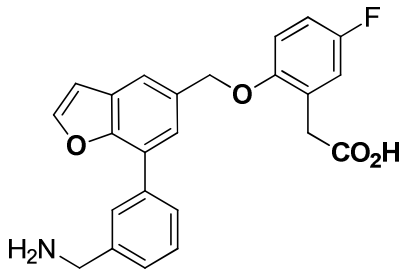
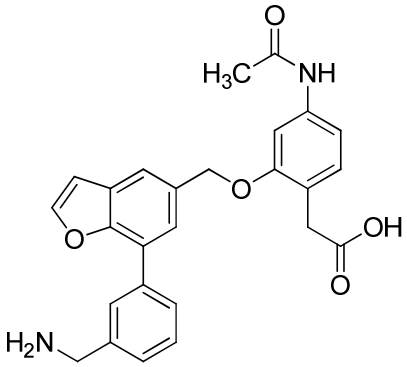
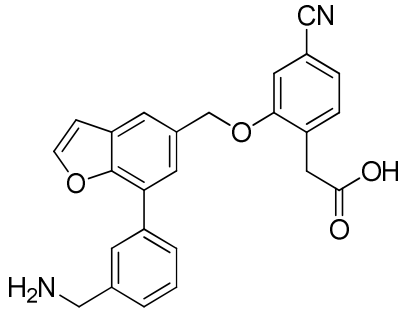
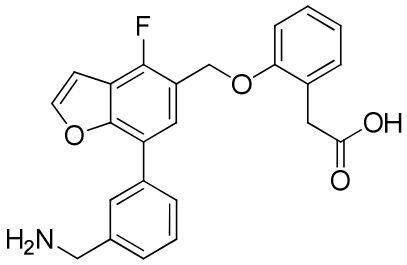
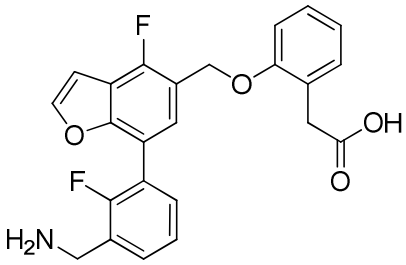
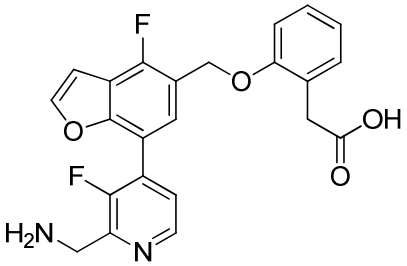
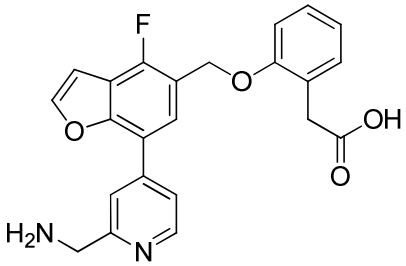
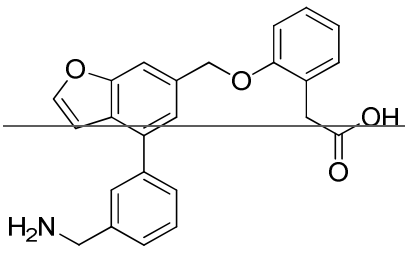
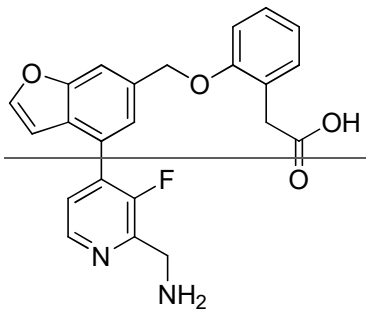
	
	
	
	
	
	

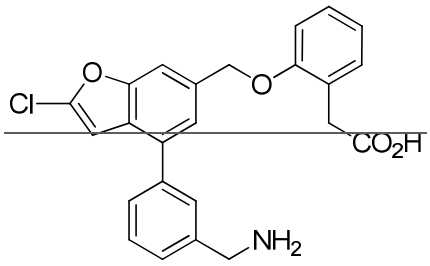
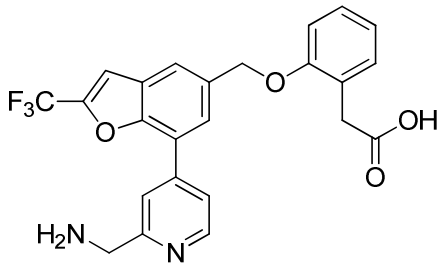
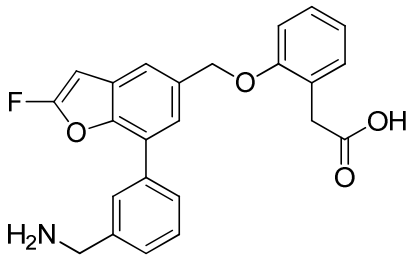
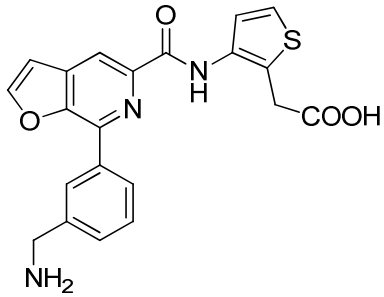
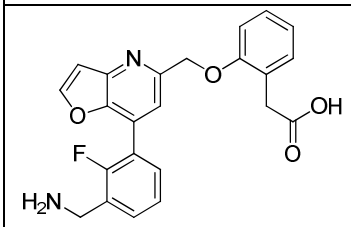
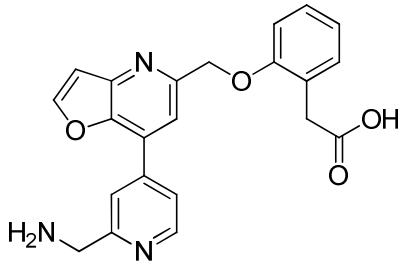
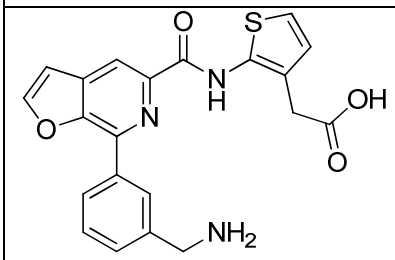
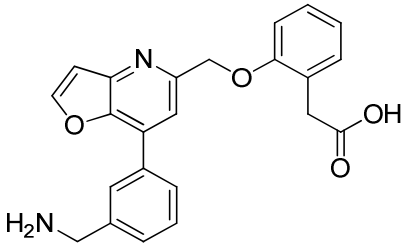
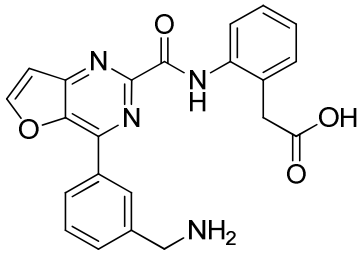
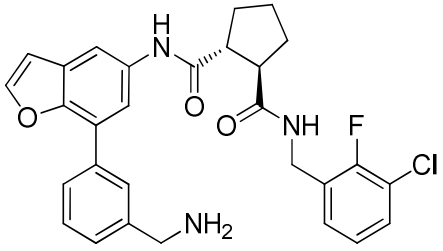
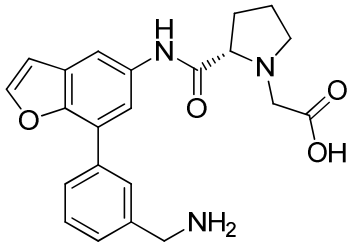
	
	
	
	
	
	

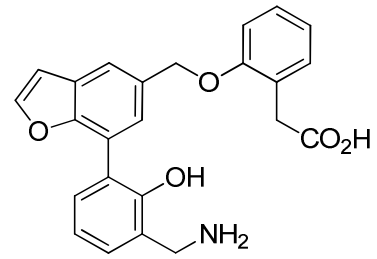
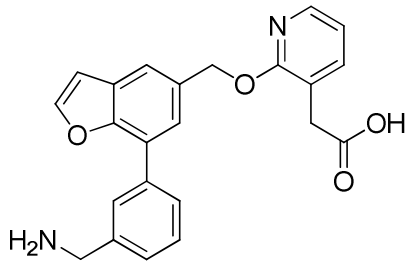
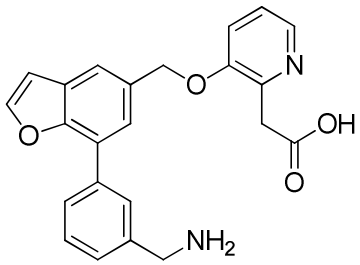
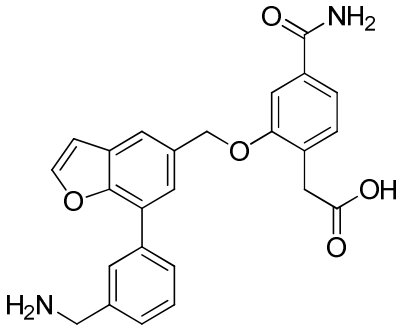
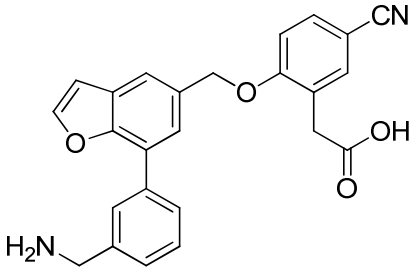
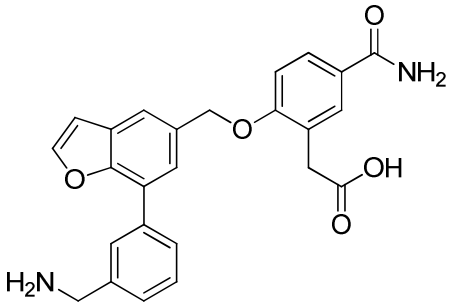
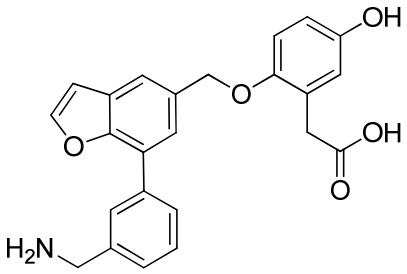
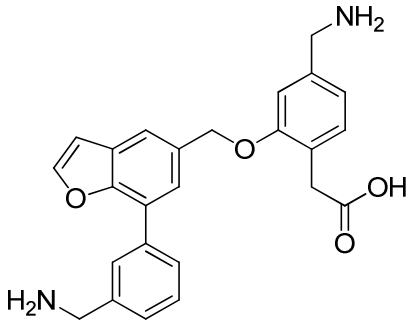
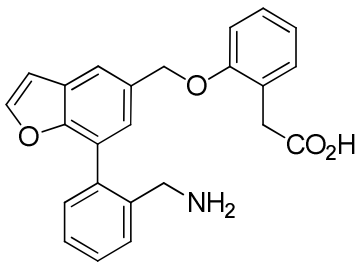
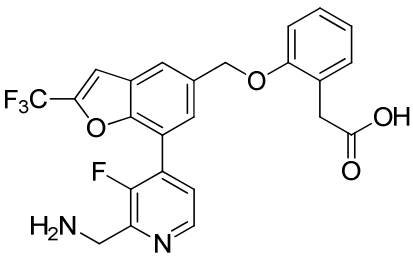
	
