CLAIMS

1. A compound selected from:

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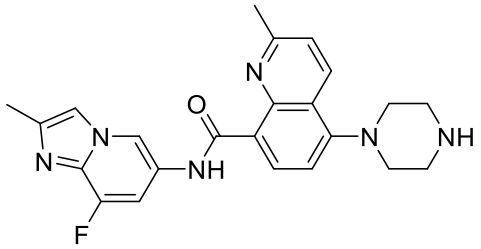
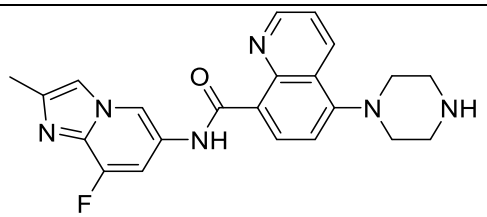
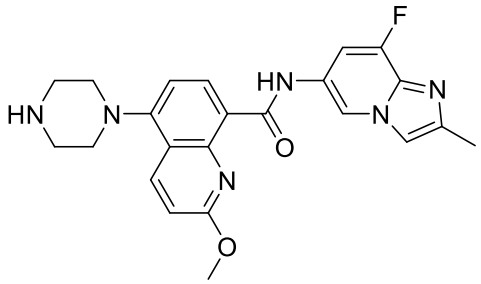
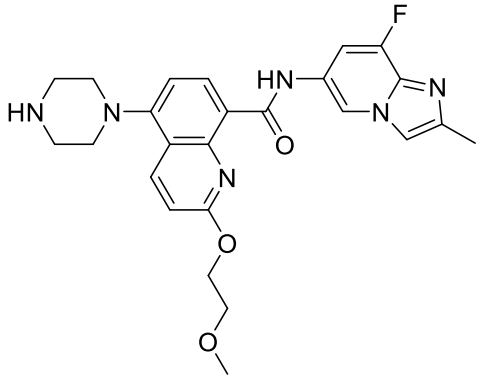
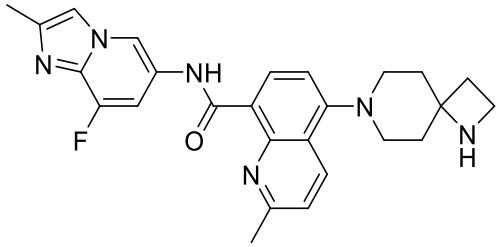
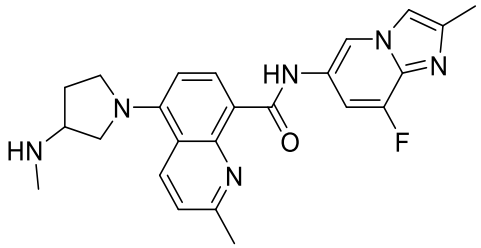
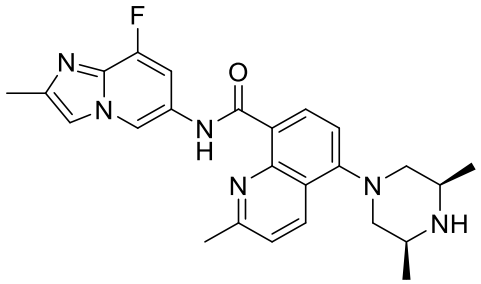
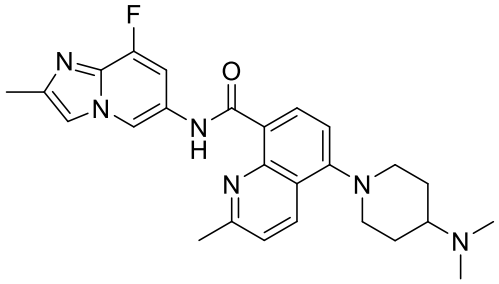
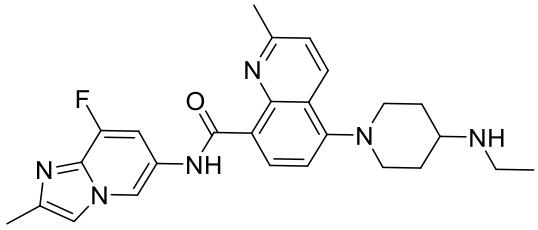
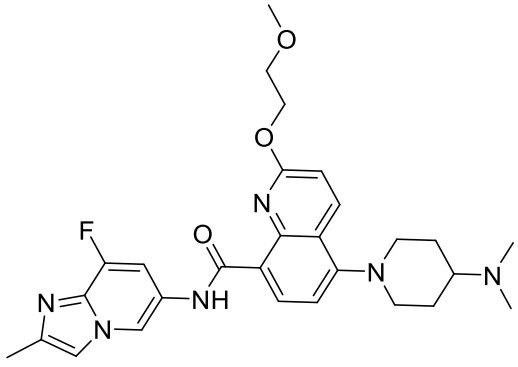
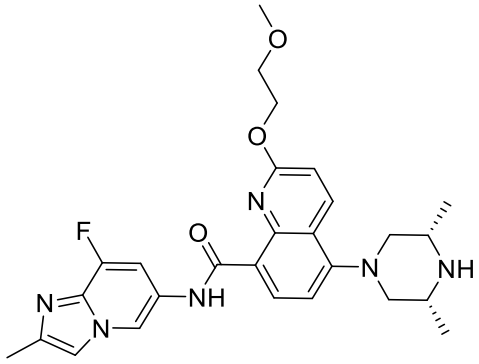
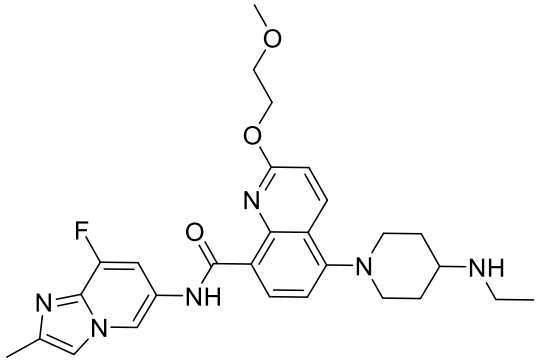
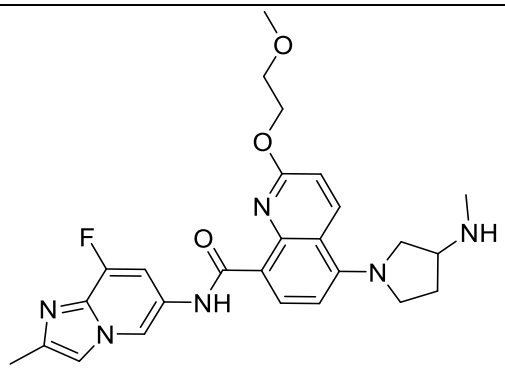
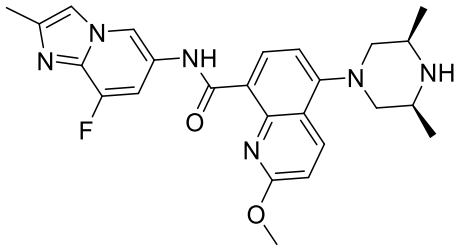
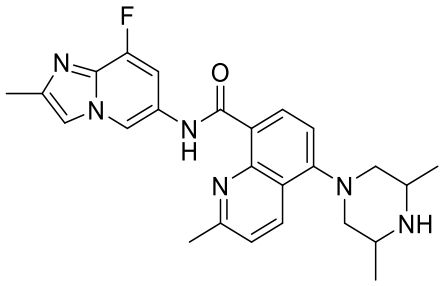
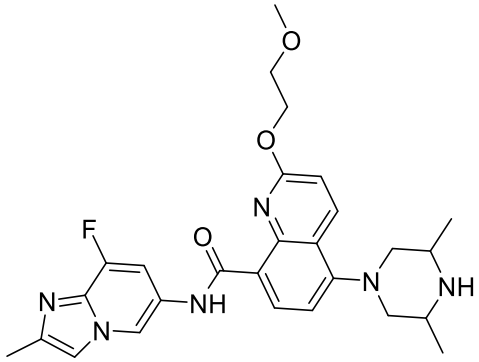
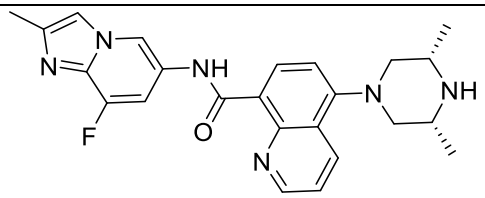
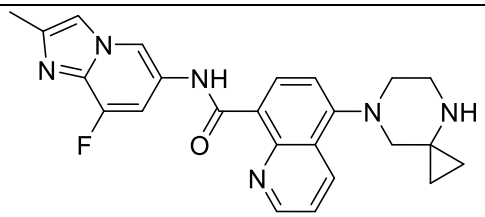
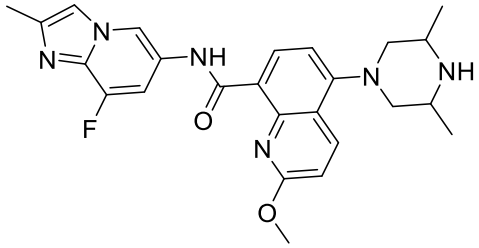
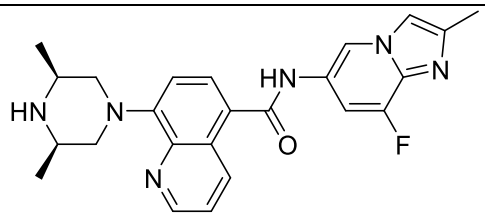
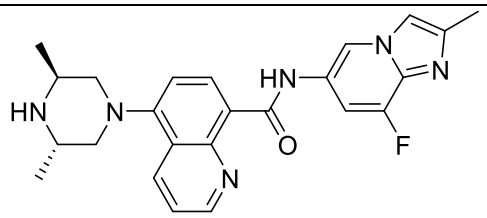
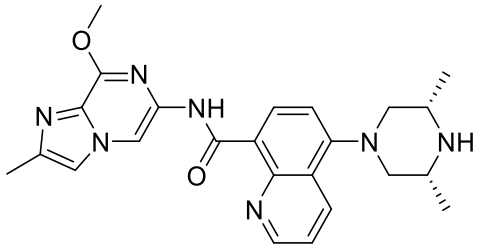
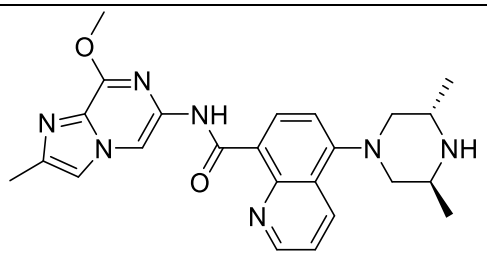
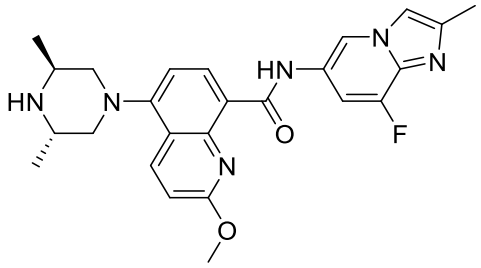
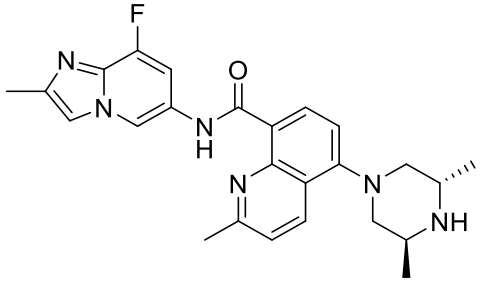
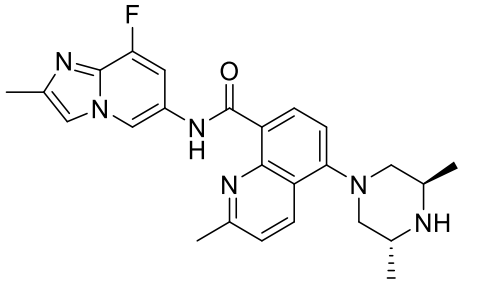
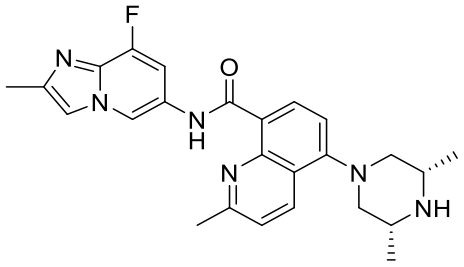
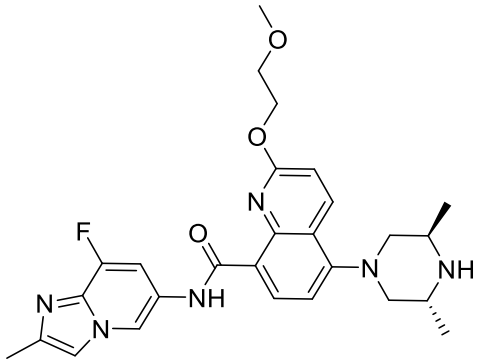
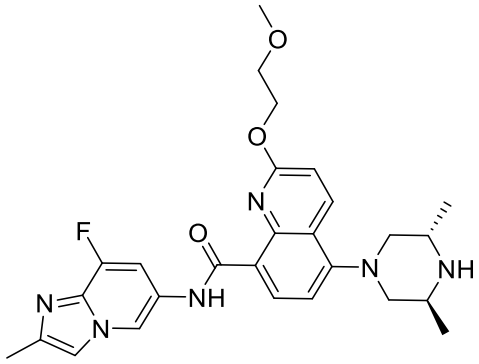
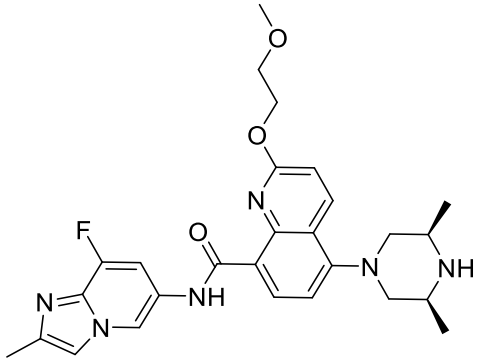
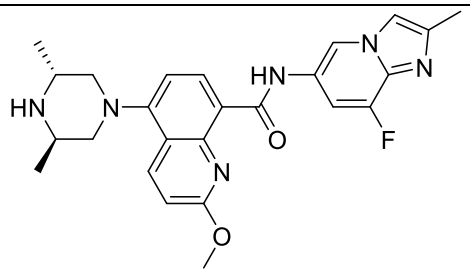
162	
165	
169	
173	
195	

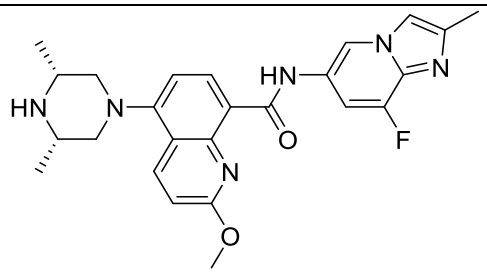
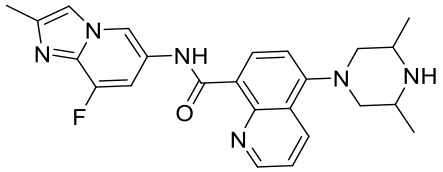
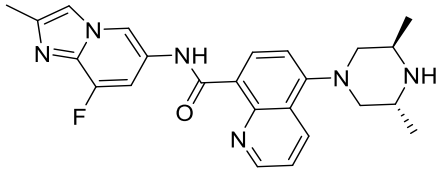
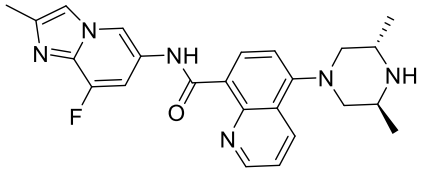
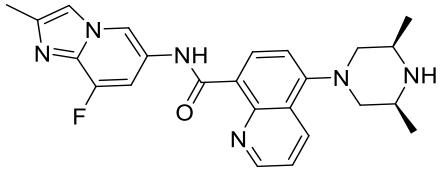
196	
214	
215	
228	
229	

232	
233	
234	
268	

328	
333	
340	
341	
346	
358	

382	
383	
384	
385	
403	
404	

405	
417	
418	
419	
440	

441	
513	
514	
515	
516	

or a pharmaceutically acceptable salt thereof.