	
	
	
	

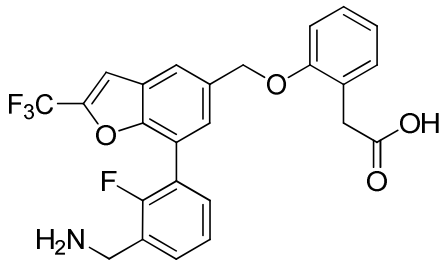
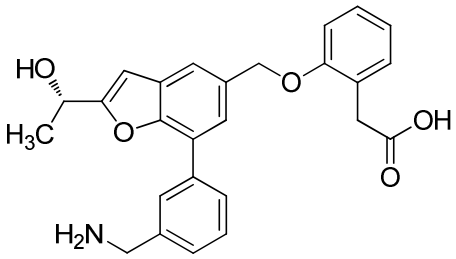
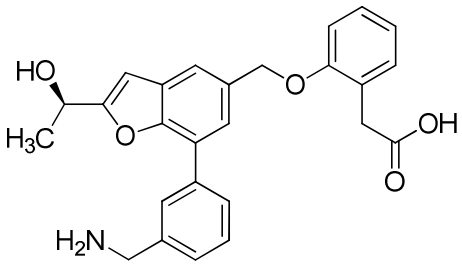
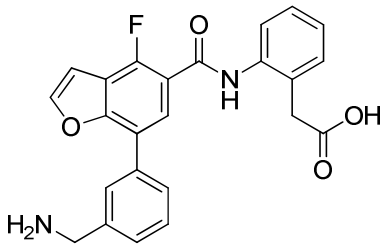
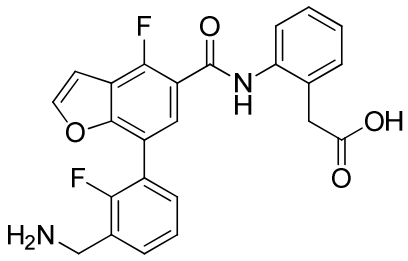
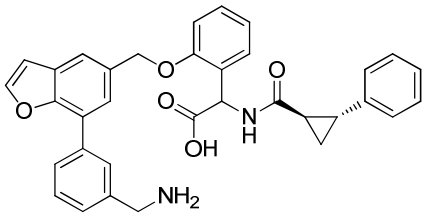
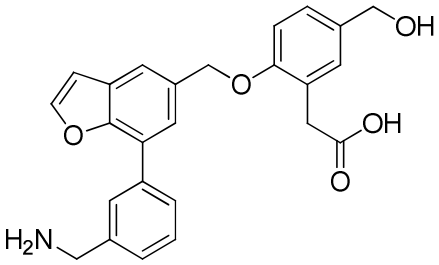
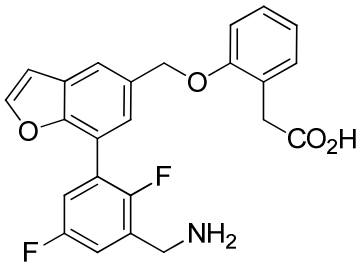
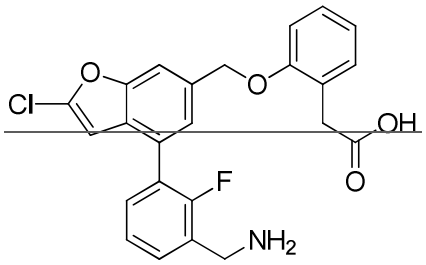
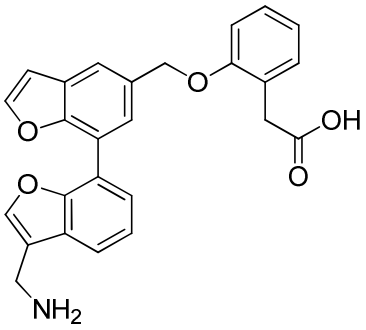
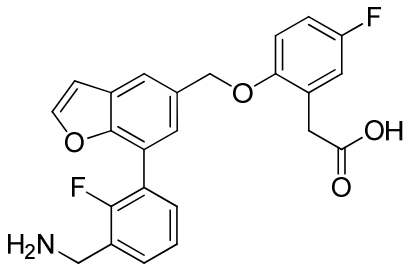
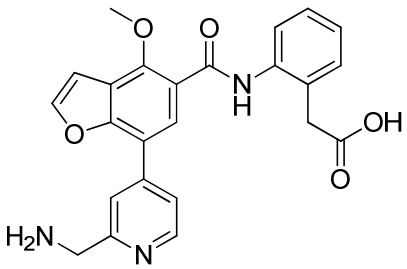
	
	
	
	
	

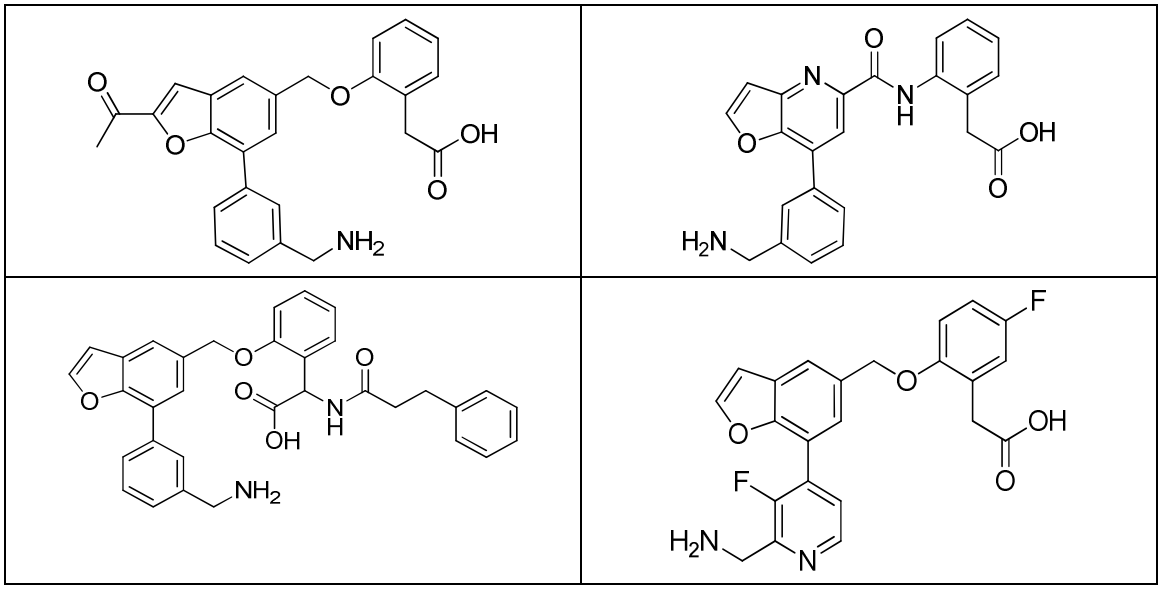
	
	
	
	
	



, or a pharmaceutically acceptable salt ~~or prodrug~~ thereof, in the manufacture of a medicament for treating or preventing a disease or condition characterized by aberrant complement system activity; wherein the disease or condition characterized by aberrant complement system activity is an immunological disorder; a disease of the central nervous system; a neurodegenerative disease or neurological disease; a renal disease; a cardiovascular disease; antineutrophil cytoplasmic antibody (ANCA)-associated vasculitis (AAV), warm autoimmune hemolytic anemia, IgA nephropathy, C3 glomerulonephritis, focal segmental glomerulosclerosis; a hematological disorder; an ocular disorder, an eye disorder; macular degeneration, age-related macular degeneration (AMD), macular edema, diabetic macular edema, choroidal neovascularization (CNV), uveitis, Behcet's uveitis, proliferative diabetic retinopathy, non-proliferative diabetic retinopathy, glaucoma, hypertensive retinopathy, a corneal neovascularization disease, post-corneal transplant rejection, a corneal dystrophic disease, an autoimmune dry eye disease, Stevens-Johnson syndrome, Sjogren's syndrome, an environmental dry eye disease, Fuchs' endothelial dystrophy, retinal vein occlusion, post-operative inflammation; paroxysmal nocturnal hemoglobinuria (PNH), atypical hemolytic uremic syndrome, organ transplant rejection, myasthenia gravis, neuromyelitis optica, membranoproliferative glomerulonephritis, dense-deposit disease, cold agglutinin disease, or catastrophic antiphospholipid syndrome;~~,~~ wherein the medicament is to be orally administered to a patient.

25. The use of claim 24, wherein the disease or condition characterized by aberrant complement system activity is an immunological disorder.

26. The use of claim 24, wherein the disease or condition characterized by aberrant complement system activity is a disease of the central nervous system.

27. The use of claim 24, wherein the disease or condition characterized by aberrant complement system activity is a neurodegenerative disease or neurological disease.

28. The use of claim 24, wherein the disease or condition characterized by aberrant complement system activity is a renal disease.

29. The use of claim 24, wherein the disease or condition characterized by aberrant complement system activity is a cardiovascular disease.

30. The use of claim 24, wherein the disease or condition characterized by aberrant complement system activity is selected from the group consisting of antineutrophil cytoplasmic antibody (ANCA)-associated vasculitis (AAV), warm autoimmune hemolytic anemia, IgA nephropathy, C3 glomerulonephritis, and focal segmental glomerulosclerosis.

31. The use of claim 24, wherein the disease or condition characterized by aberrant complement system activity is a hematological disorder.

32. The use of claim 24, wherein the disease or condition characterized by aberrant complement system activity is an ocular disorder or an eye disorder.

33. The use of claim 24, wherein the disease or condition characterized by aberrant complement system activity is macular degeneration, age-related macular degeneration (AMD), macular edema, diabetic macular edema, choroidal neovascularization (CNV), uveitis, Behcet's uveitis, proliferative diabetic retinopathy, non-proliferative diabetic retinopathy, glaucoma, hypertensive retinopathy, a corneal neovascularization disease, post-corneal transplant rejection, a corneal dystrophic disease, an autoimmune dry eye disease, Stevens-Johnson syndrome, Sjogren's syndrome, an environmental dry eye disease, Fuchs' endothelial dystrophy, retinal vein occlusion, or post-operative inflammation.

34. The use of claim 24, wherein the disease or condition characterized by aberrant complement system activity is selected from the group consisting of paroxysmal nocturnal hemoglobinuria (PNH), atypical hemolytic uremic syndrome, organ transplant rejection, myasthenia gravis, neuromyelitis optica, membranoproliferative glomerulonephritis, dense-deposit disease, cold agglutinin disease, and catastrophic antiphospholipid syndrome.

35. The use of claim 34, wherein the disease is PNH.

36. The use of claim 35, wherein the PNH is characterized by one or more of hemolytic anemia, thrombosis, and impaired bone marrow function.

~~37. The use of any one of claims 34-36, wherein the medicament to be orally administered suppresses hemolysis of erythrocytes or blocks C3 fragment deposition on erythrocytes or both.~~

~~38. The use of claim 37, wherein the suppression is greater than or equal to about 50%.~~

~~39. The use of claim 37, wherein the suppression is greater than or equal to about 80%.~~

~~40. The use of claim 37, wherein the suppression is greater than or equal to about 90%.~~

~~41. The use of claim 37, wherein the suppression is greater than or equal to about 95%.~~

~~42~~37. The use of any one of claims 24-~~41~~36, wherein the therapeutically effective amount is ~~about~~ 1 mg to ~~about~~ 1500 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

~~43~~38. The use of claim ~~42~~37, wherein the therapeutically effective amount is ~~about~~ 1 mg to ~~about~~ 1200 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

~~44~~39. The use of claim ~~42~~37, wherein the therapeutically effective amount is ~~about~~ 1 to ~~about~~ 1000 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

~~45~~40. The use of claim ~~42~~37, wherein the therapeutically effective amount is ~~about~~ 1 to ~~about~~ 800 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

~~46~~41. The use of claim ~~42~~37, wherein the therapeutically effective amount is ~~about~~ 1 to ~~about~~ 600 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

~~47~~42. The use of claim ~~42~~37, wherein the therapeutically effective amount is ~~about~~ 1 to ~~about~~ 400 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

~~48~~43. The use of claim ~~42~~37, wherein the therapeutically effective amount is ~~about~~ 1 to ~~about~~ 300 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

~~49~~44. The use of claim ~~42~~37, wherein the therapeutically effective amount is ~~about~~ 1 to ~~about~~ 200 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

~~50~~45. The use of claim ~~42~~37, wherein the therapeutically effective amount is ~~about~~ 1 to ~~about~~ 100 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

~~51~~46. The use of claim ~~42~~37, wherein the therapeutically effective amount is ~~about~~ 10 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

~~52~~47. The use of claim ~~42~~37, wherein the therapeutically effective amount is ~~about~~ 30 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

~~53~~48. The use of claim ~~42~~37, wherein the therapeutically effective amount is ~~about~~ 100 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

~~54~~49. The use of claim ~~42~~37, wherein the therapeutically effective amount is ~~about~~ 200 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

~~55~~50. The use of claim ~~42~~37, wherein the therapeutically effective amount is ~~about~~ 300 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

~~56~~51. The use of claim ~~42~~37, wherein the therapeutically effective amount is ~~about~~ 400 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

~~57~~52. The use of claim ~~42~~37, wherein the therapeutically effective amount is ~~about~~ 600 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

~~58~~53. The use of claim ~~42~~37, wherein the therapeutically effective amount is ~~about~~ 800 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

~~59~~54. The use of claim ~~42~~37, wherein the therapeutically effective amount is ~~about~~ 1000 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

~~60~~55. The use of claim ~~42~~37, wherein the therapeutically effective amount is ~~about~~ 1200 mg of the compound or a pharmaceutically acceptable salt ~~or prodrug~~ thereof.

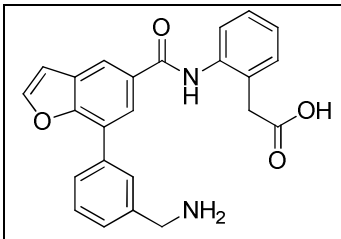
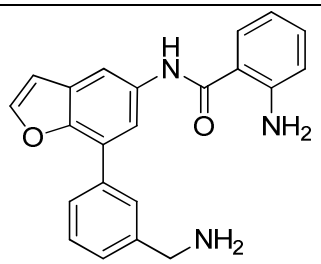
~~61. The use of any one of claims 24-60, wherein the medicament to be orally administered is to be administered once daily.~~

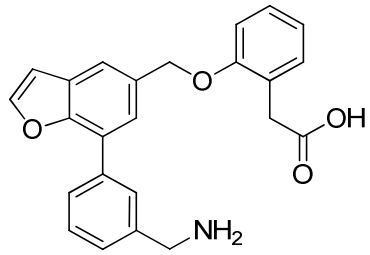
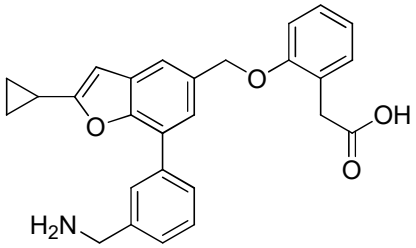
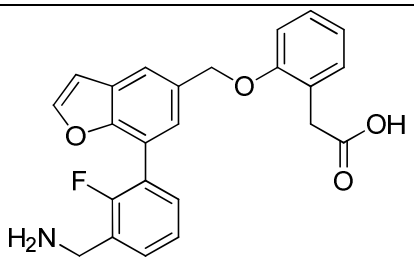
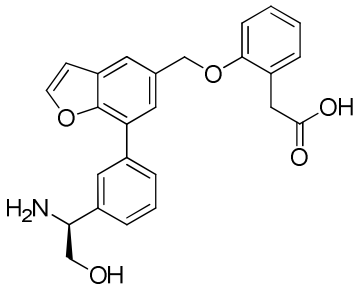
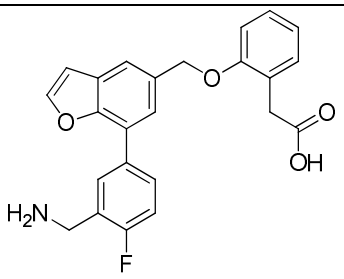
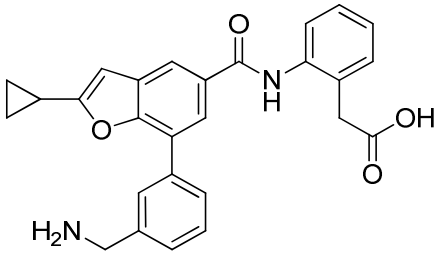
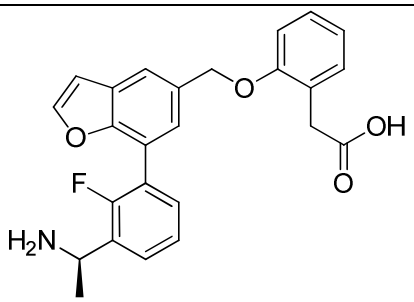
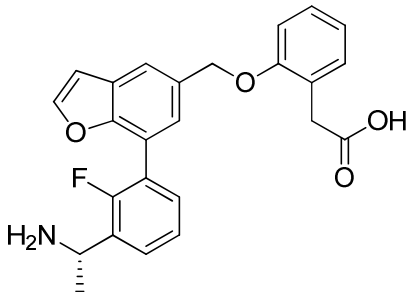
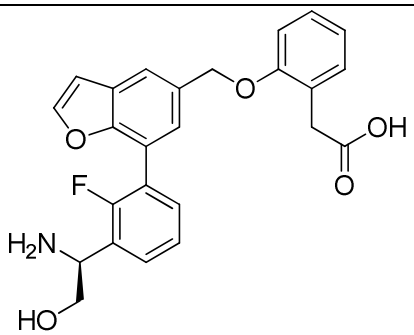
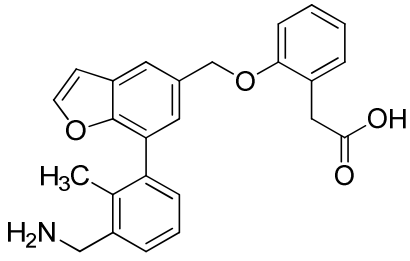
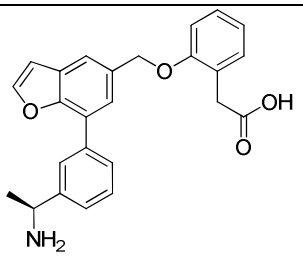
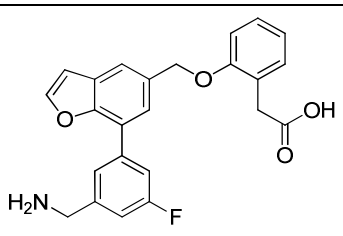
~~62. The use of any one of claims 24-60, wherein medicament to be orally administered is to be administered twice daily.~~