2. A compound selected from:

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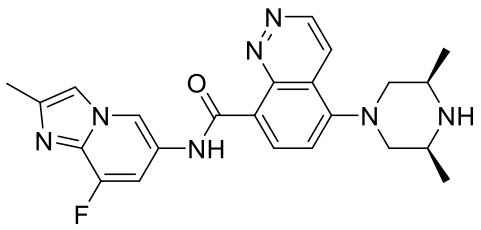
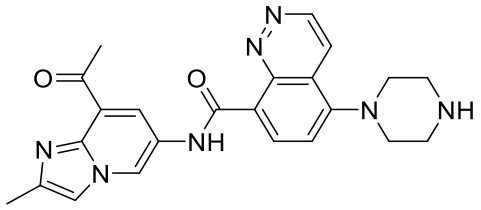
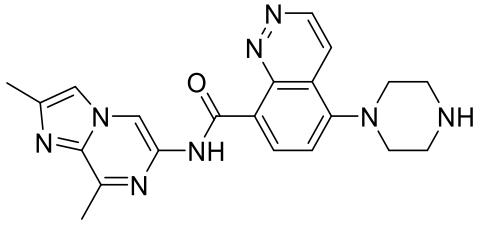
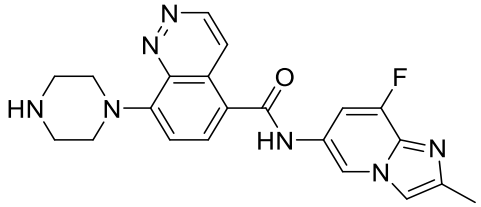
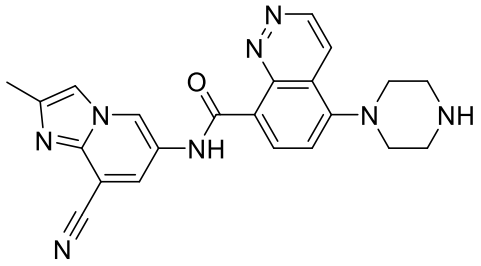
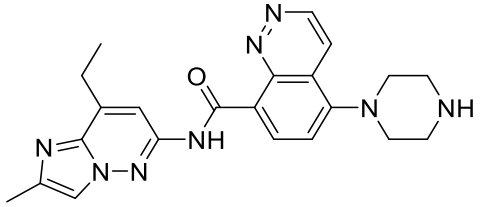
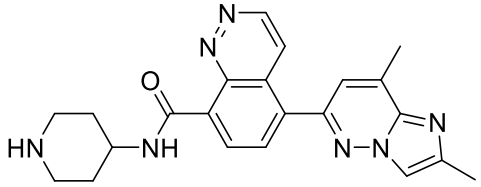
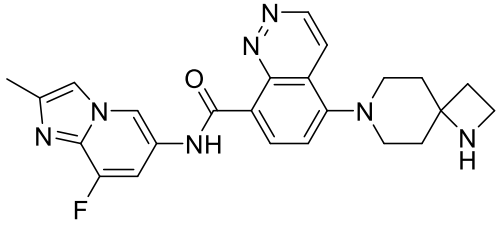
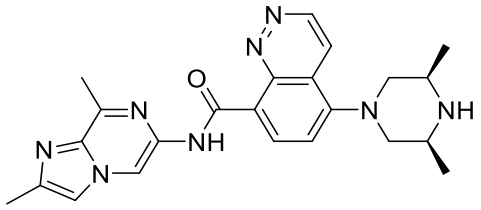
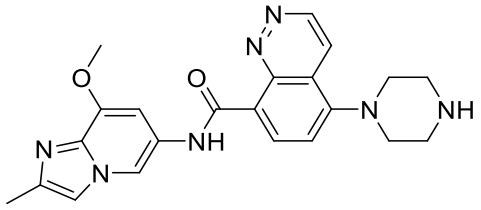
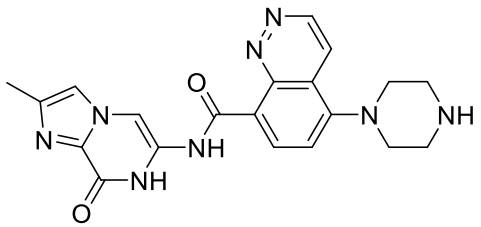
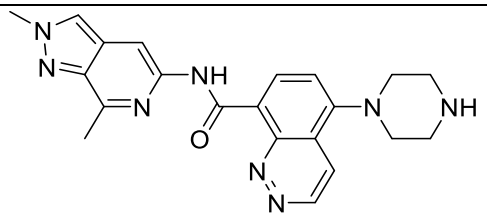
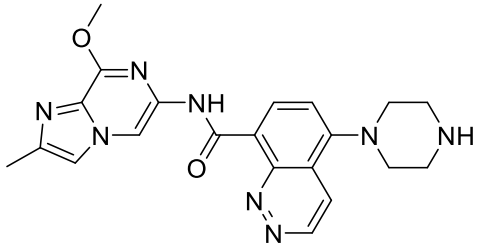
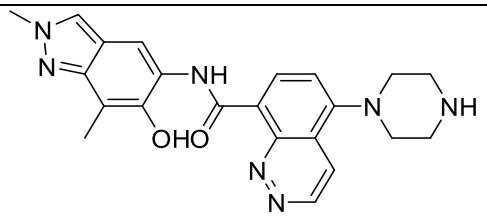
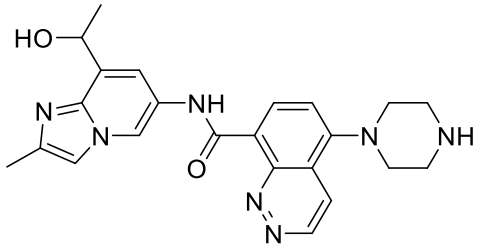
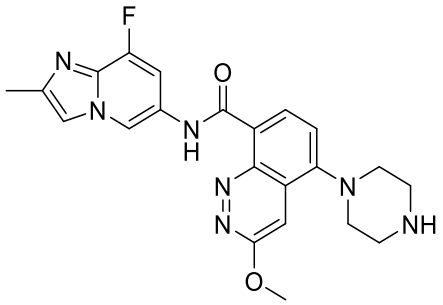
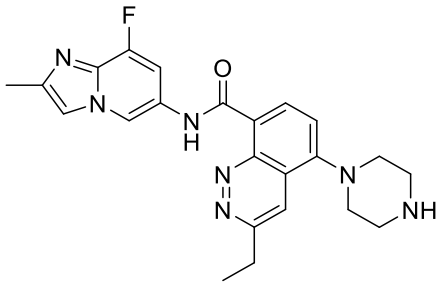
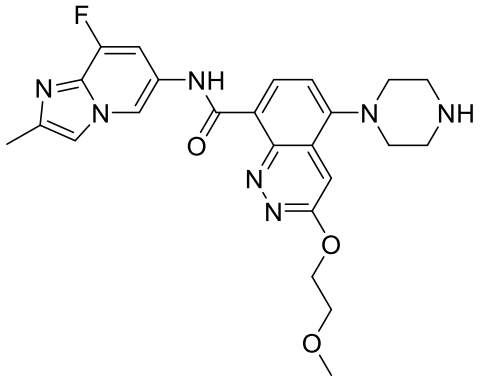
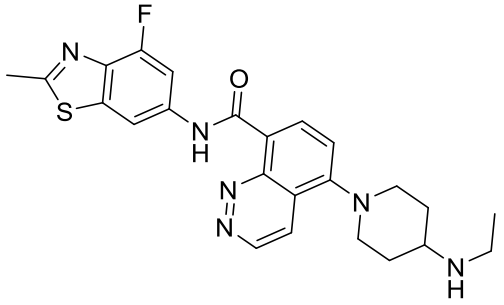
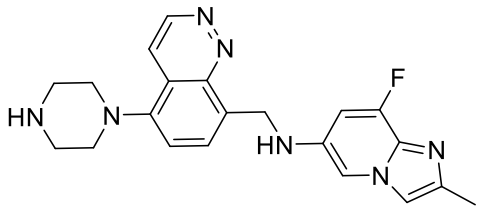
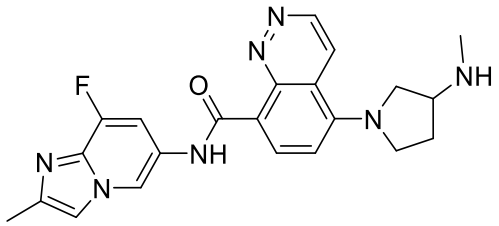
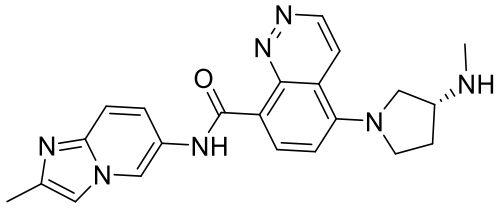
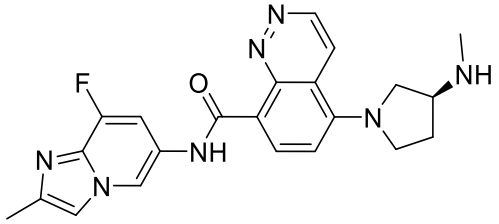
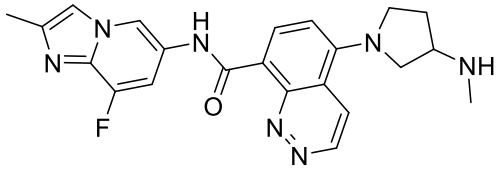
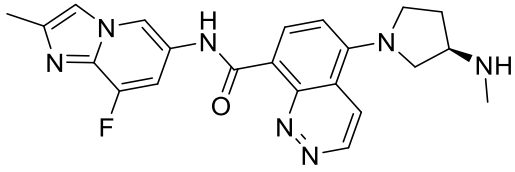
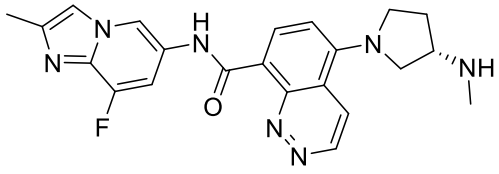
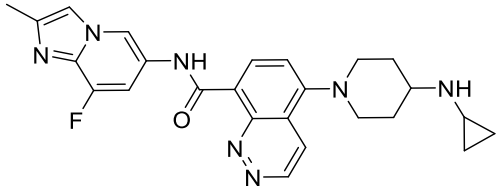
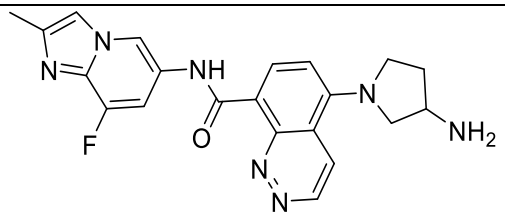
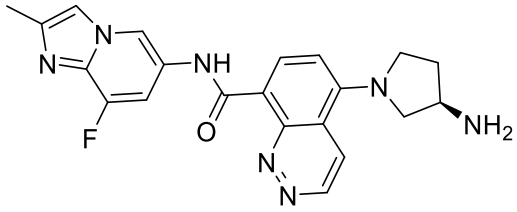
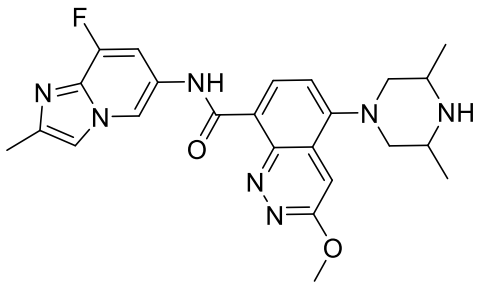
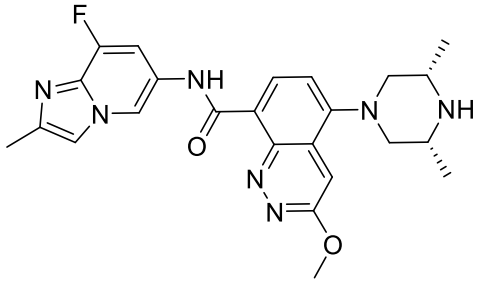
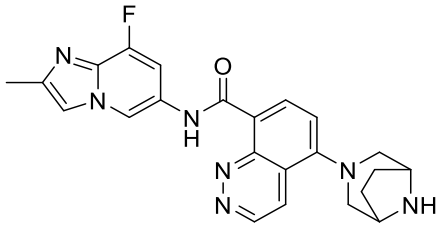
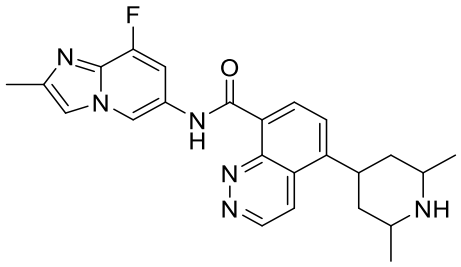
161	

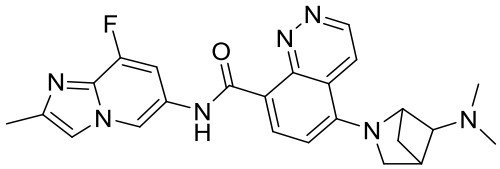
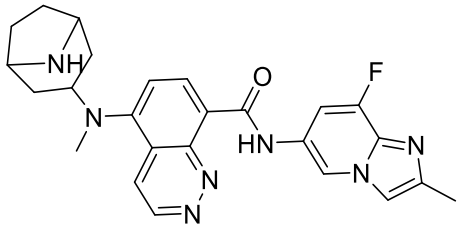
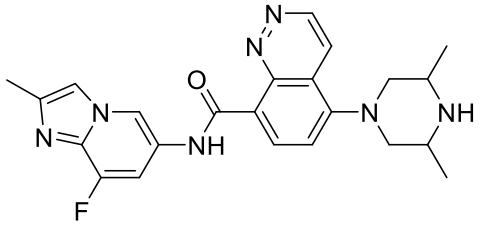
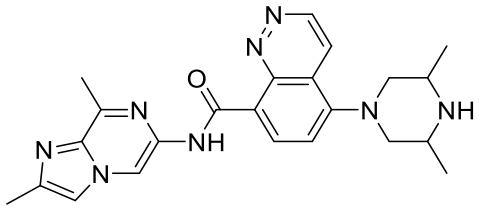
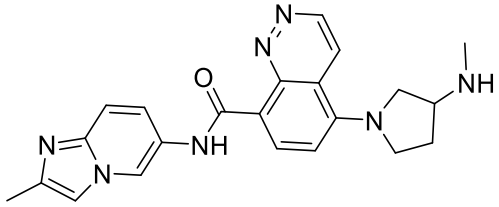
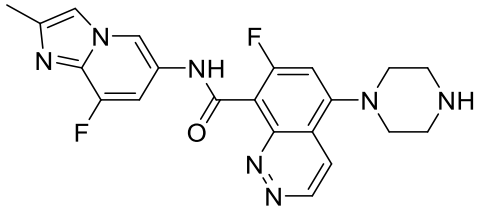
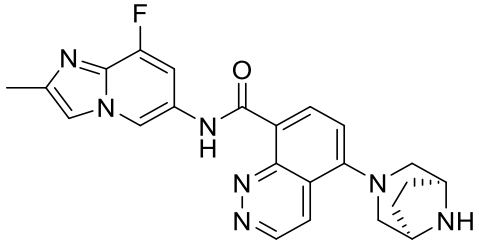
163	
164	
166	
167	
168	
170	
171	

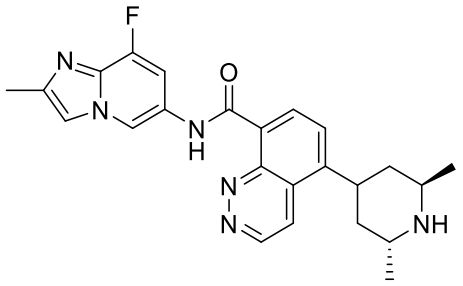
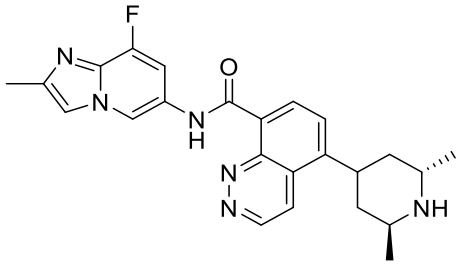
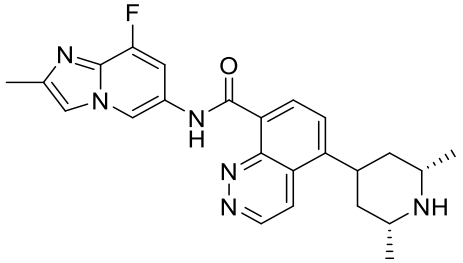
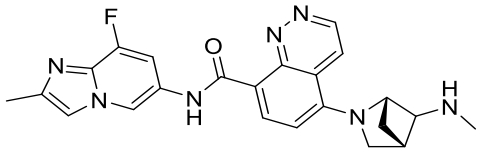
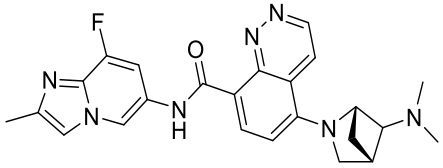
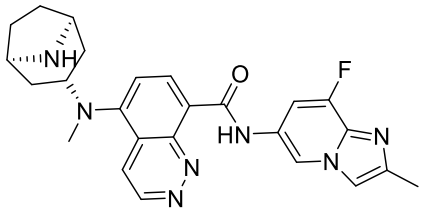
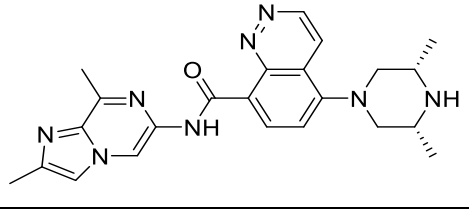
172	
176	
177	
178	
179	
181	
182	

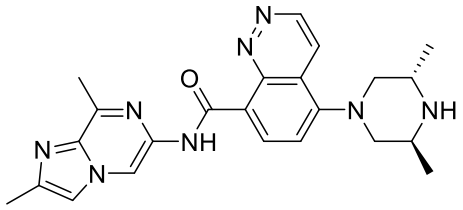
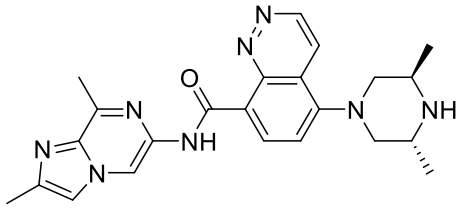
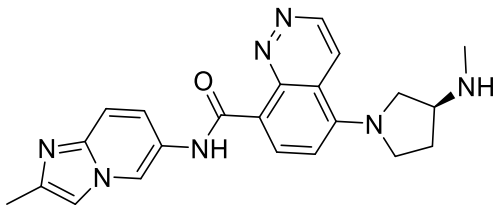
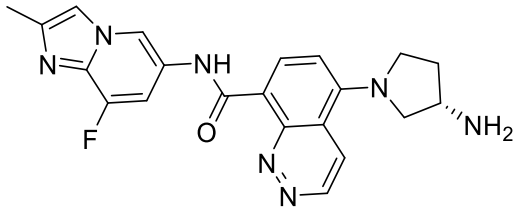
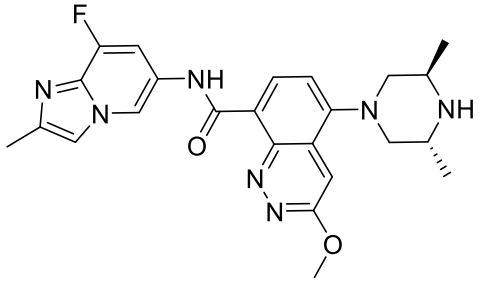
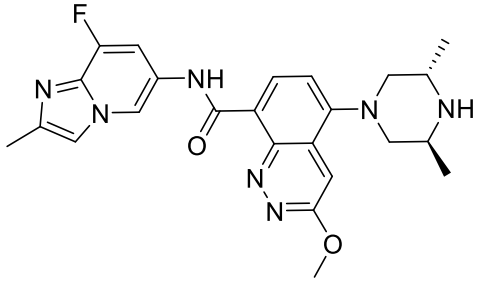
183	
185	
199	
221	
230	