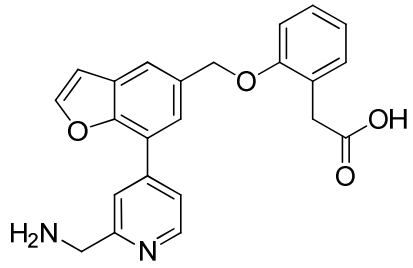
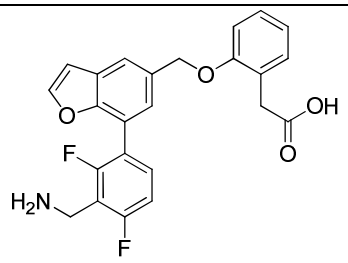
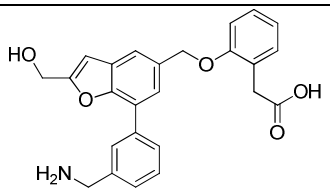
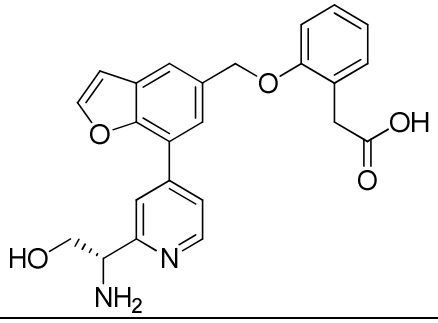
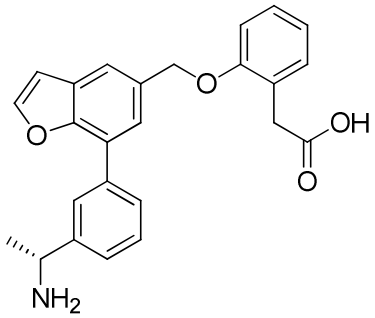
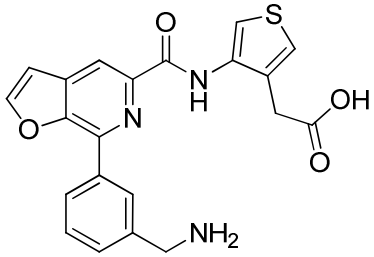
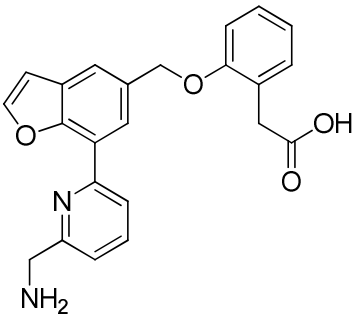
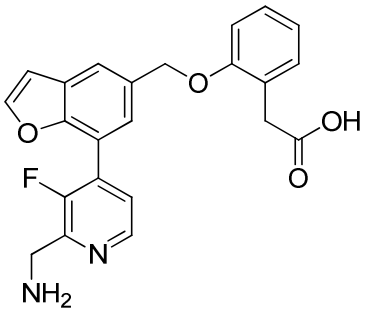
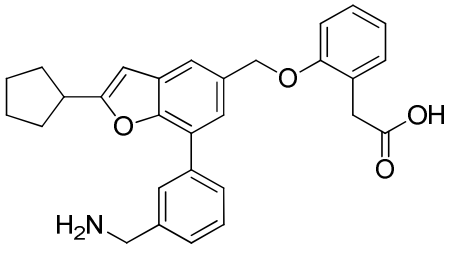
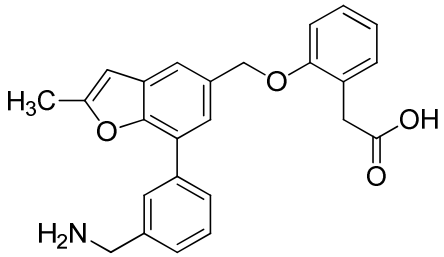
~~63. The use of any one of claims 24-60, wherein the medicament to be orally administered is to be administered three times daily.~~

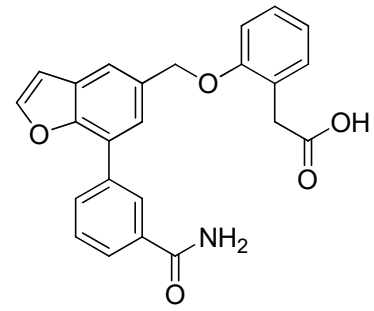
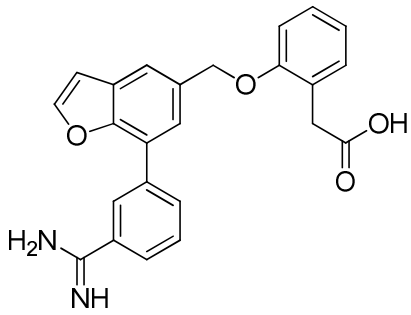
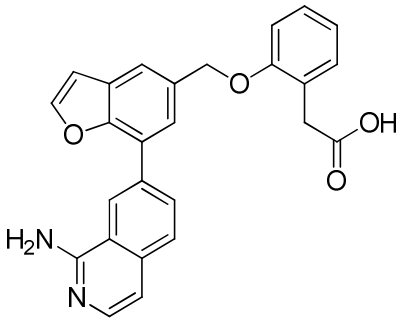
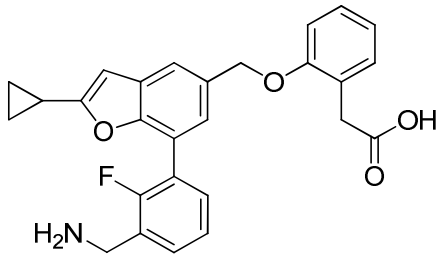
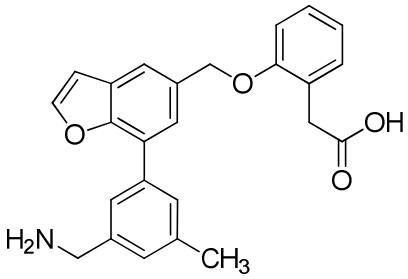
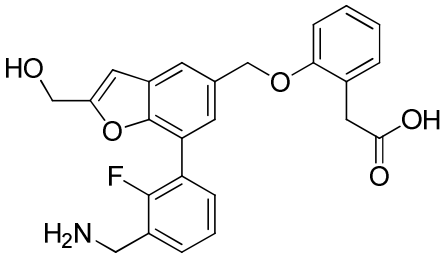
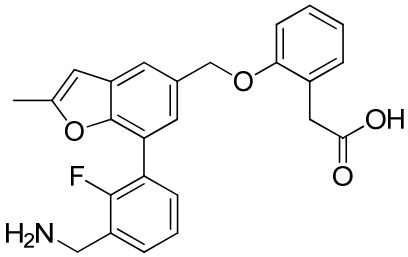
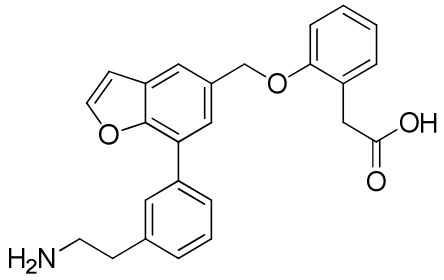
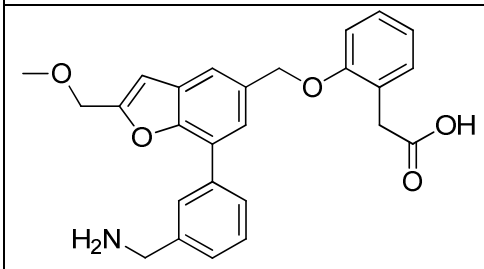
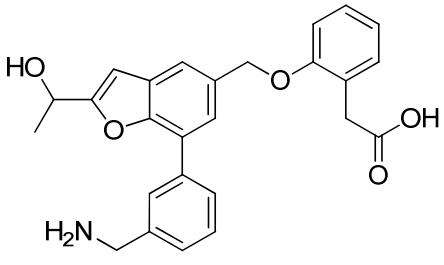
~~64. The use of any one of claims 24-63, wherein the medicament to be orally administered is to be administered as an oral dosage form of any one of claims 1-23.~~