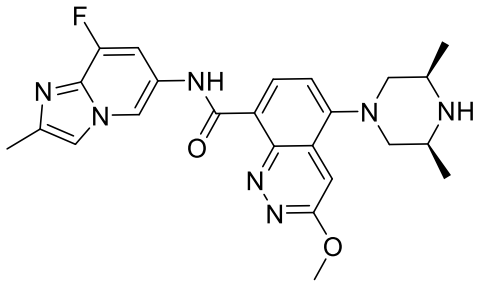
231	
235	
236	
237	
238	
239	
259	

269	
270	
271	
273	
319	
320	

321	
322	
323	
324	
334	
350	
388	

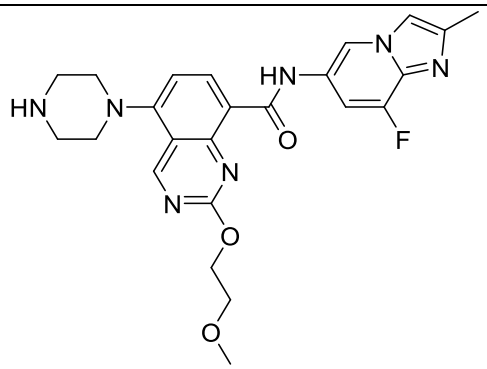
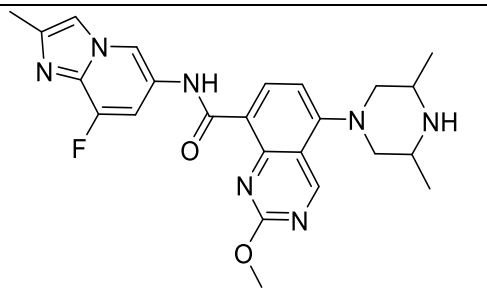
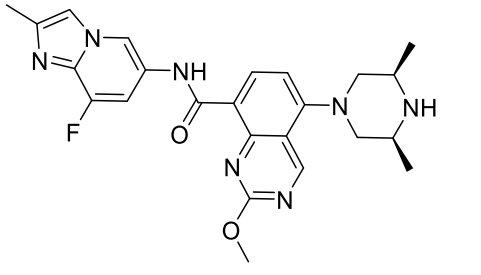
389	
390	
391	
392	
393	
394	
395	

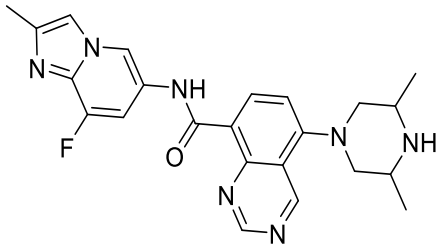
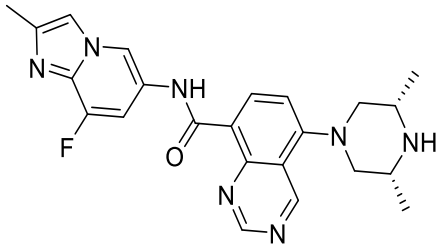
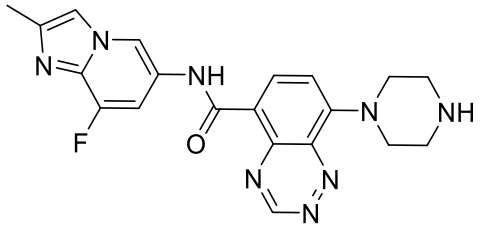
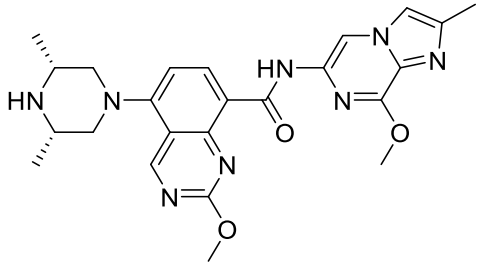
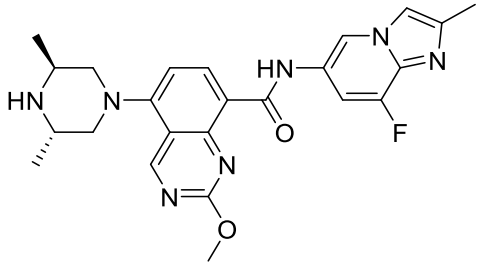
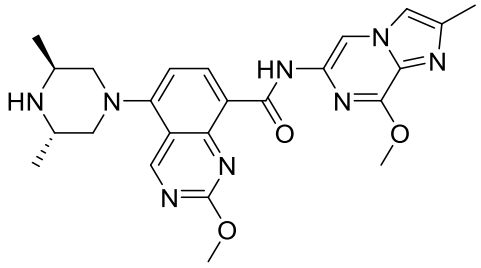
396	
397	
420	
442	
443	
444	

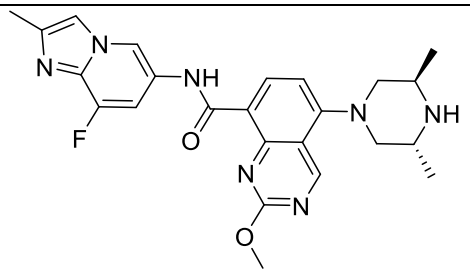
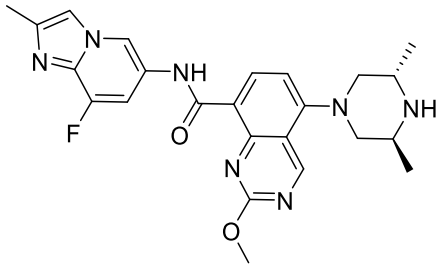
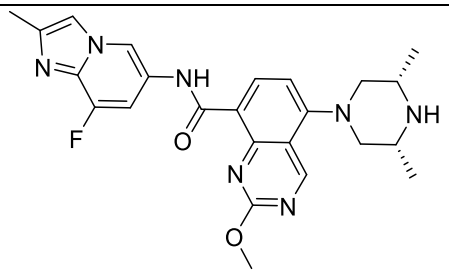
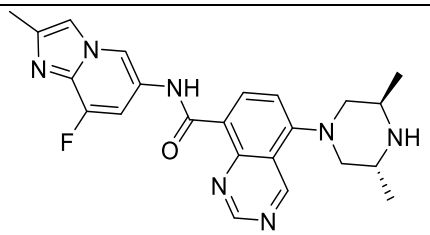
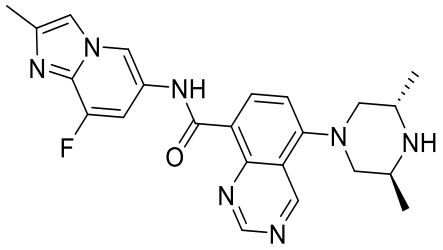
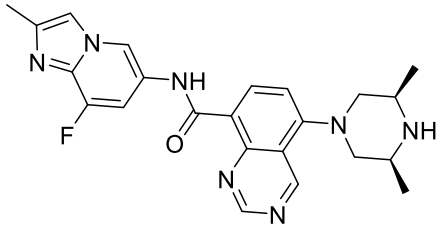
445	

or a pharmaceutically acceptable salt thereof.

3. A compound selected from:

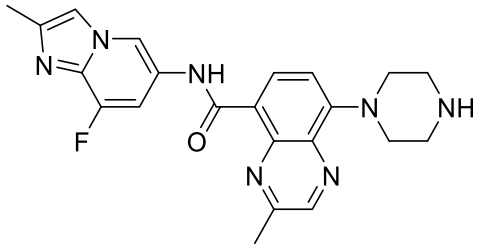
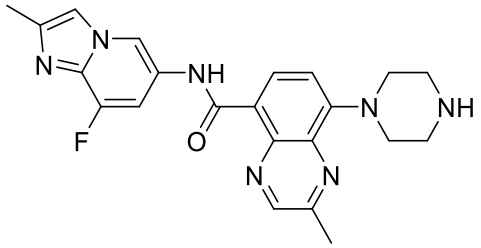
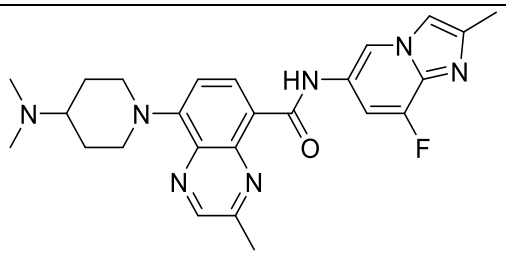
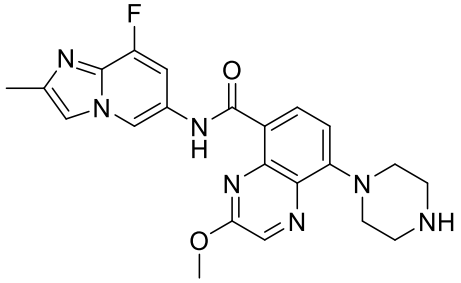
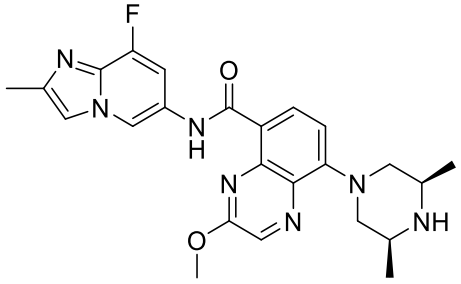
Cmpd No.	**Structure**
180	
299	
300	

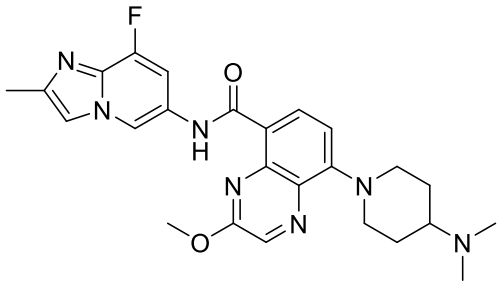
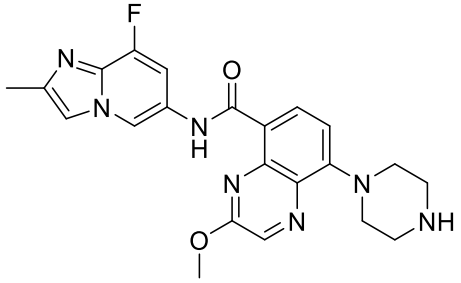
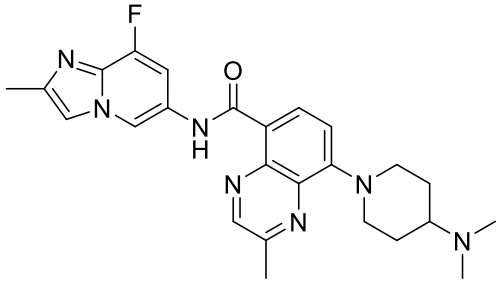
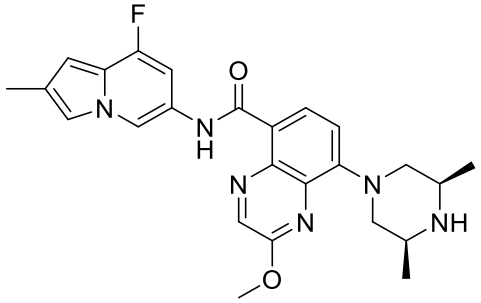
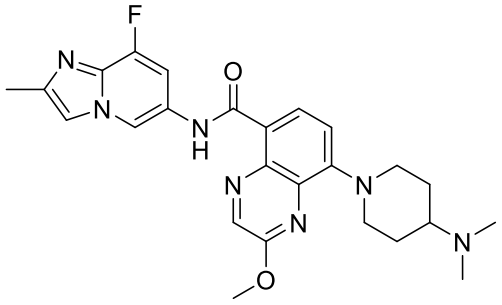
301	
302	
345	
378	
386	
387	

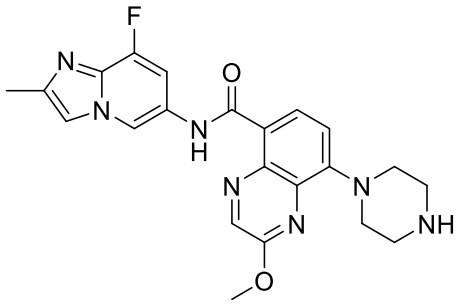
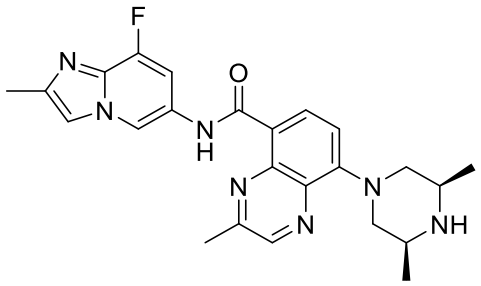
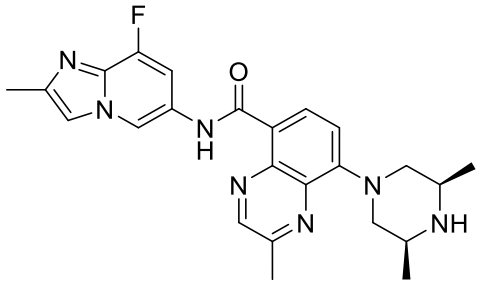
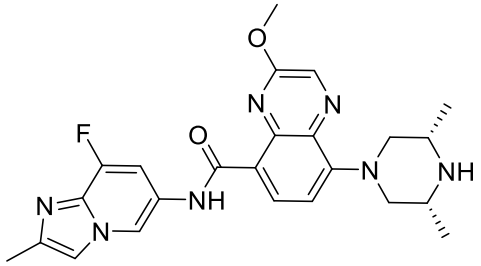
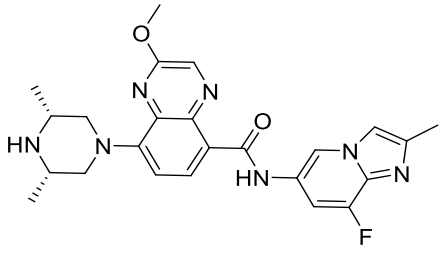
487	
488	
489	
490	
491	
492	

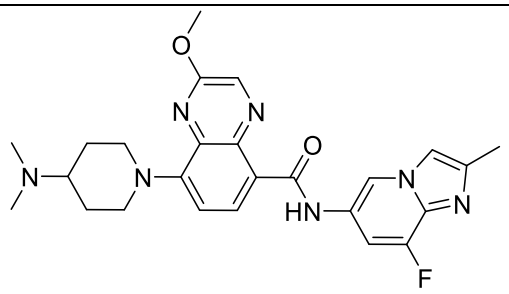
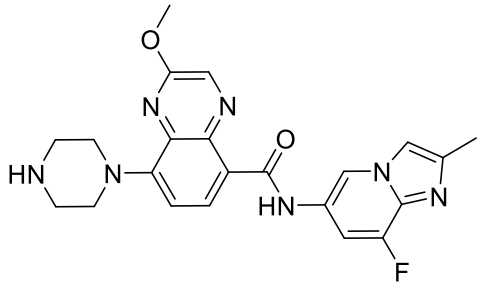
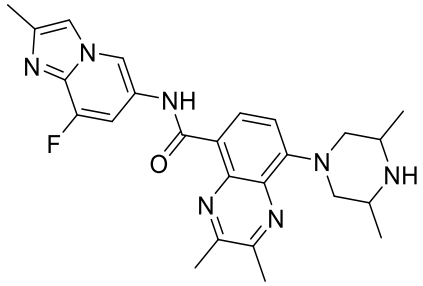
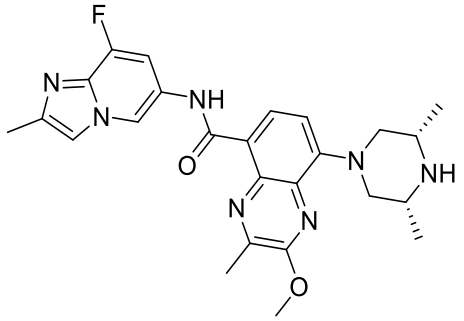
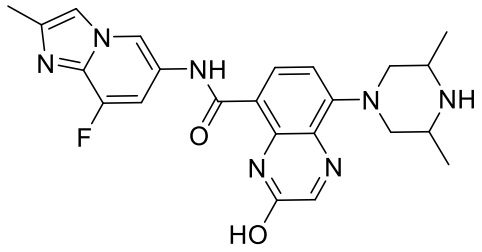
or a pharmaceutically acceptable salt thereof.