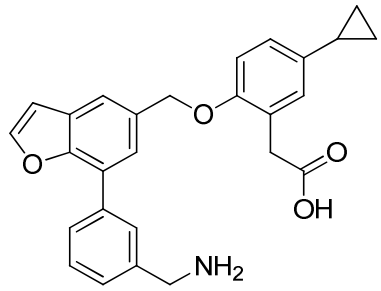
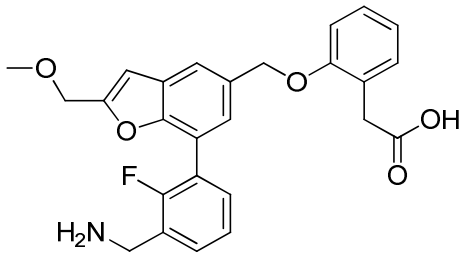
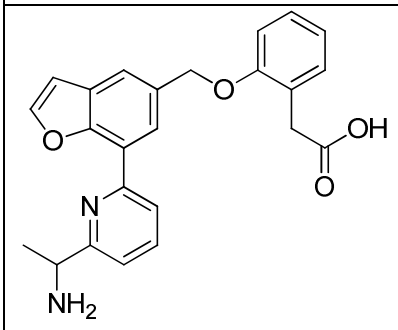
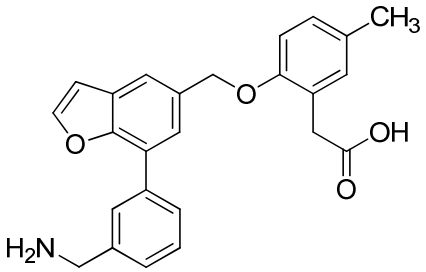
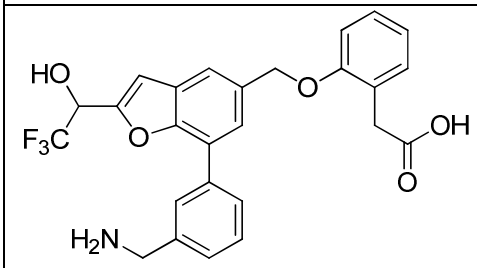
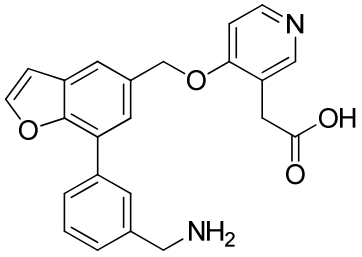
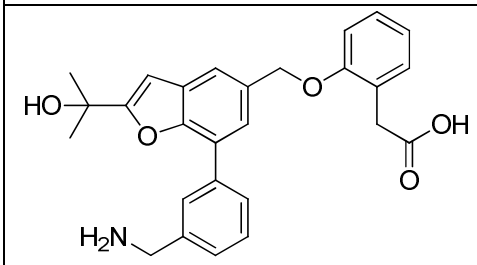
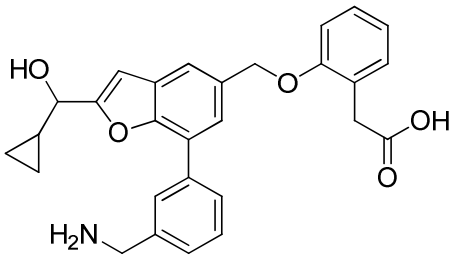
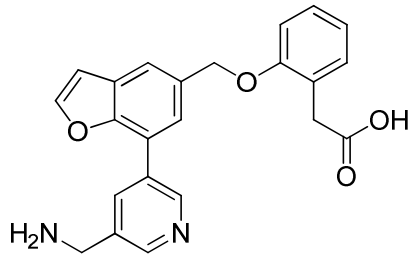
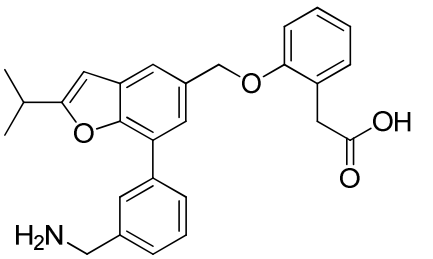
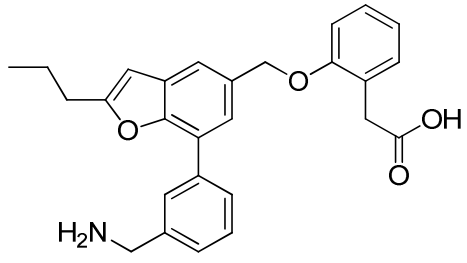
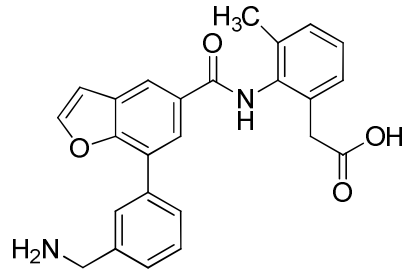
~~65~~56. An oral dosage form, comprising a compound selected from:

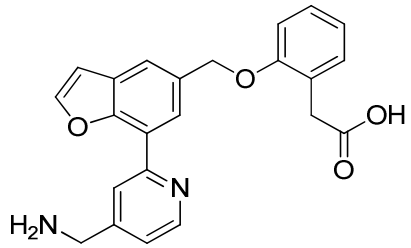
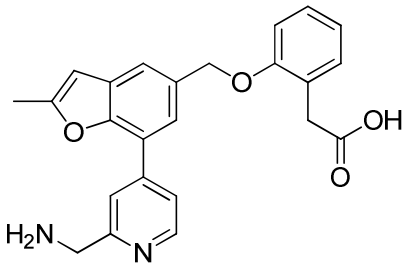
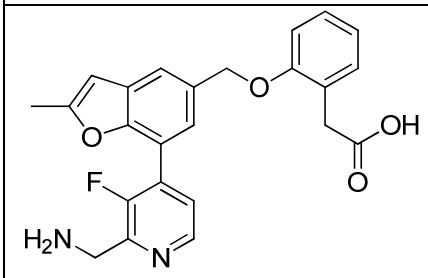
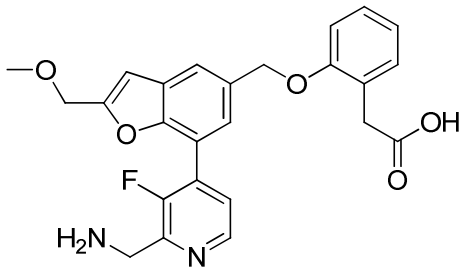
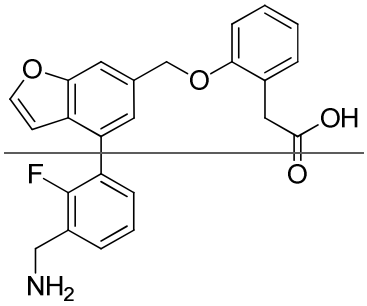
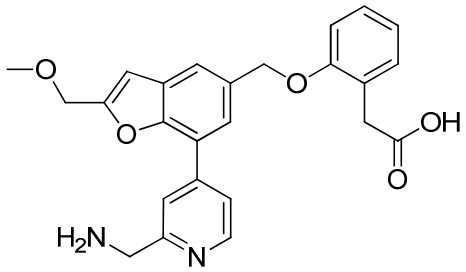
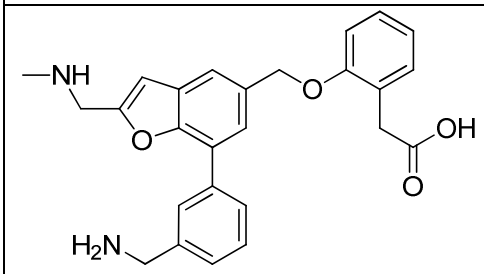
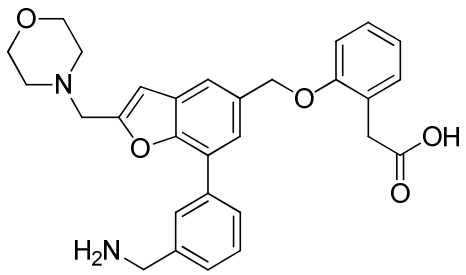
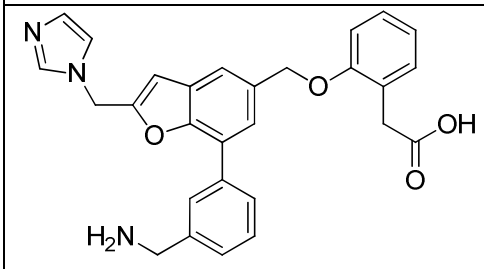
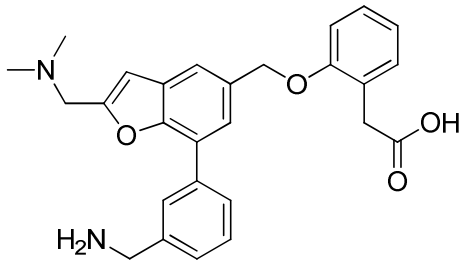
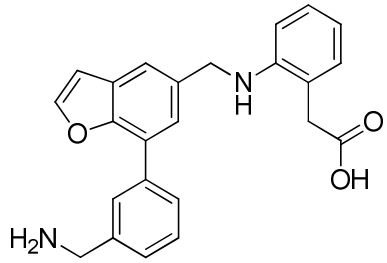
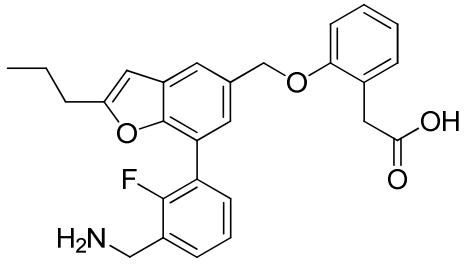
	

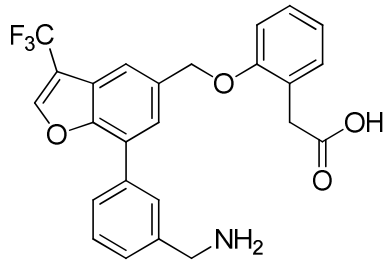
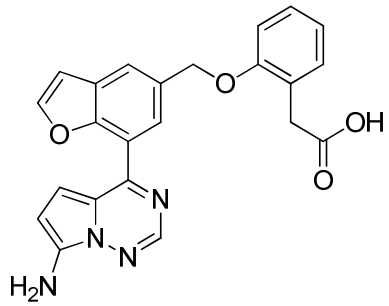
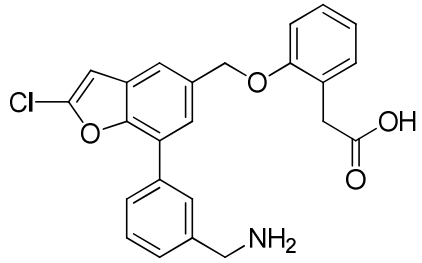
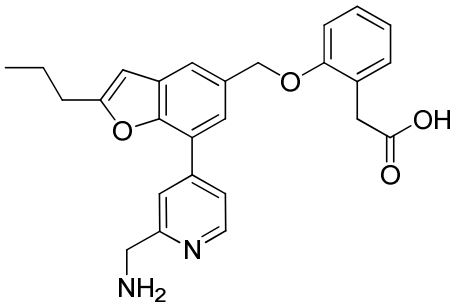
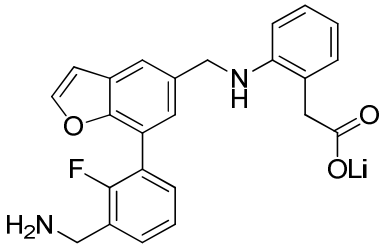
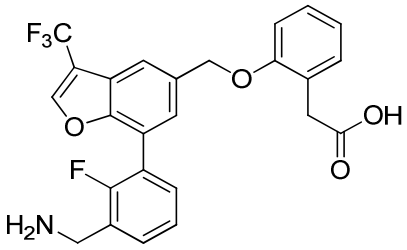
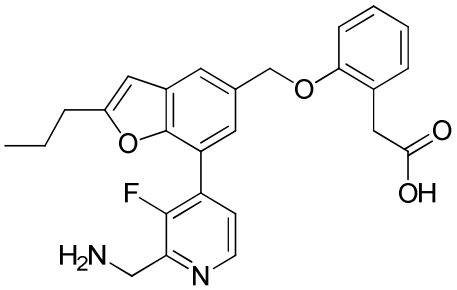
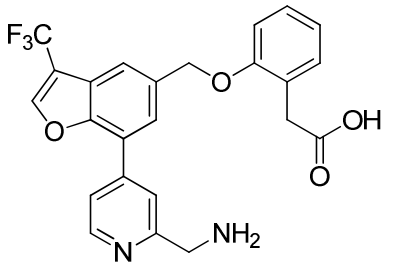
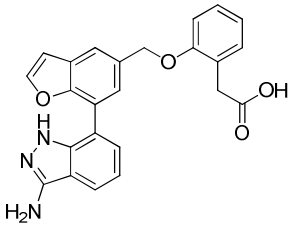
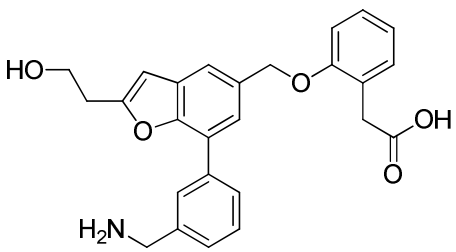
	