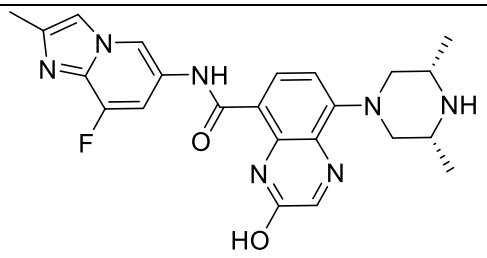
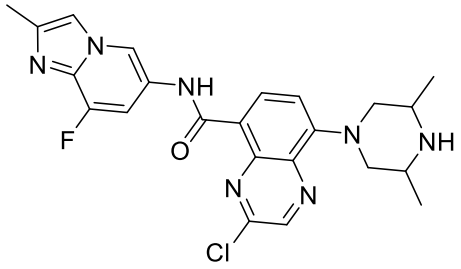
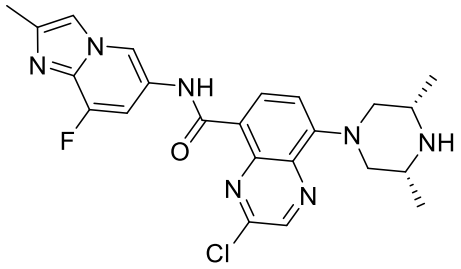
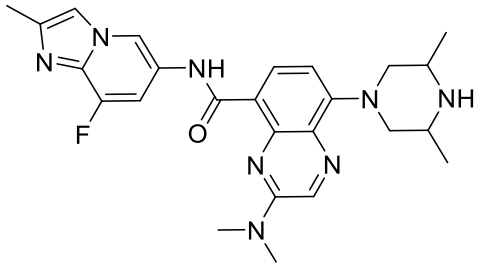
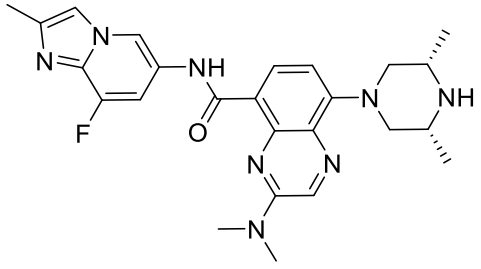
4. A compound selected from:

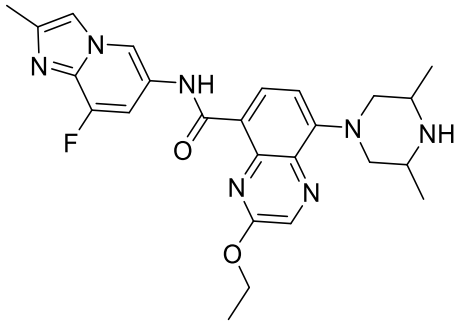
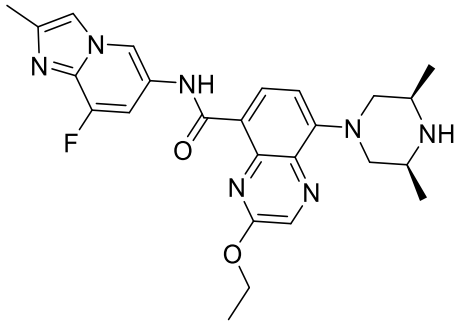
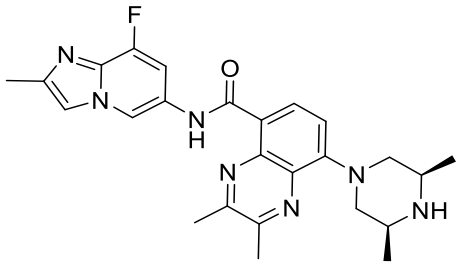
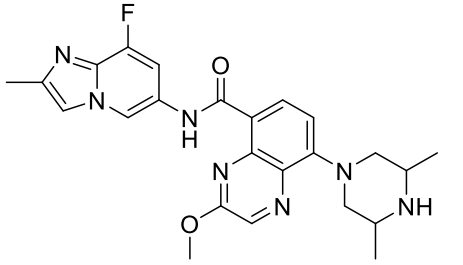
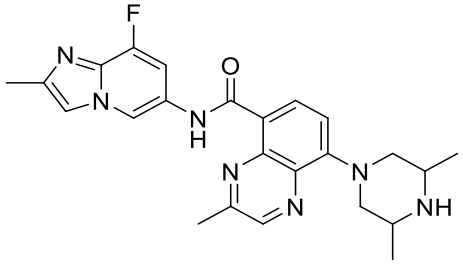
Cmpd No.	Structure
197	
198	
200	
201	
205	

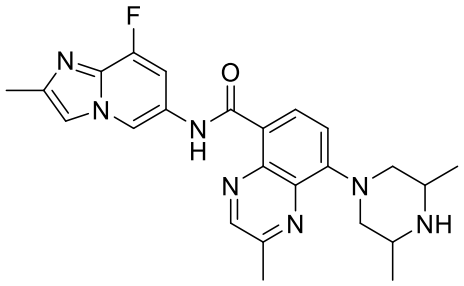
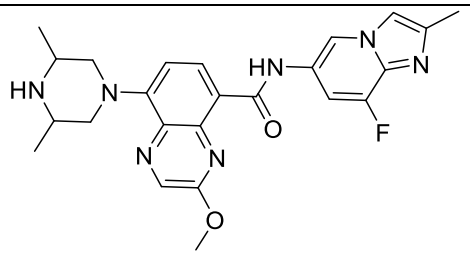
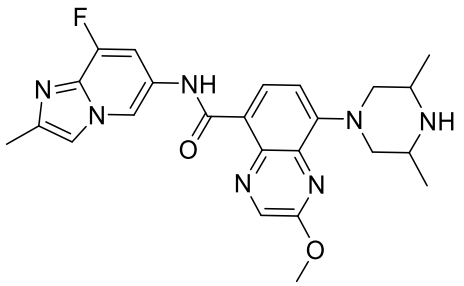
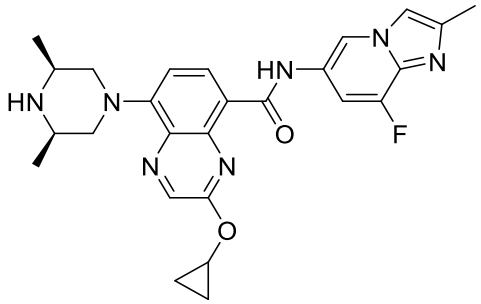
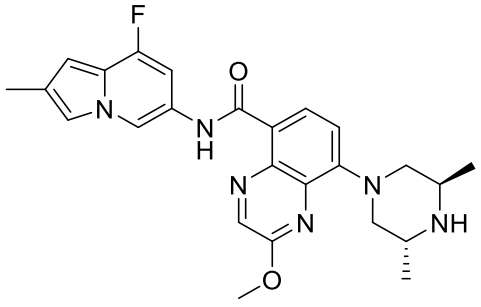
206	
207	
208	
209	
210	

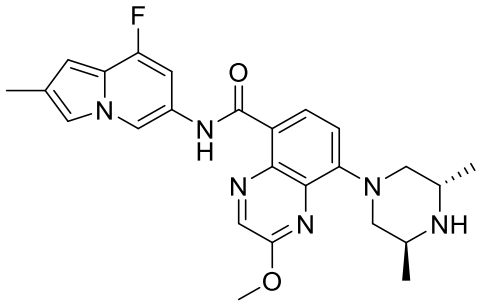
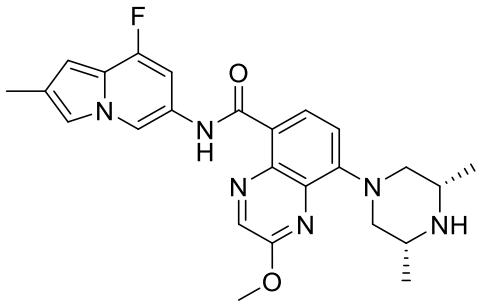
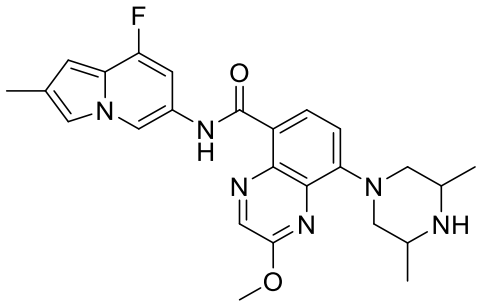
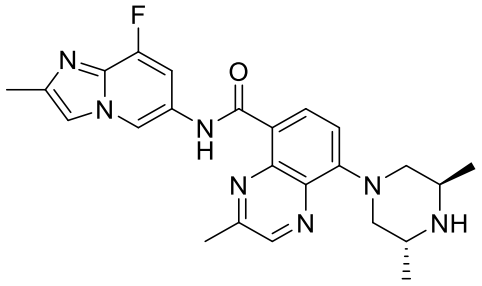
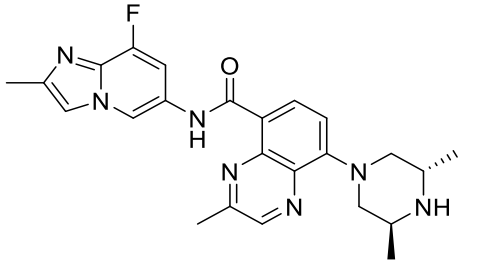
211	
216	
217	
222	
223	

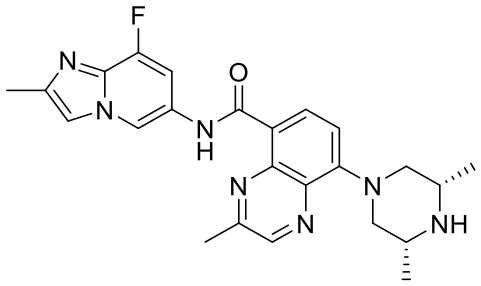
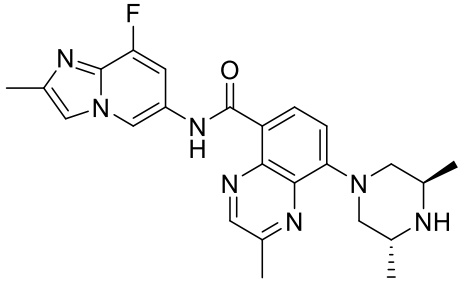
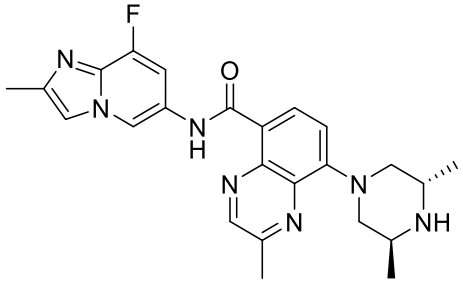
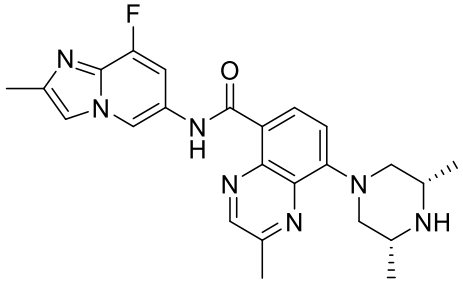
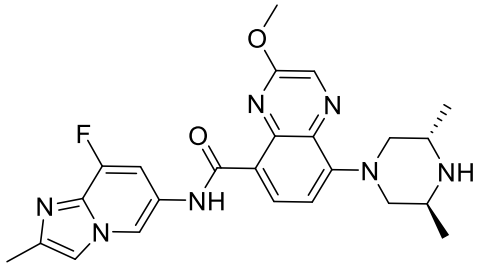
224	
225	
257	
260	
274	

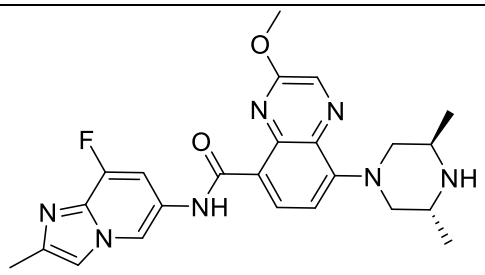
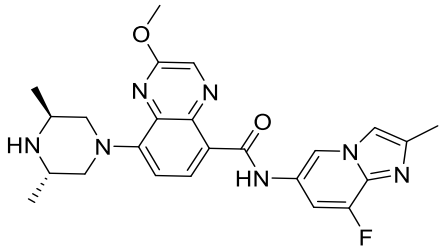
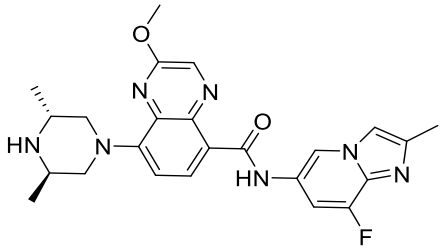
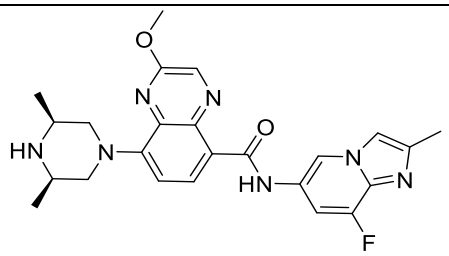
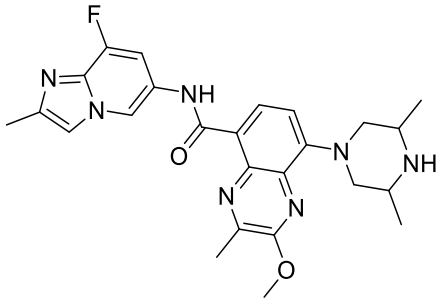
275	
280	
281	
282	
283	

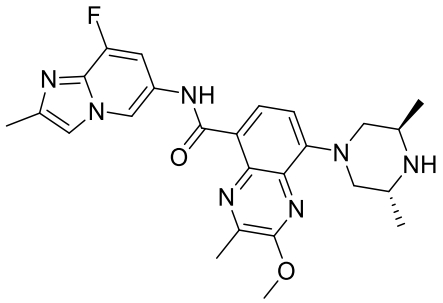
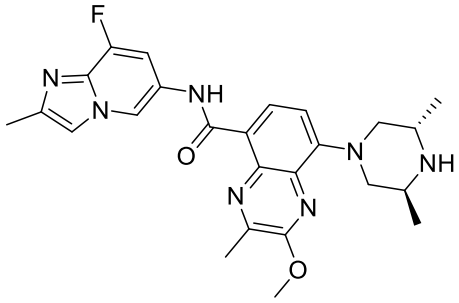
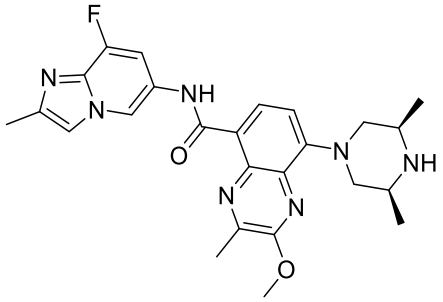
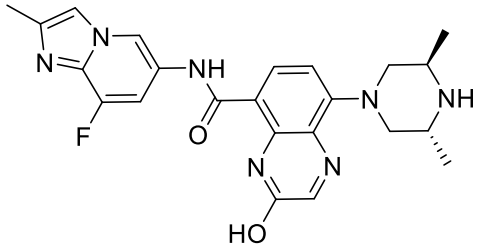
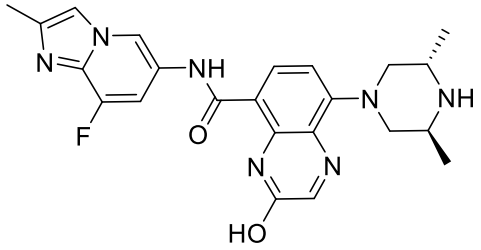
295	
296	
318	
326	
329	

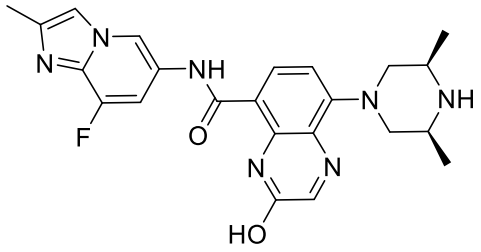
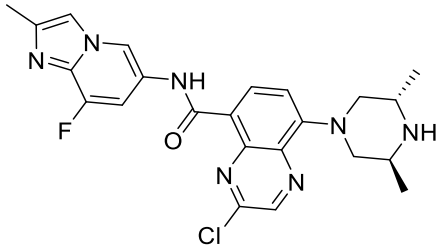
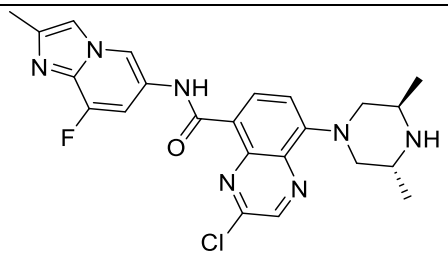
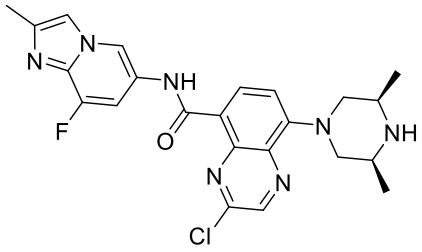
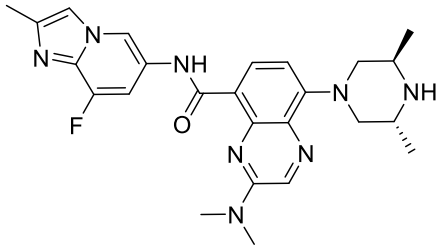
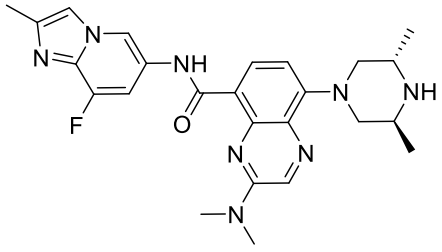
330	
331	
332	
351	
399	

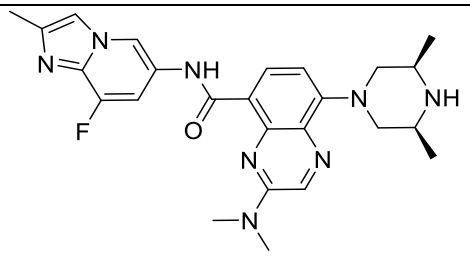
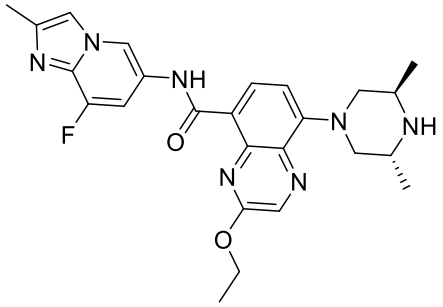
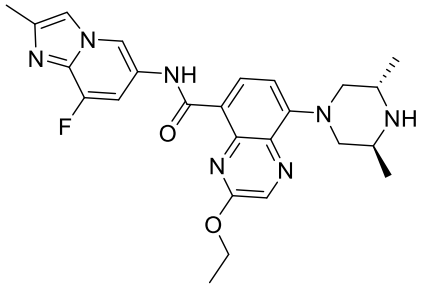
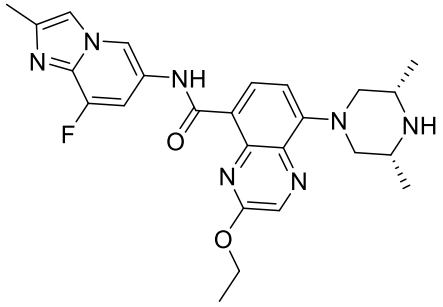
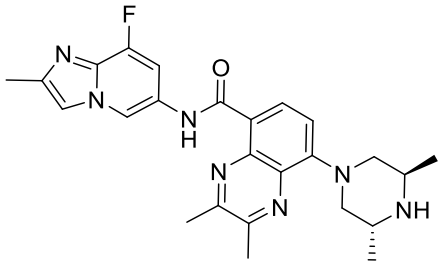
400	
401	
402	
406	
407	