	
	
	
	
	

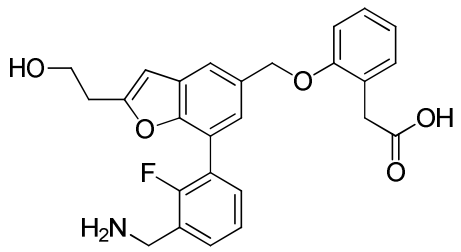
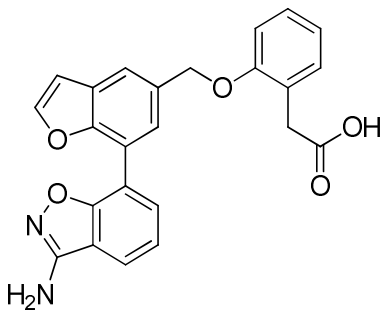
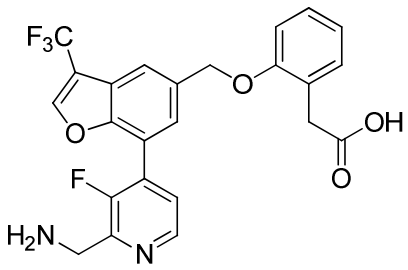
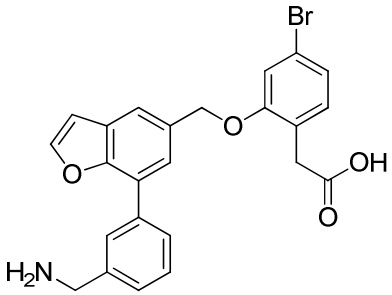
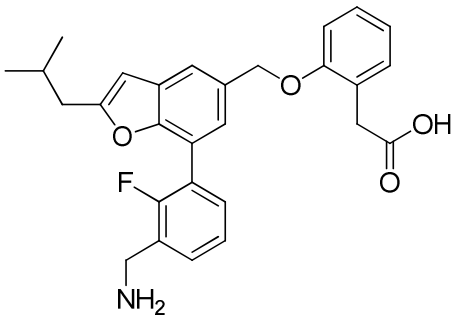
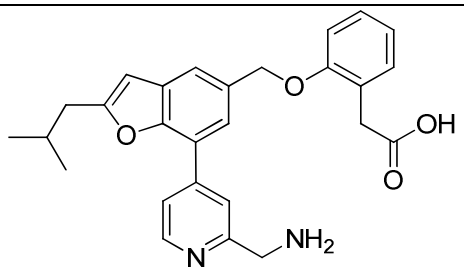
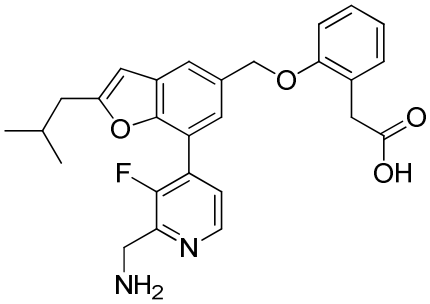
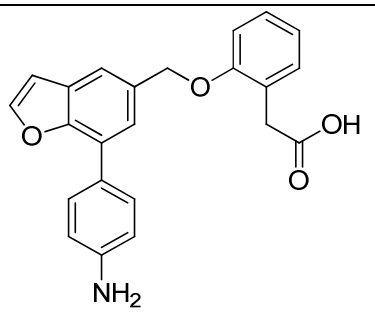
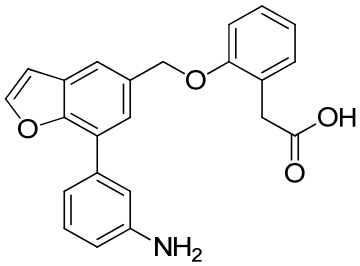
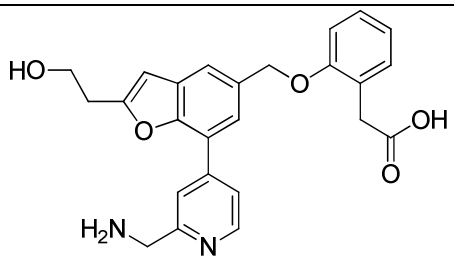
	
	
	
	
	

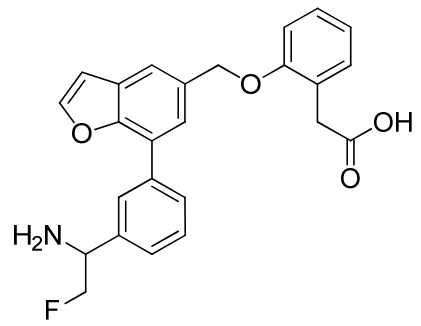
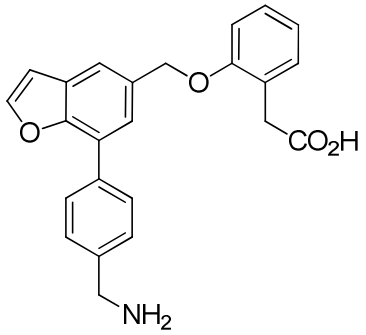
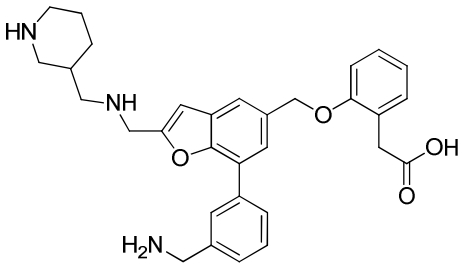
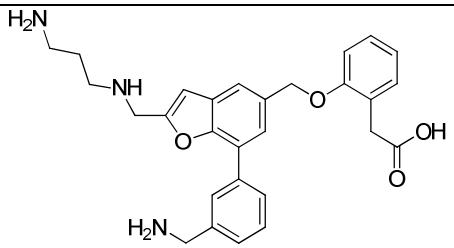
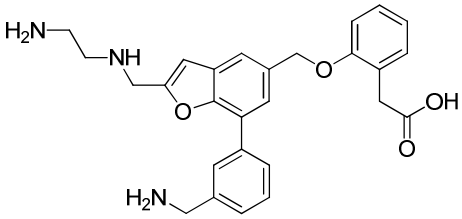
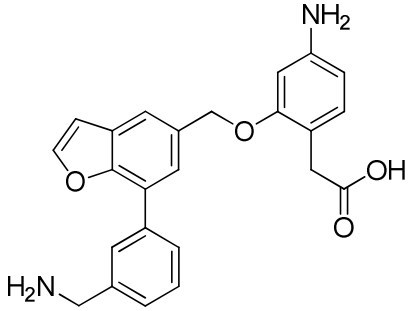
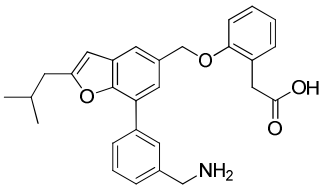
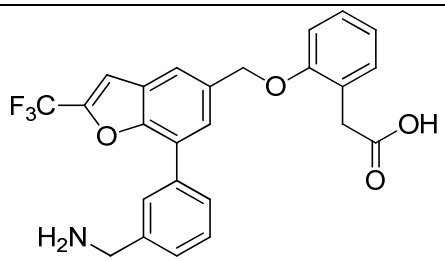
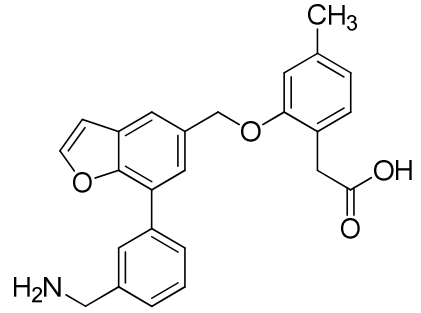
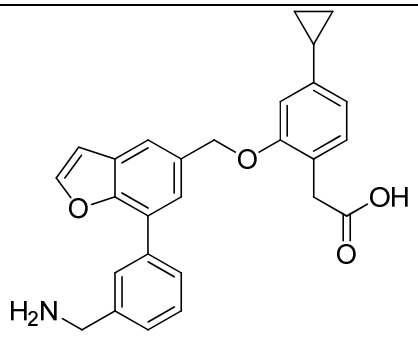
	
	
	
	
	