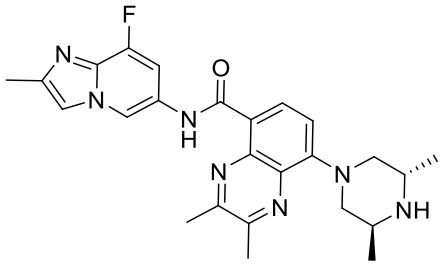
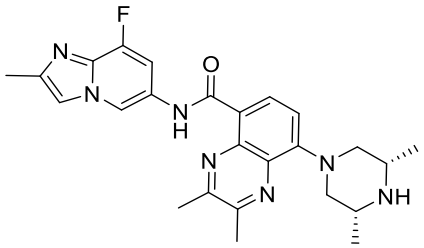
408	
409	
410	
411	
412	

413	
414	
415	
416	
427	

428	
429	
430	
446	
447	

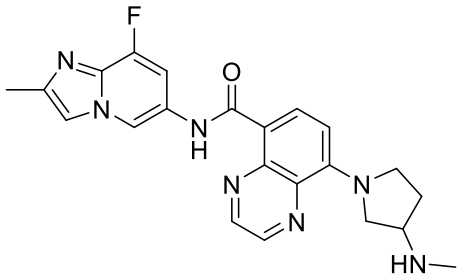
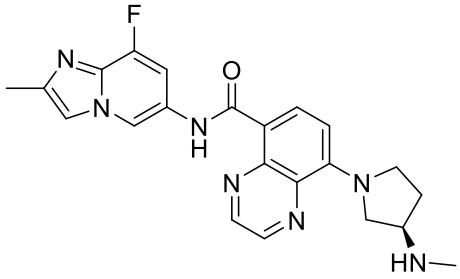
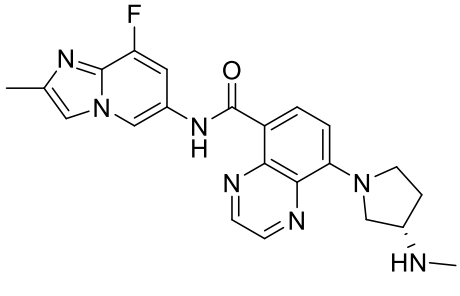
448	
455	
456	
457	
458	
459	

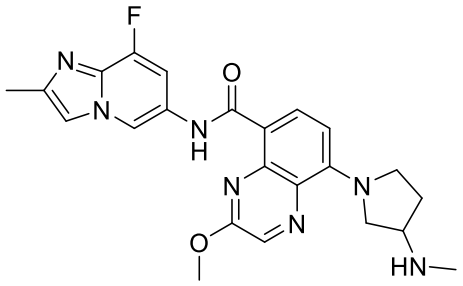
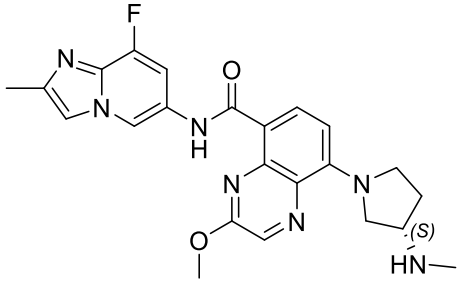
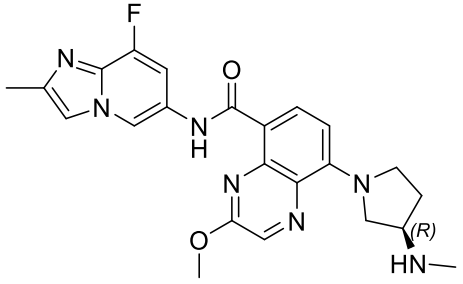
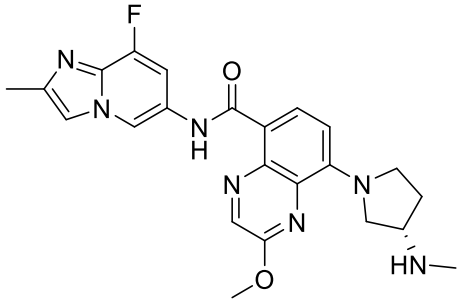
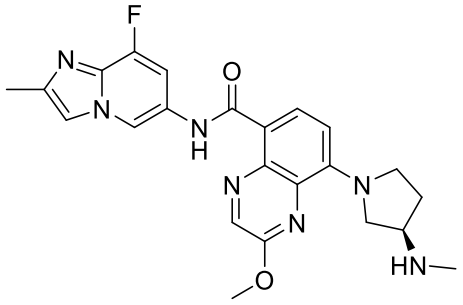
460	
481	
482	
483	
506	

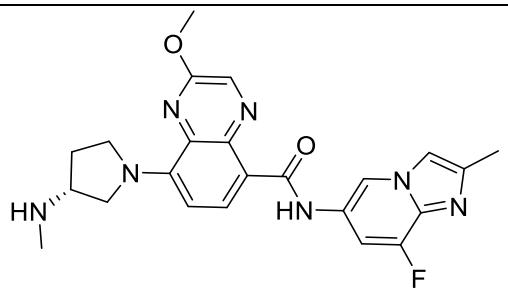
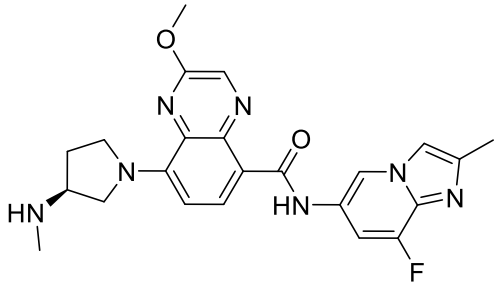
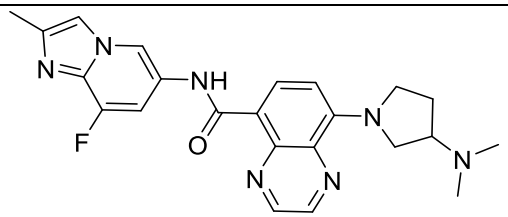
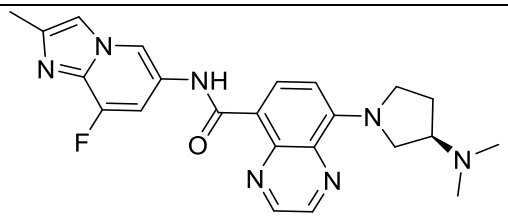
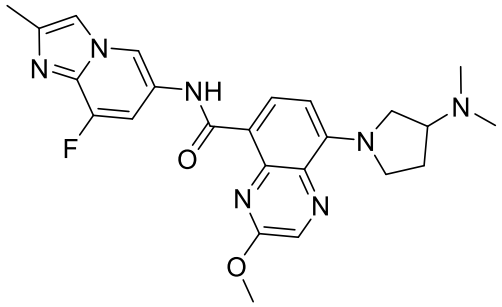
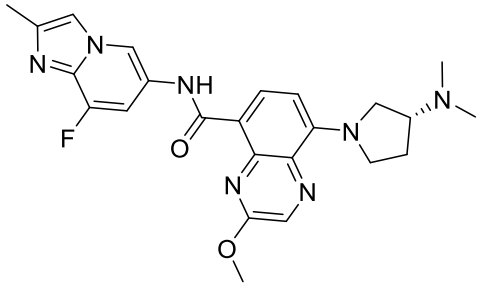
507	
508	

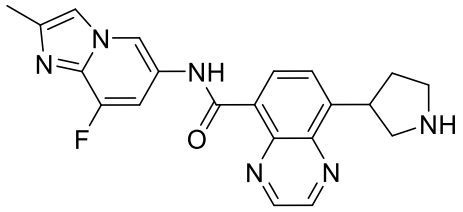
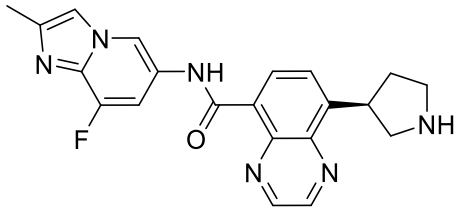
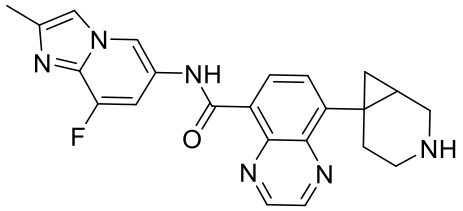
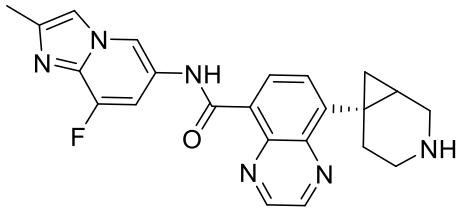
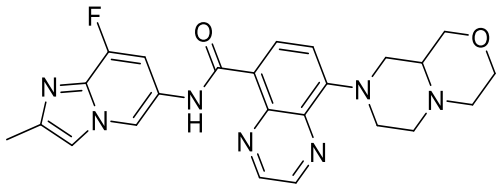
or a pharmaceutically acceptable salt thereof.

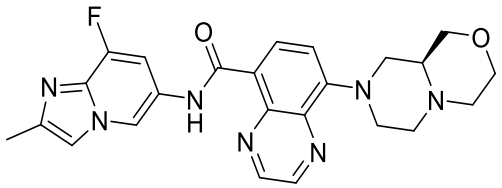
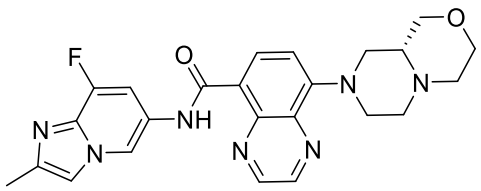
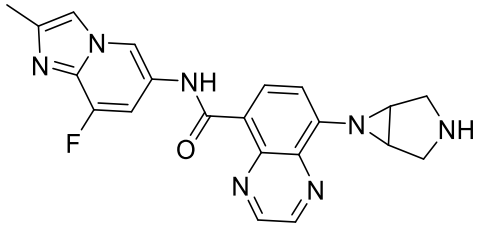
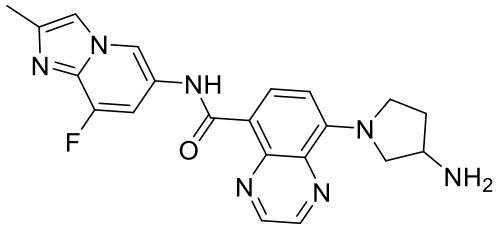
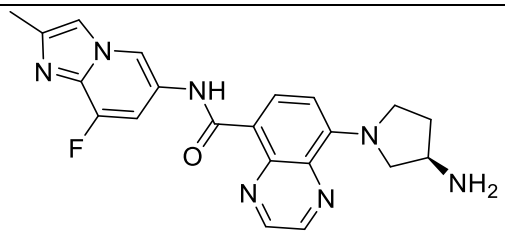
5. A compound selected from:

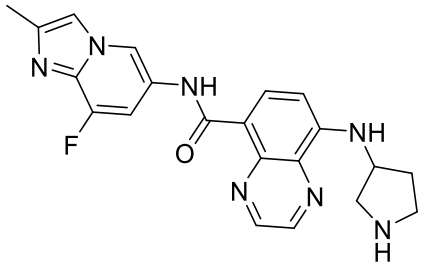
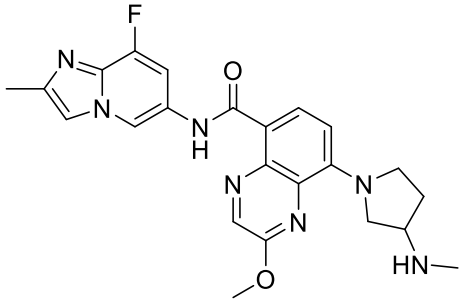
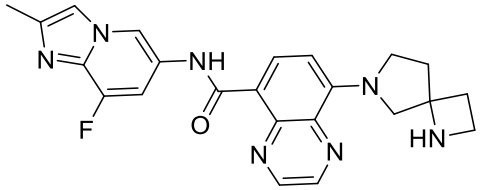
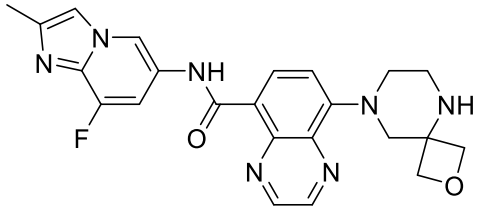
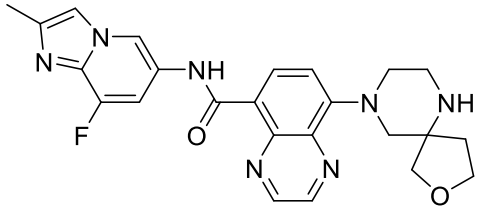
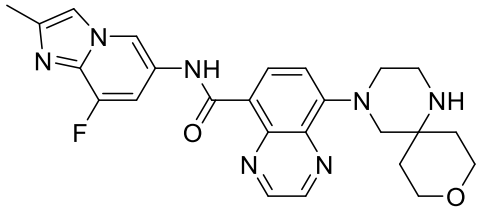
Cmpd No.	**Structure**
190	
193	
194	

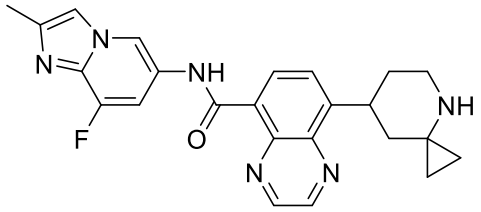
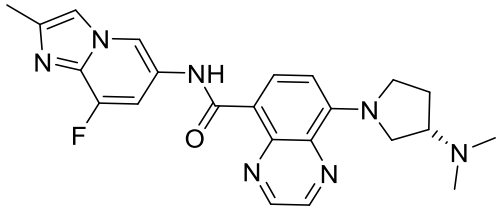
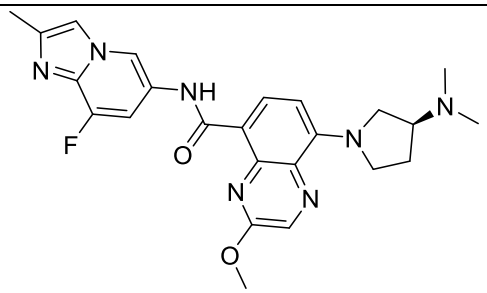
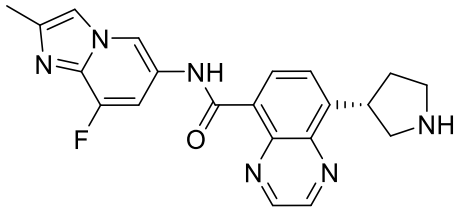
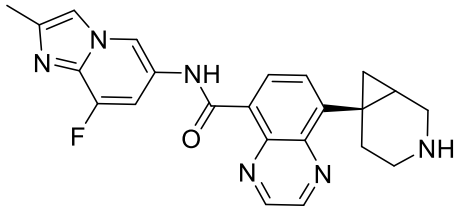
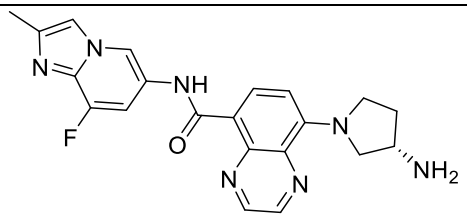
202	
203	
204	
212	
213	

226	
227	
241	
242	
243	
244	

245	
246	
247	
248	
254	

255	
256	
262	
314	
315	

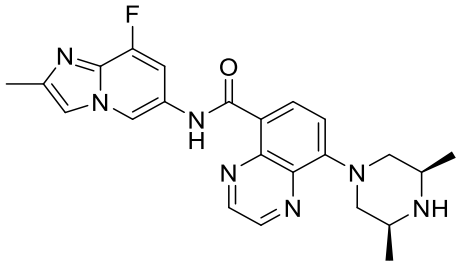
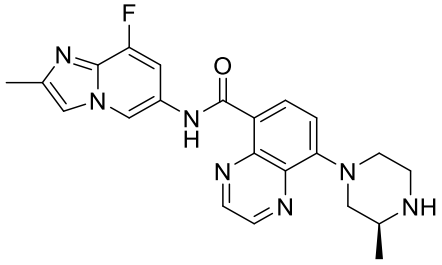
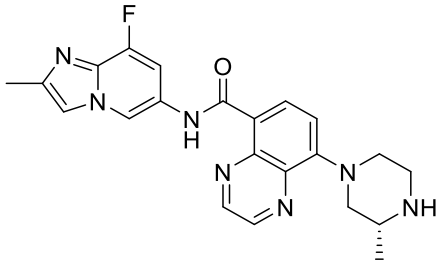
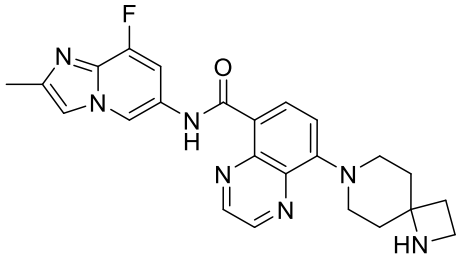
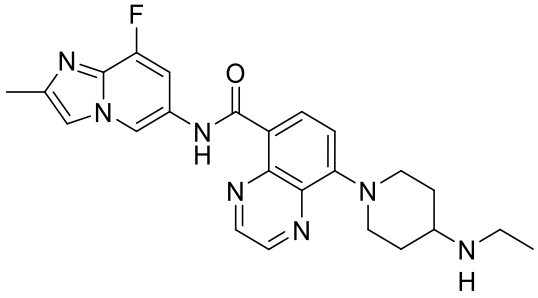
316	
327	
339	
372	
373	
374	

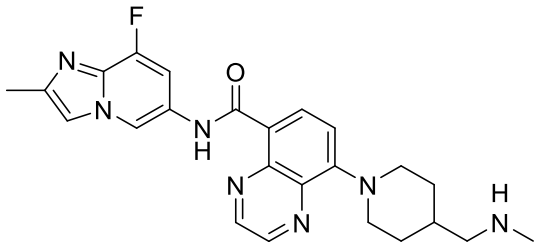
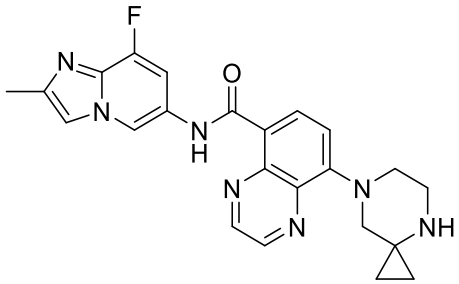
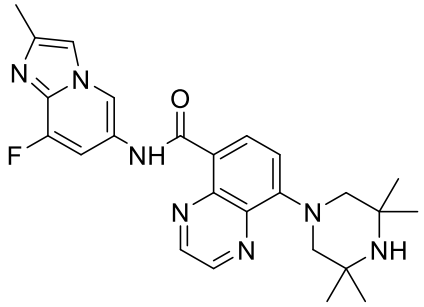
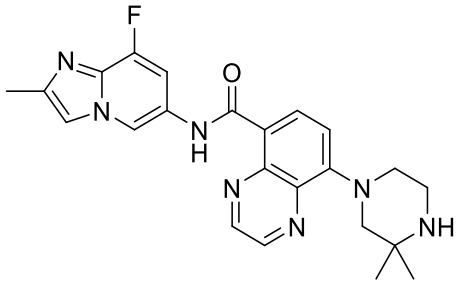
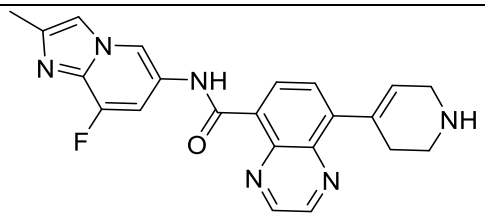
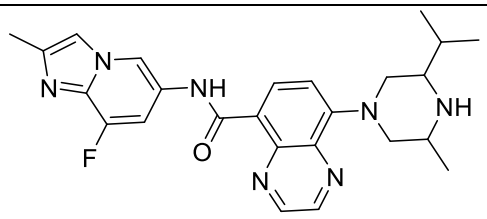
375	
421	
422	
423	
424	
505	

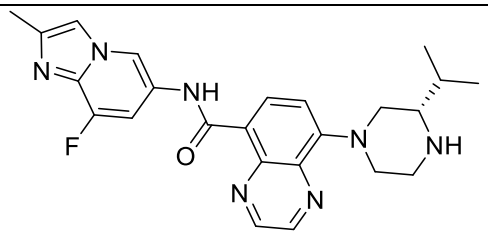
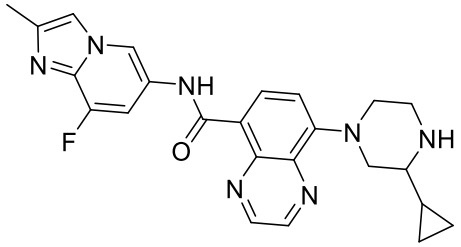
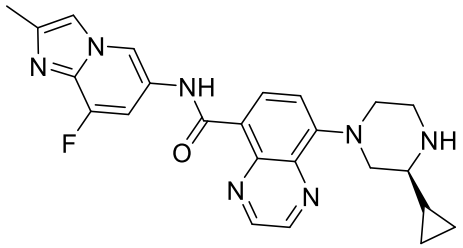
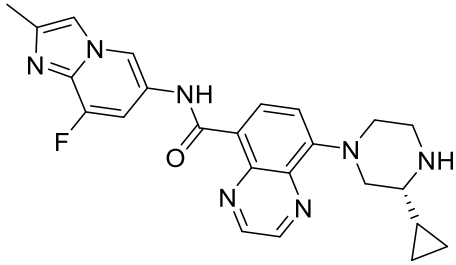
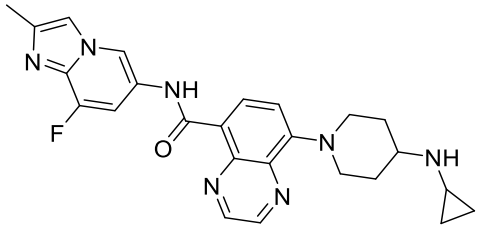
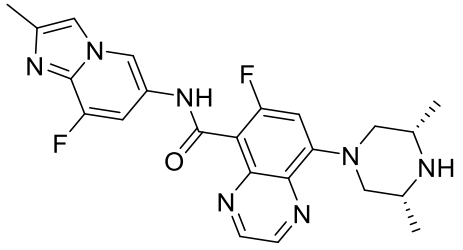
or a pharmaceutically acceptable salt thereof.

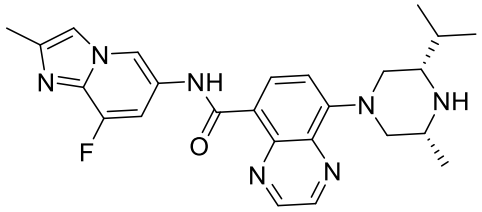
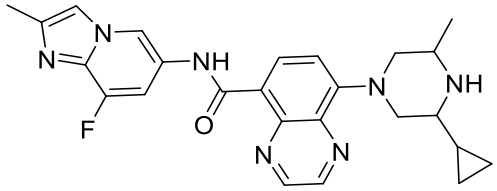
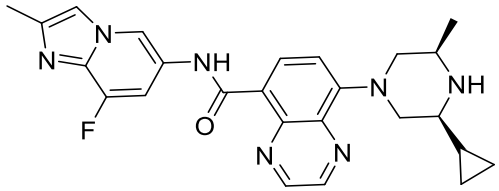
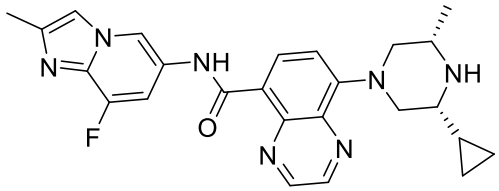
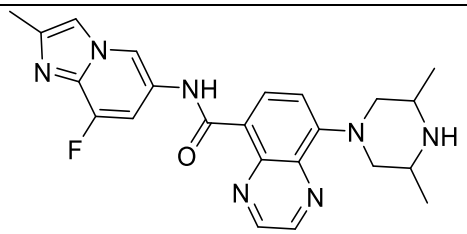
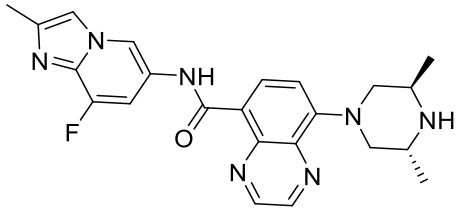
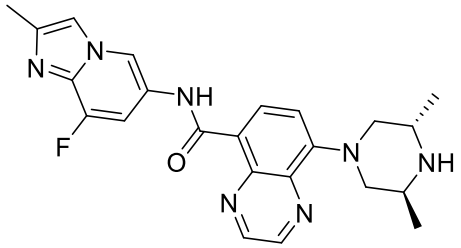
6. A compound selected from:

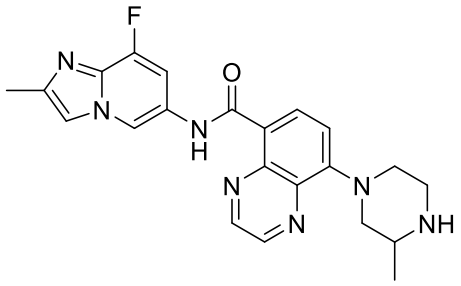
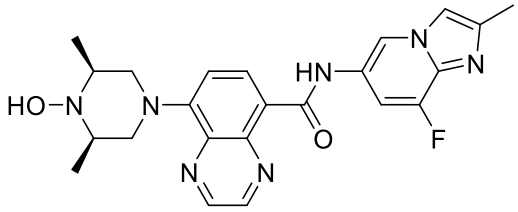
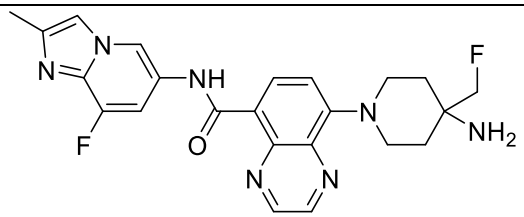
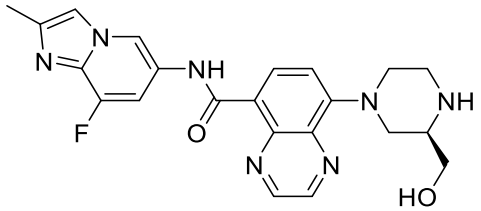
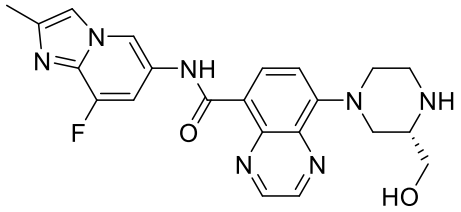
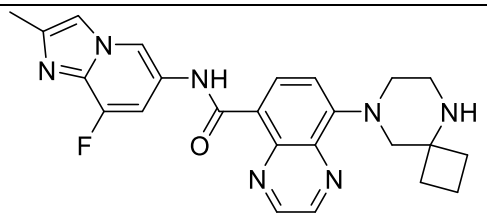
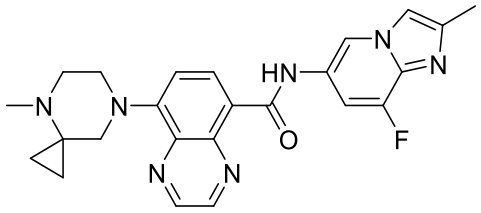
Cmpd No.	**Structure**

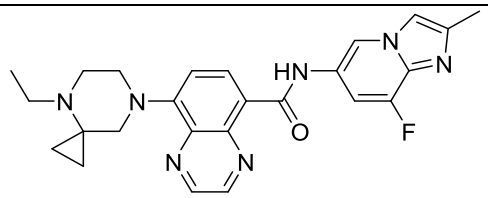
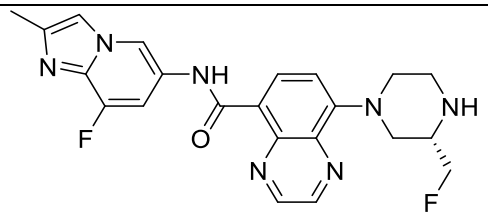
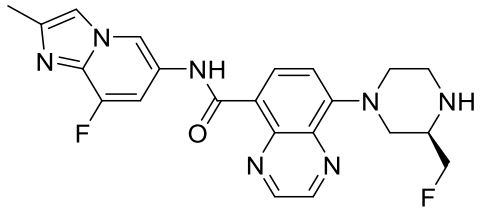
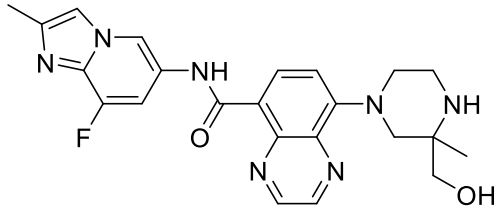
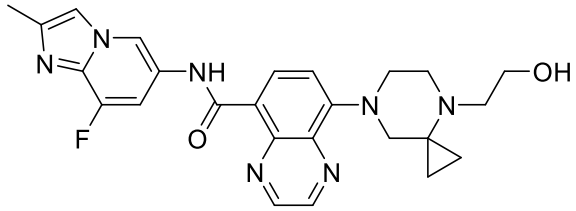
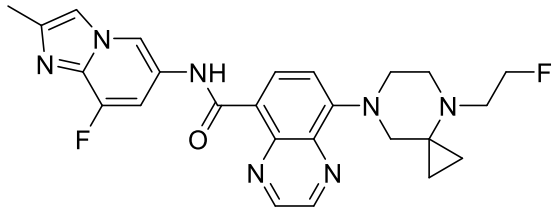
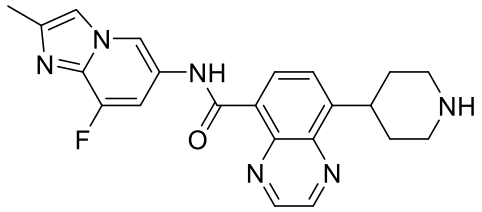
186	
187	
188	
189	
191	

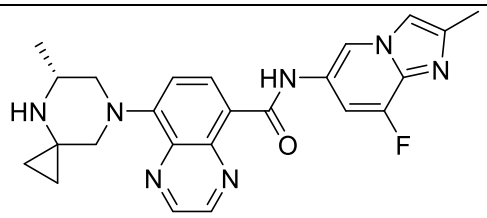
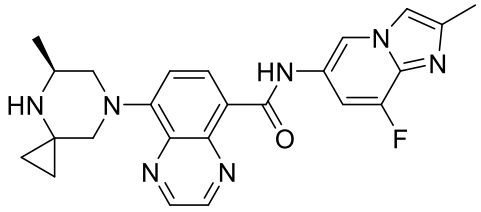
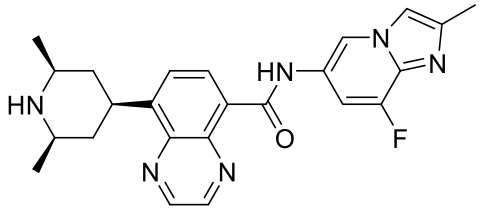
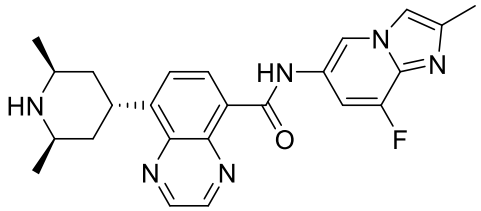
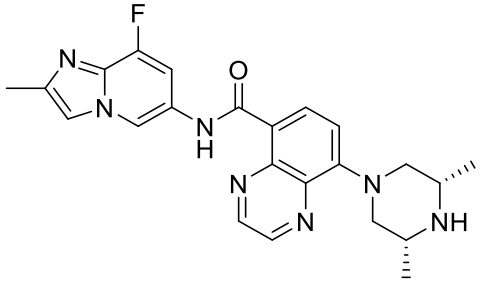
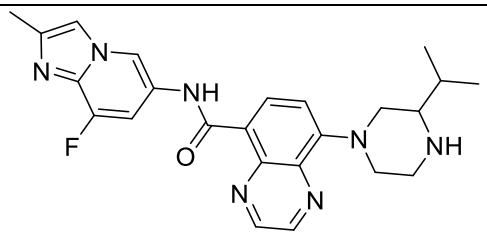
192	
218	
219	
220	
240	
249	

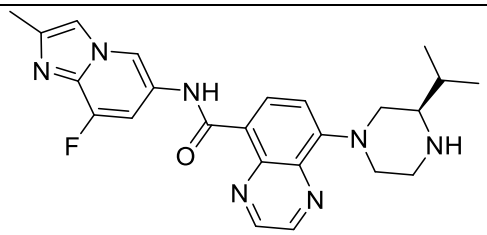
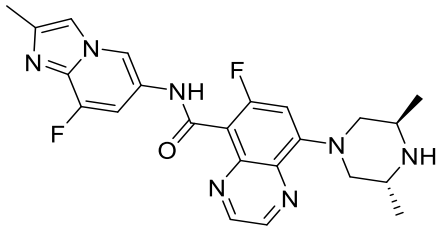
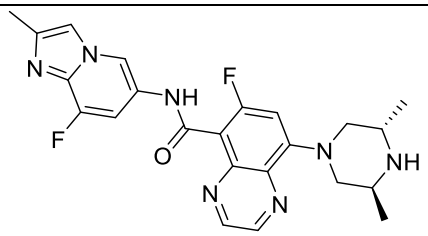
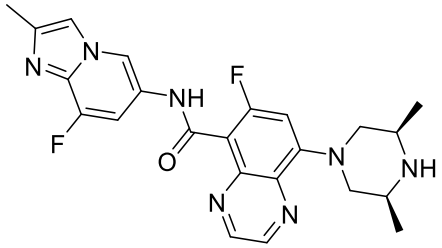
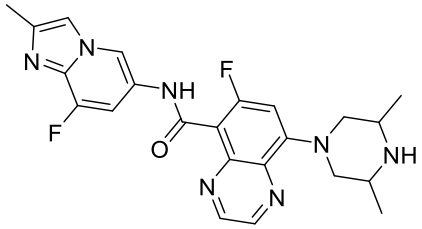
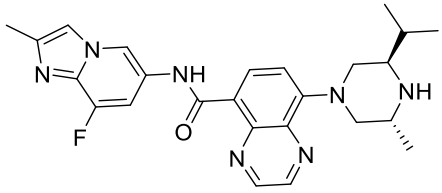
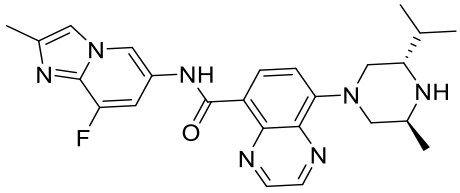
250	
251	
252	
253	
258	
261	