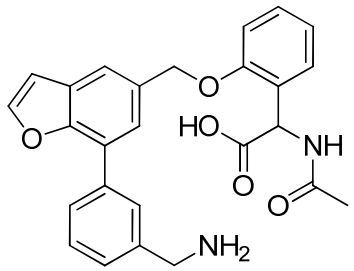
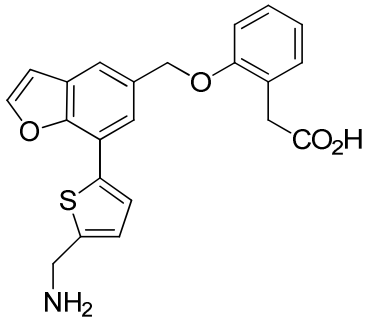
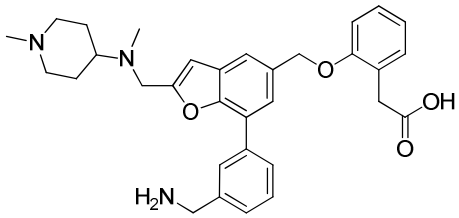
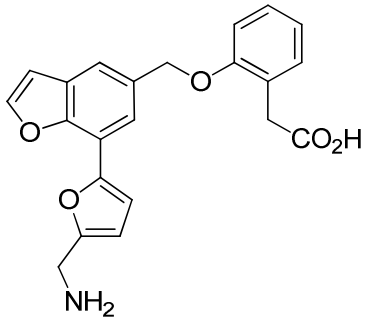
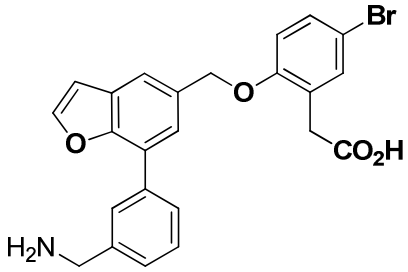
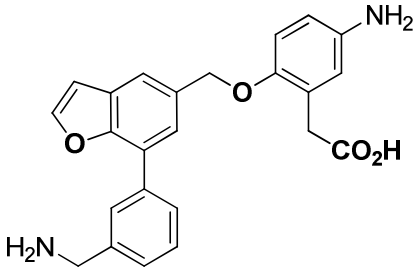
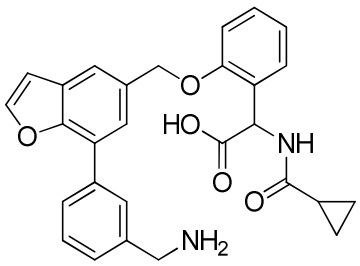
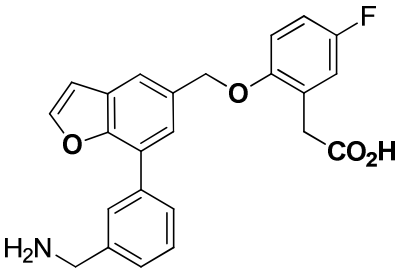
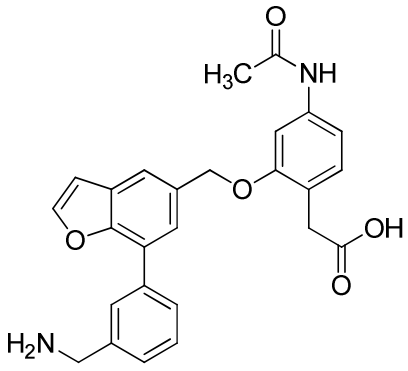
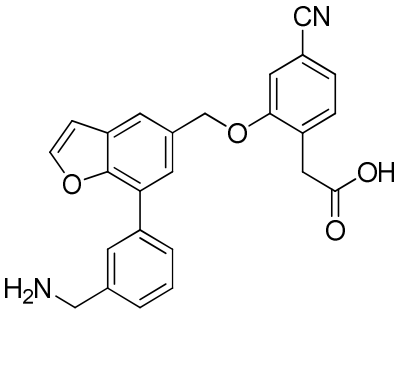
	
	
	
	
	
	

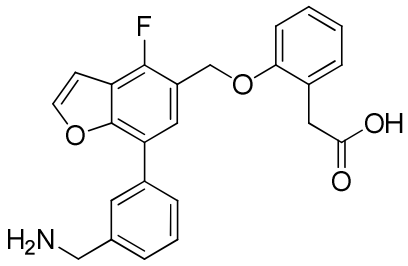
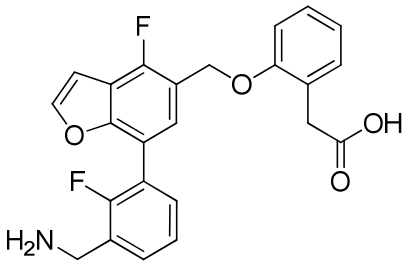
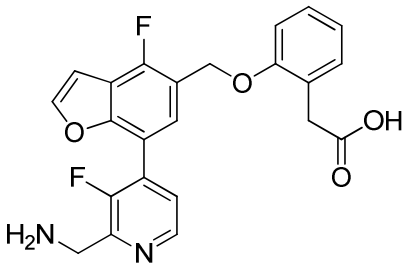
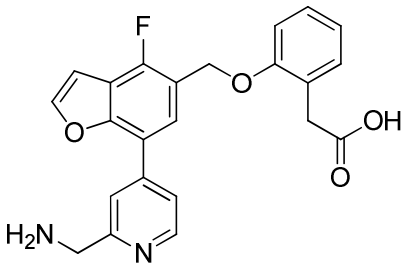
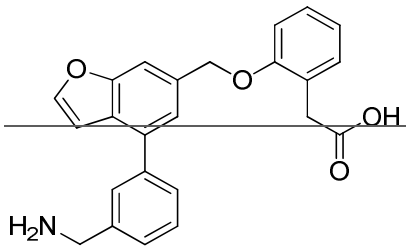
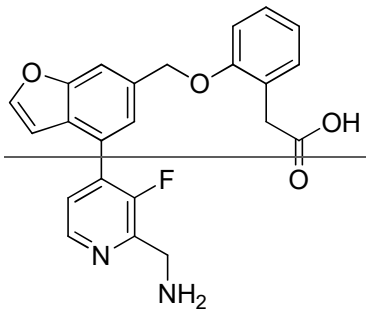
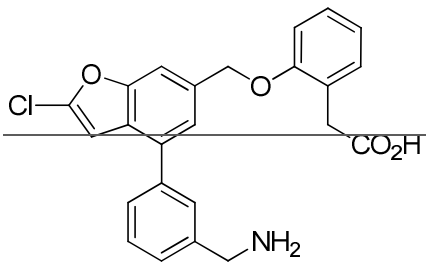
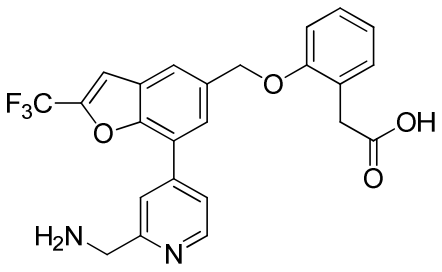
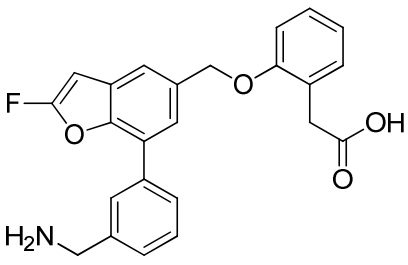
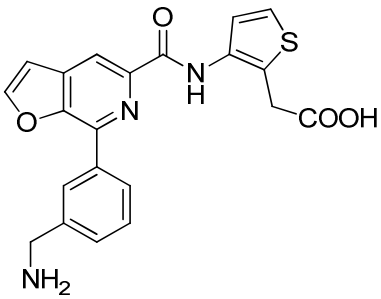
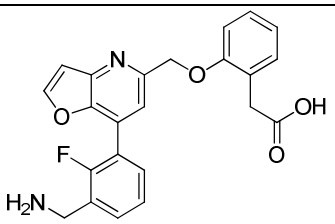
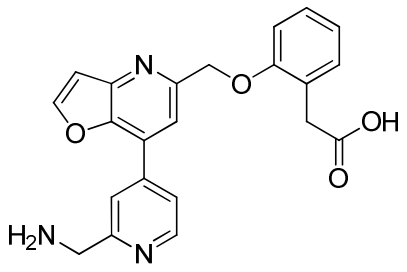
	
	
	
	
	
	