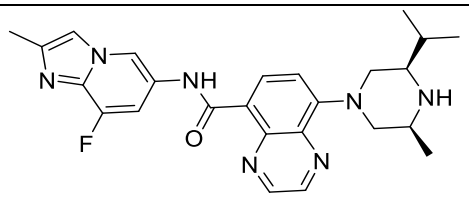
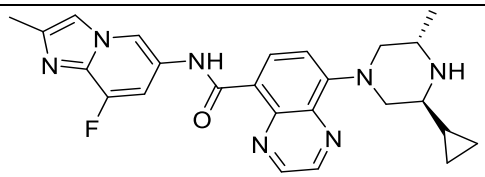
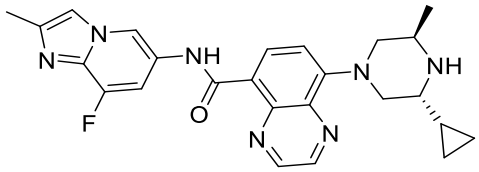
264	
265	
266	
267	
311	
312	
313	

325	
355	
356	
361	
362	
363	
364	

365	
366	
367	
368	
369	
370	
371	

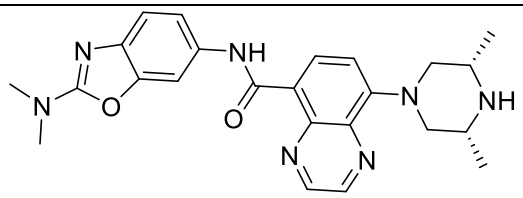
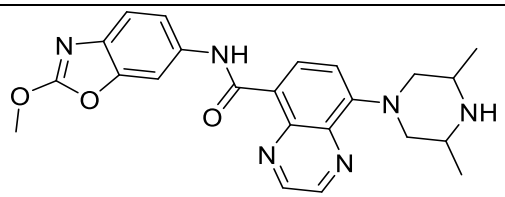
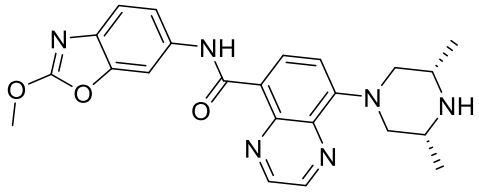
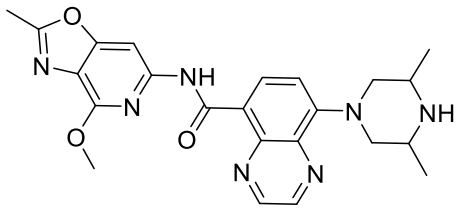
376	
377	
379	
380	
398	
425	

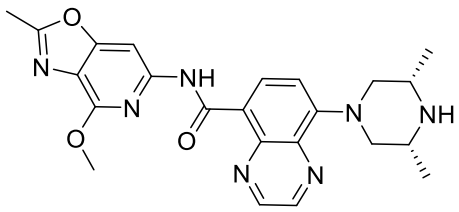
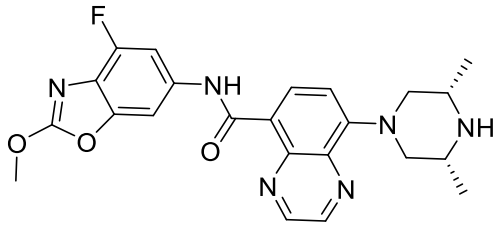
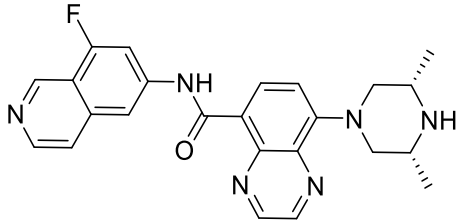
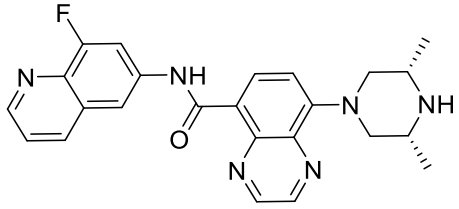
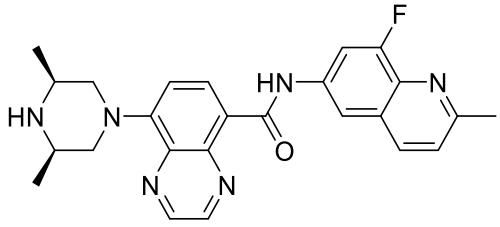
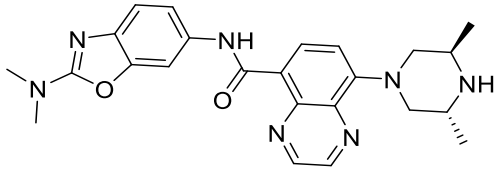
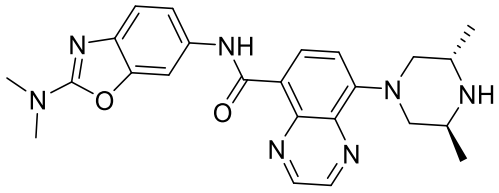
426	
431	
432	
433	
434	
435	
436	

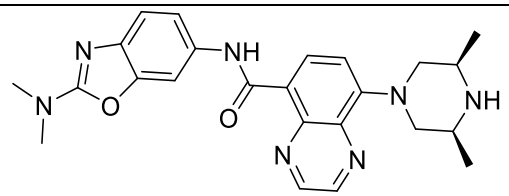
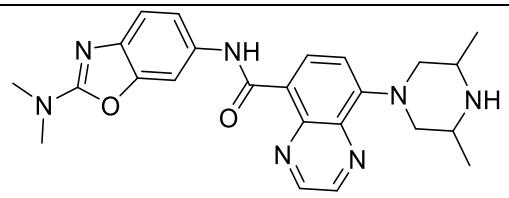
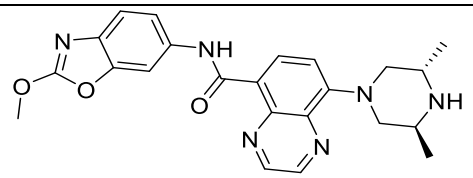
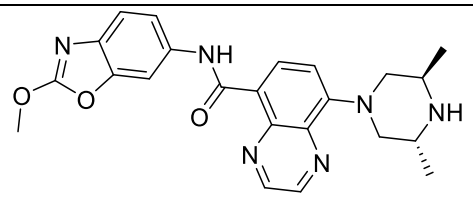
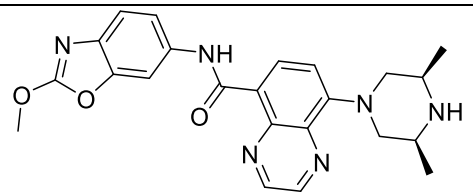
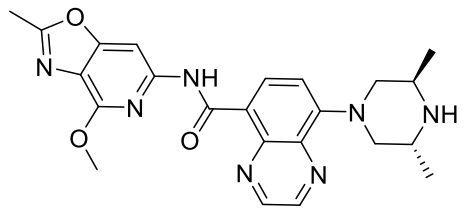
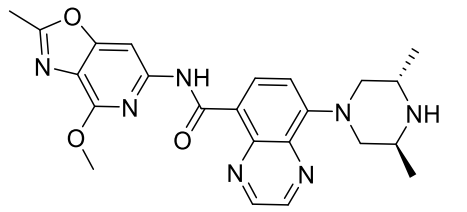
437	
438	
439	

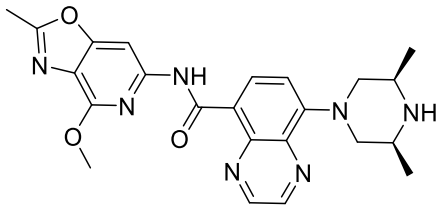
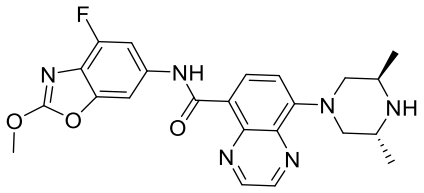
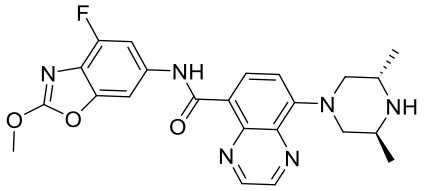
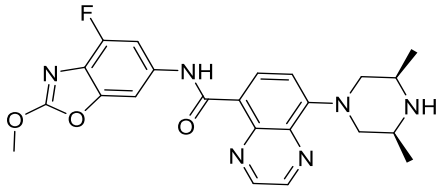
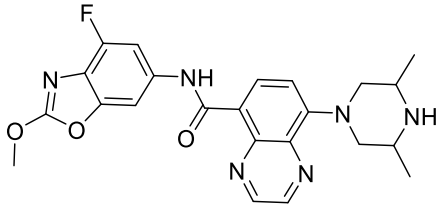
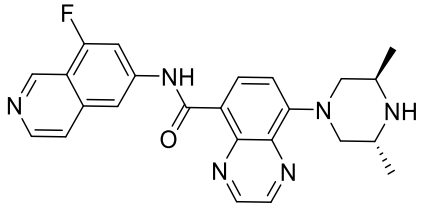
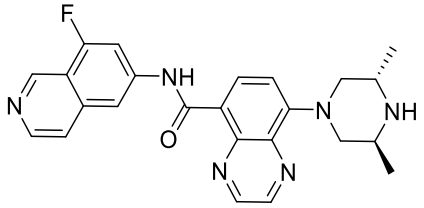
or a pharmaceutically acceptable salt thereof.

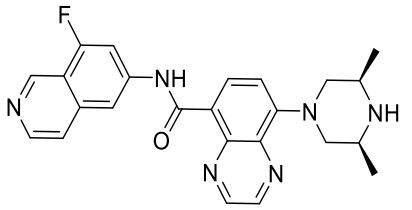
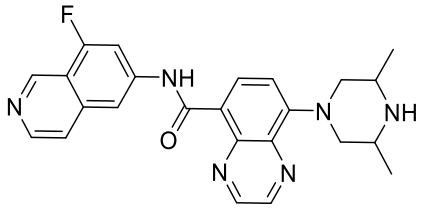
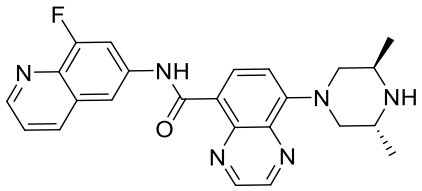
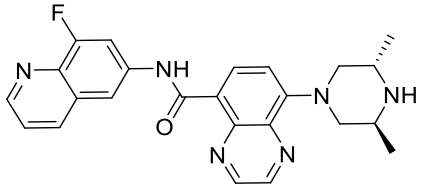
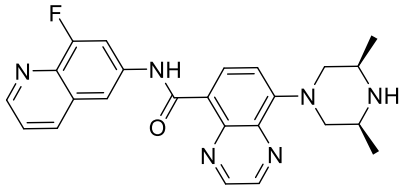
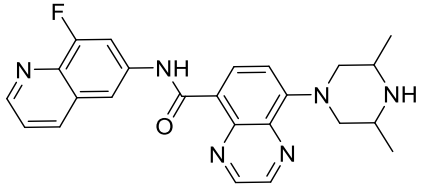
7. A compound selected from:

Cmpd No.	**Structure**
290	
291	
292	
297	

298	
342	
343	
344	
353	
471	
472	

473	
474	
475	
476	
477	
484	
485	

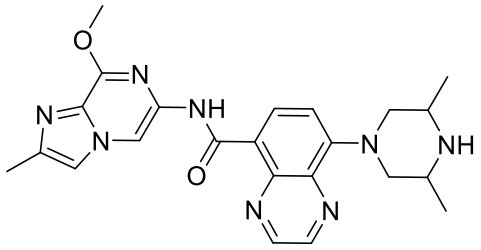
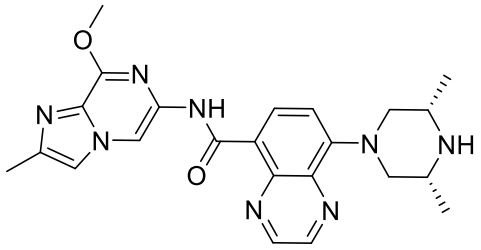
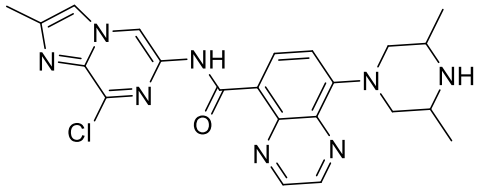
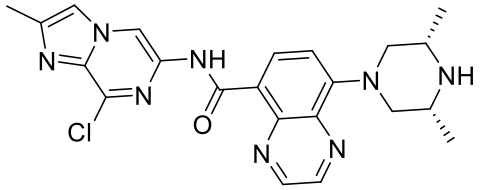
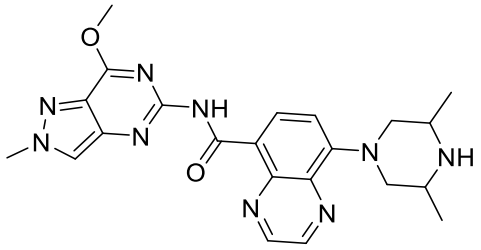
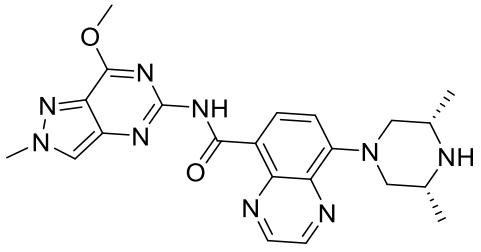
486	
517	
518	
519	
520	
521	
522	

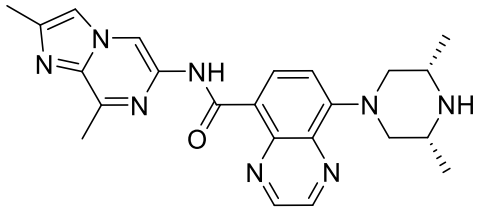
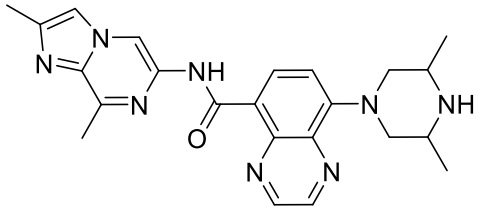
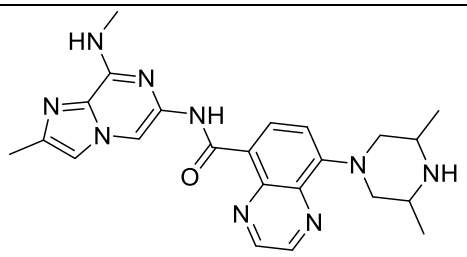
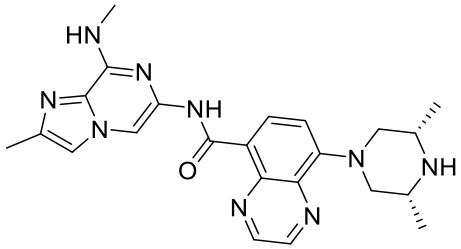
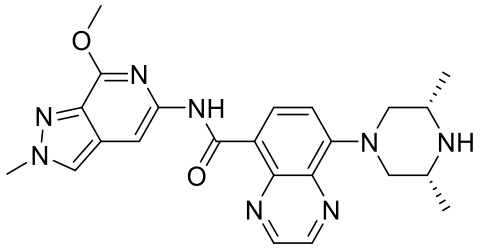
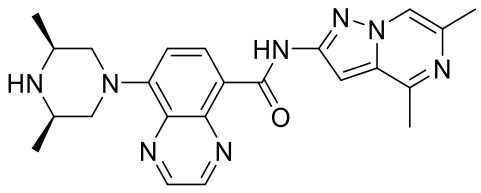
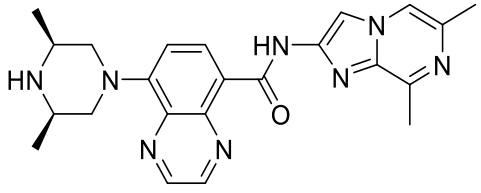
523	
524	
525	
526	
527	
529	

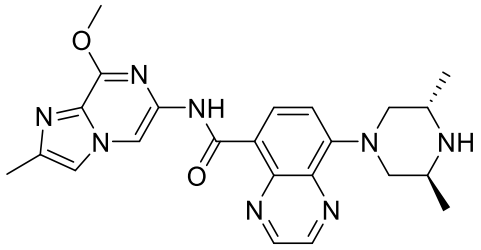
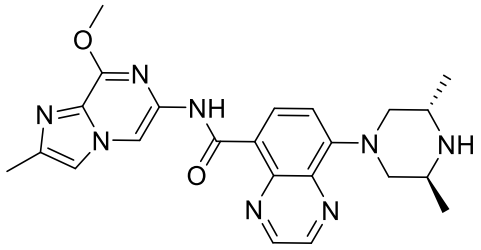
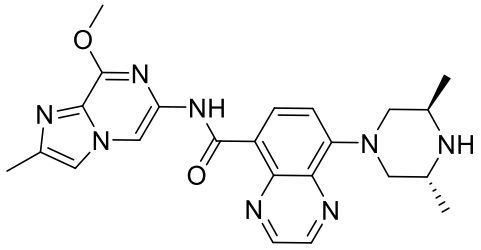
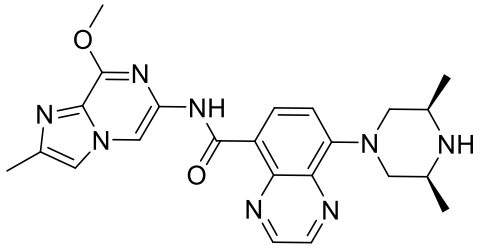
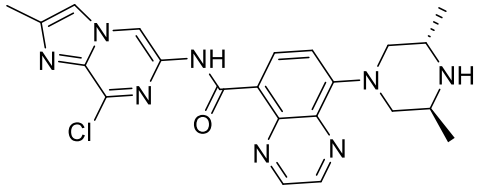
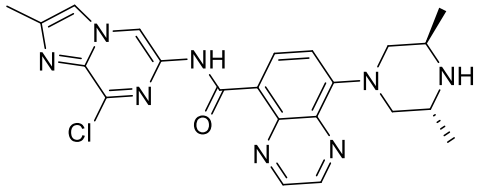
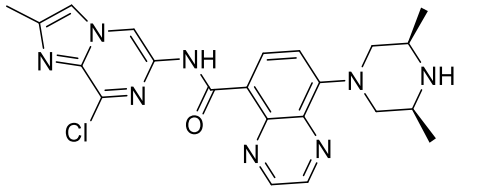
or a pharmaceutically acceptable salt thereof.