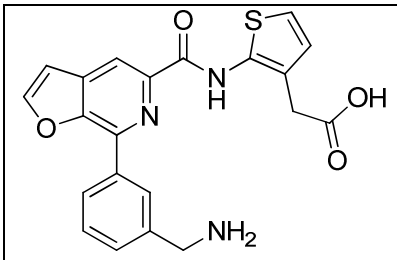
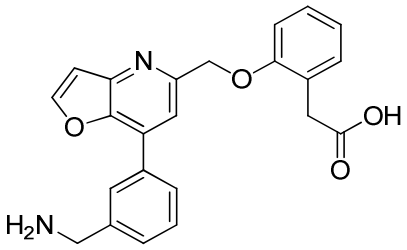
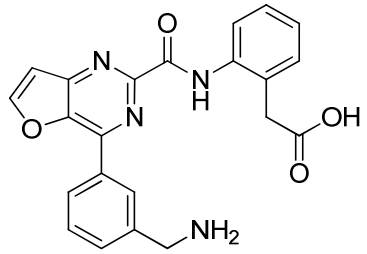
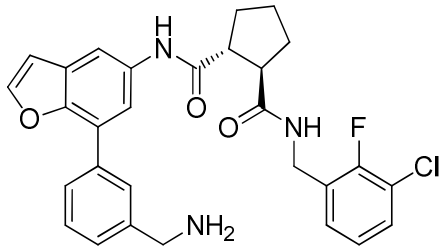
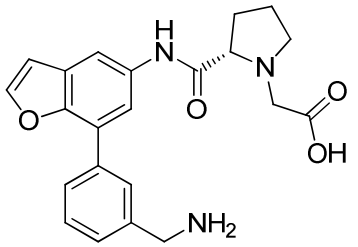
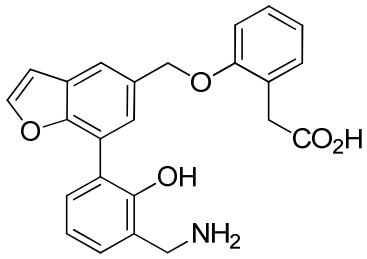
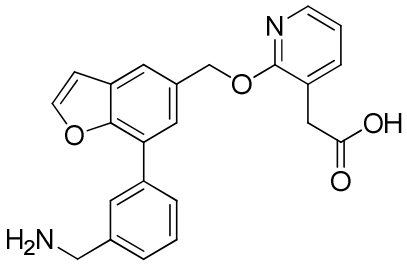
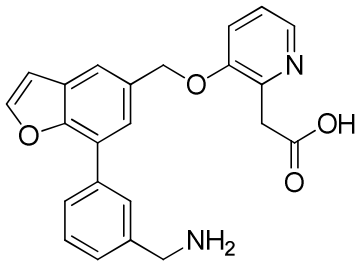
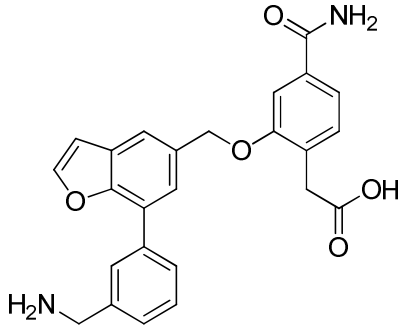
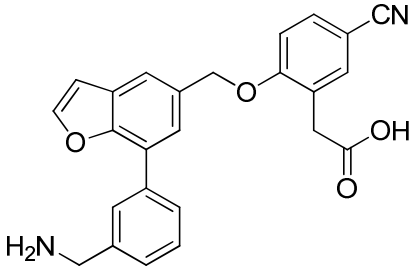
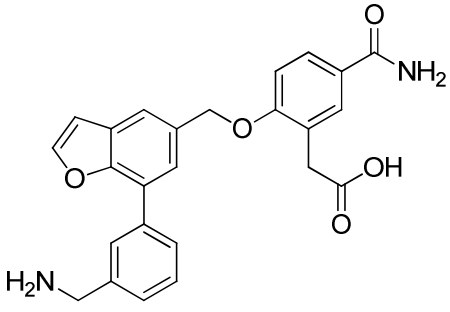
	
	
	
	
	

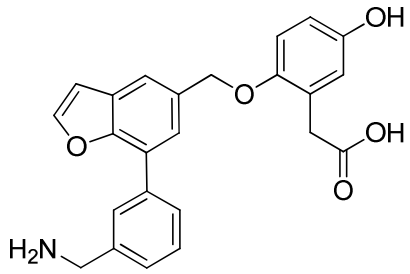
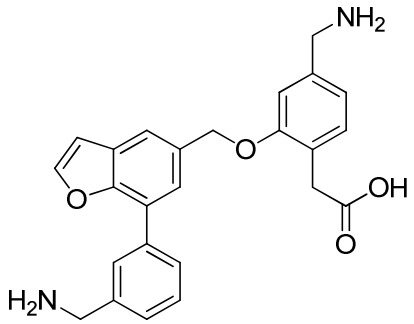
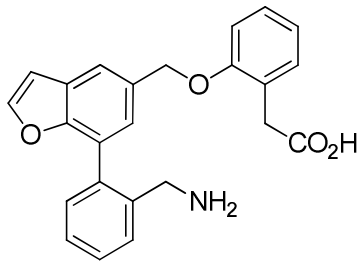
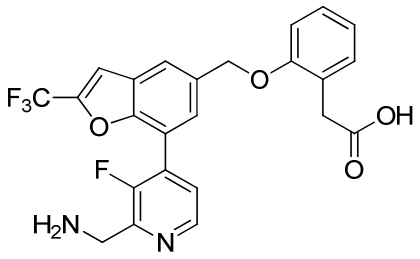
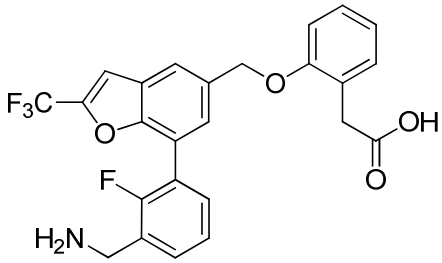
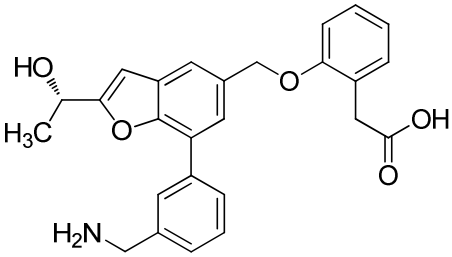
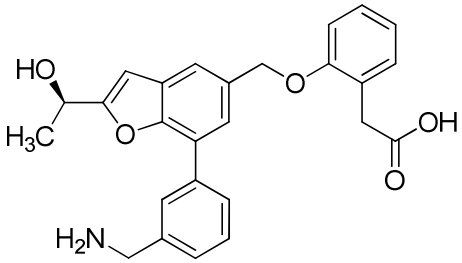
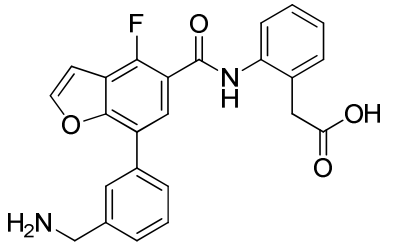
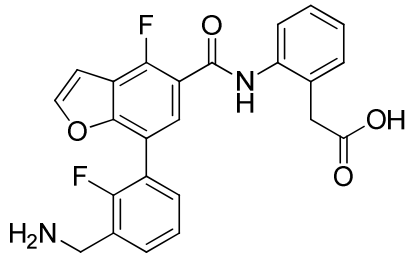
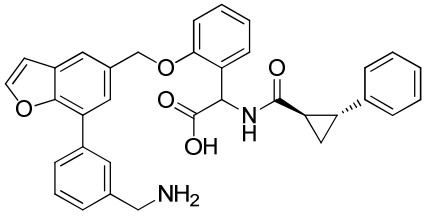
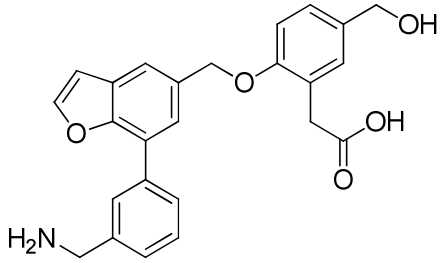
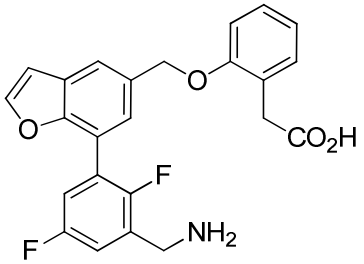
	
	
	
	
	

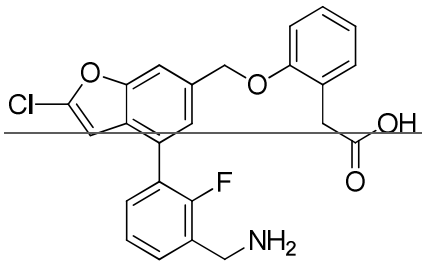
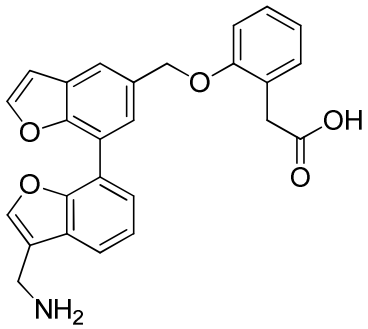
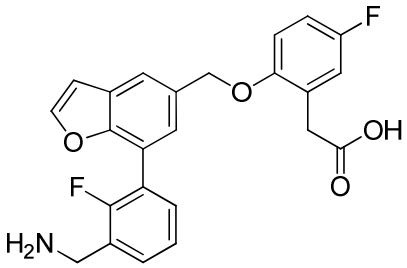
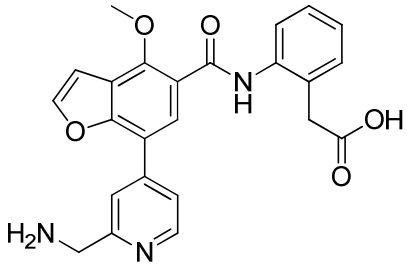
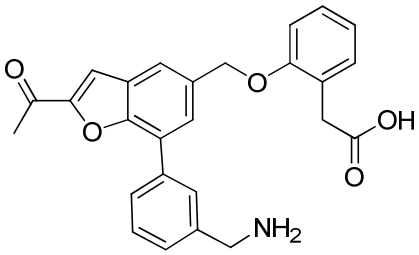
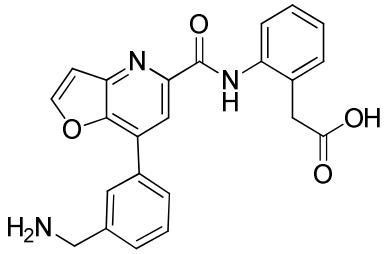
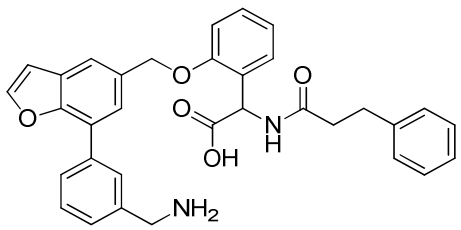
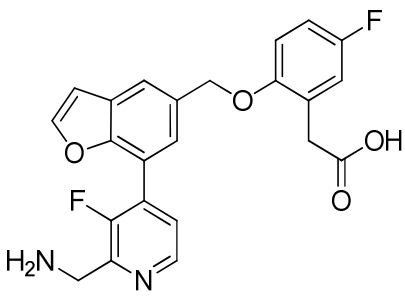
	
	
	
	
	

, or a pharmaceutically acceptable salt ~~or prodrug~~ thereof, for use in therapy.