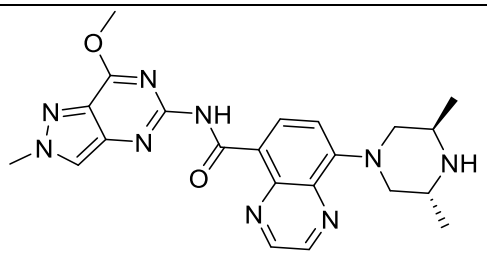
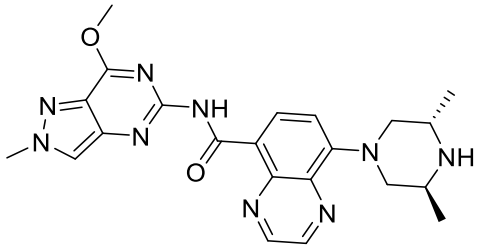
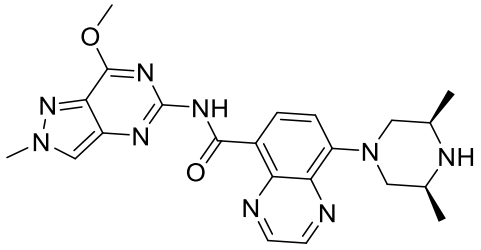
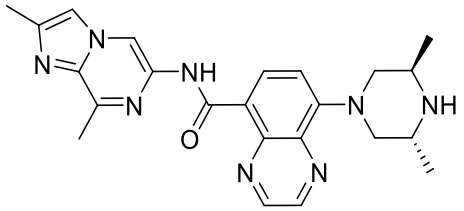
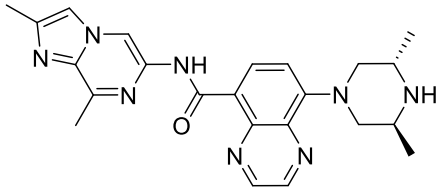
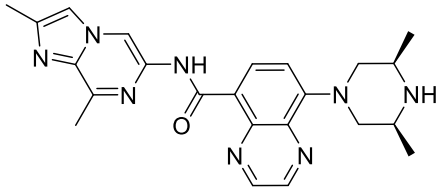
8. A compound selected from:

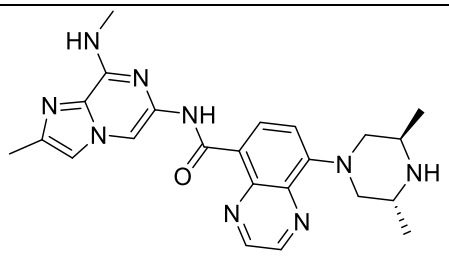
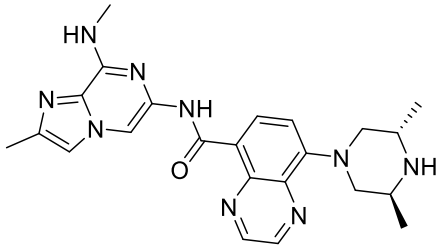
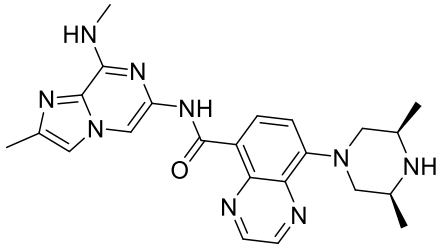
Cmpd No.	**Structure**

276	
277	
278	
279	
286	
287	

288	
289	
307	
308	
352	
354	
359	

381	
449	
450	
451	
452	
453	
454	

465	
466	
467	
468	
469	
470	

499	
500	
501	

or a pharmaceutically acceptable salt thereof.

9. A compound selected from:

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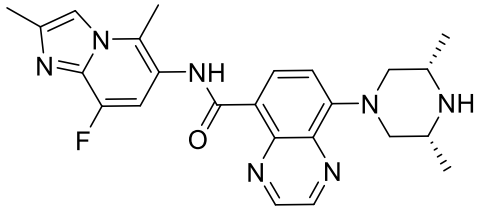
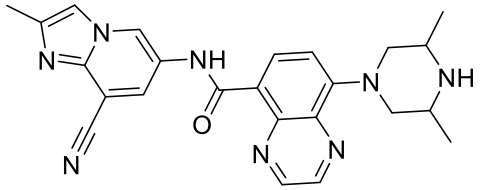
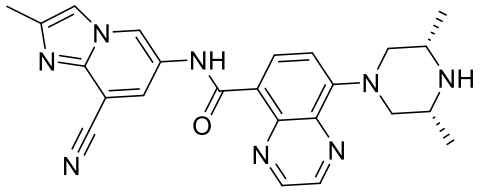
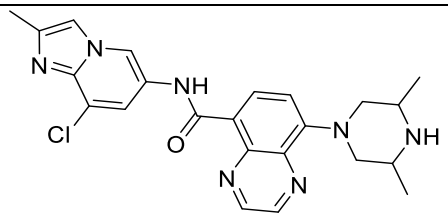
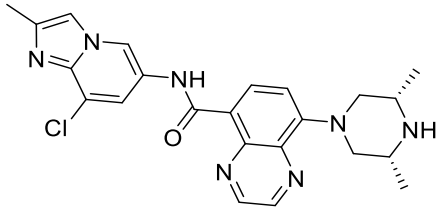
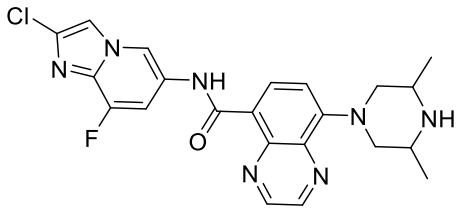
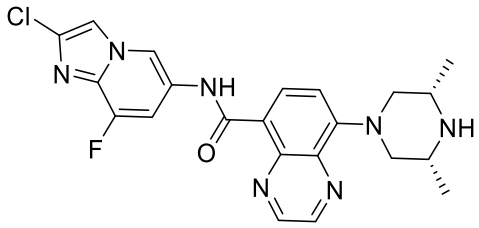
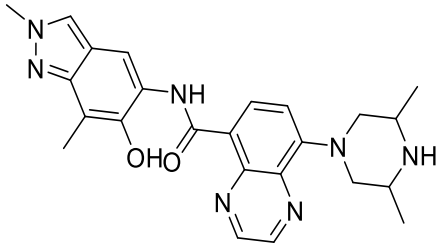
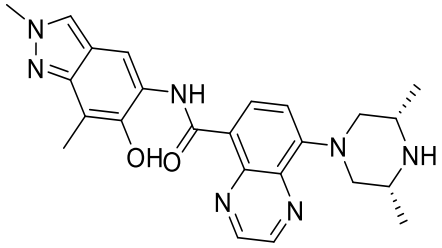
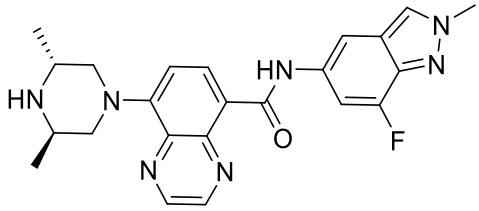
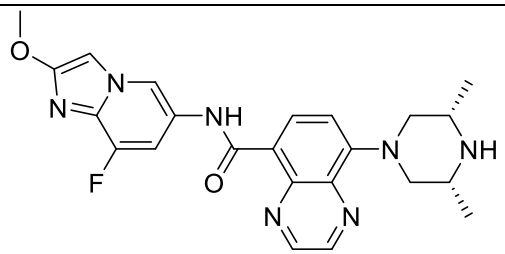
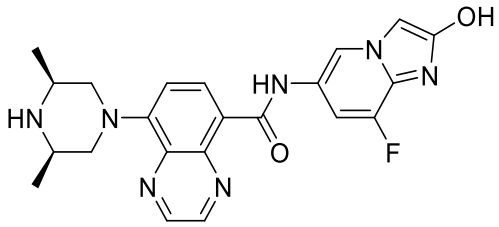
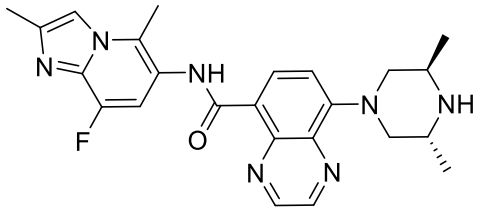
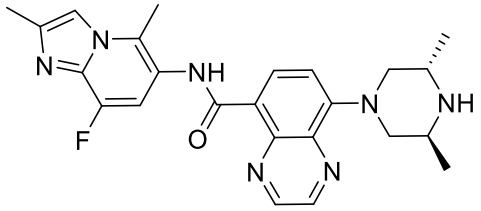
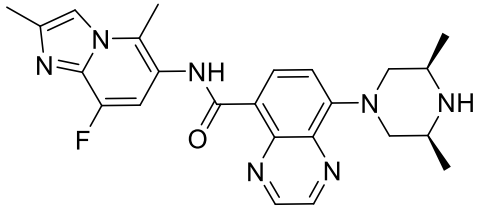
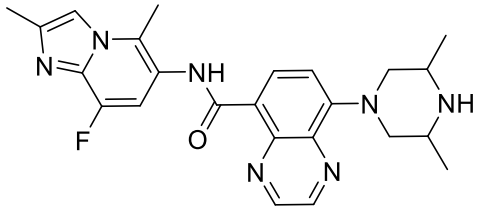
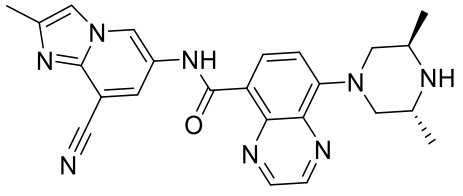
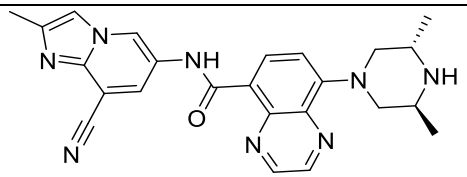
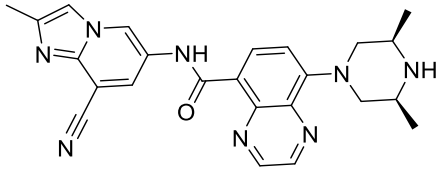
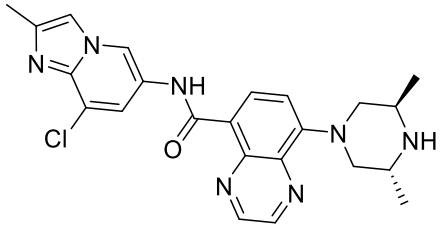
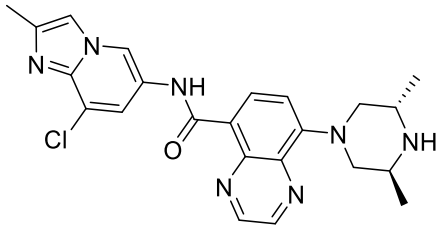
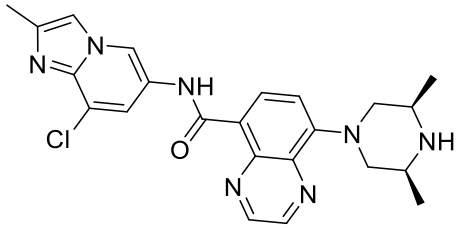
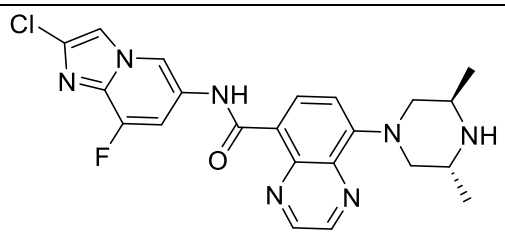
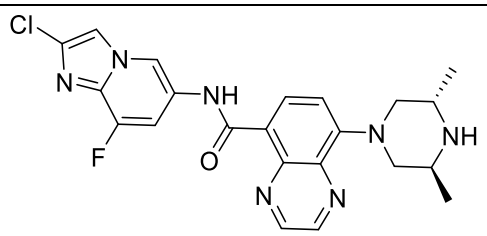
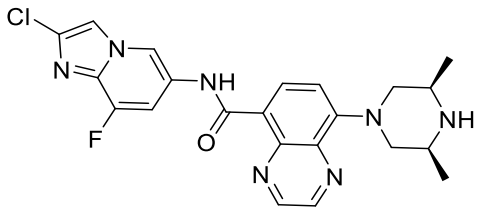
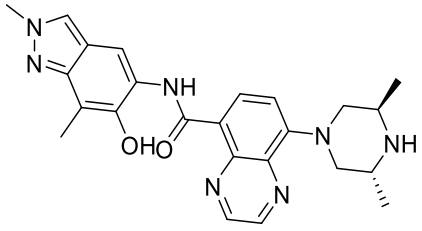
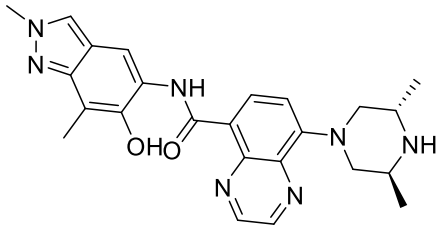
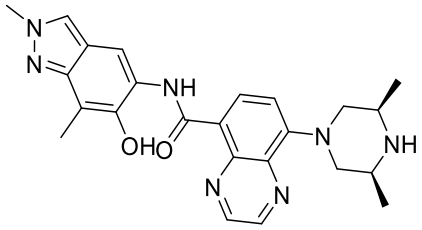
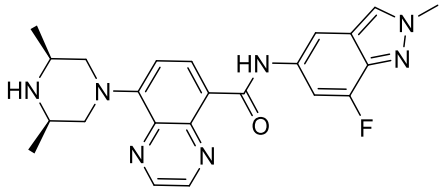
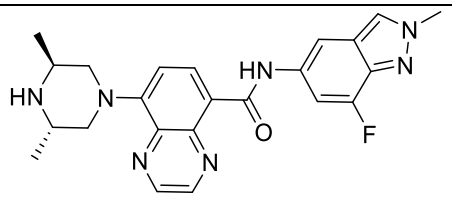
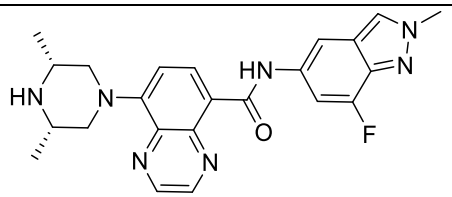
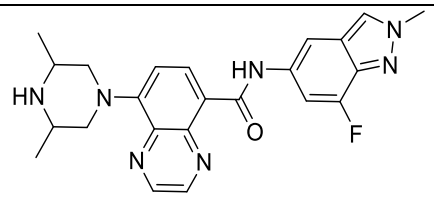
285	
293	
294	

303	
304	
305	
306	
309	
310	
335	

357	
360	
461	
462	
463	
464	
478	

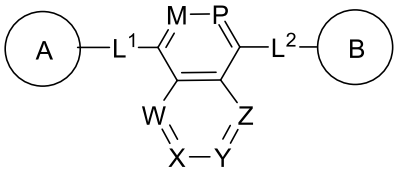
479	
480	
493	
494	
495	
496	
497	

498	
502	
503	
504	
509	
510	
511	

512	

or a pharmaceutically acceptable salt thereof.

10. A compound of Formula (II):



(II)

or a pharmaceutically acceptable salt, solvate, hydrate, tautomer, or stereoisomer thereof, wherein:

A and B are each independently heterocyclyl or heteroaryl, each of which is optionally substituted with one or more R^1;

M and P are each independently $C(R^2)$ or N, wherein at least one of M and P is independently N;

Y and Z are each independently $C(R^3)$ or N, W is N; X is N;

each of L^1 and L^2 is independently absent, $-N(R^4)C(O)-$, or $-C(O)N(R^4)-$;

each R^1 is independently hydrogen, C_1-C_6-alkyl, C_2-C_6-alkenyl, C_2-C_6-alkynyl, C_1-C_6-heteroalkyl, C_1-C_6-haloalkyl, cycloalkyl, heterocyclyl, aryl, C_1-C_6 alkylene-aryl, C_2-C_6 alkenylene-aryl, C_1-C_6 alkylene-heteroaryl, heteroaryl, halo, cyano, oxo, $–OR^A$, $–NR^BR^C$, $–NR^BC(O)R^D$, $–NO_2$, $–C(O)NR^BR^C$, $–C(O)R^D$, $–C(O)OR^D$, or $–S(O)_xR^D$, wherein each alkyl, alkylene, alkenyl, alkynyl, heteroalkyl, haloalkyl, cycloalkyl, heterocyclyl, aryl, and heteroaryl is optionally substituted with one or more R^6; or

two R^1 groups, together with the atoms to which they are attached, form a 3-7-membered cycloalkyl, heterocyclyl, aryl, or heteroaryl, wherein each cycloalkyl, heterocyclyl, aryl, and heteroaryl is optionally substituted with one or more R^6;

each R^2 is independently hydrogen, C_1-C_6-alkyl, C_2-C_6-alkenyl, C_2-C_6-alkynyl, C_1-C_6-heteroalkyl, C_1-C_6-haloalkyl, halo, cyano, $–OR^A$, $–NR^BR^C$,$–NR^BC(O)R^D$, $–NO_2$, $–C(O)NR^BR^C$, $–C(O)OR^D$, or $–S(O)_xR^D$, or $–C(O)R^D$;

R^3 is hydrogen, C_1-C_6-alkyl, C_2-C_6-alkenyl, C_2-C_6-alkynyl, C_1-C_6-heteroalkyl, C_1-C_6-haloalkyl, halo, cyano, $–OR^A$, $–NR^BR^C$,$–NR^BC(O)R^D$, $–NO_2$, $–C(O)NR^BR^C$, $–C(O)OR^D$, or $–S(O)_xR^D$, or $–C(O)R^D$;

each R^4 is independently hydrogen, C_1-C_6-alkyl, or C_1-C_6-haloalkyl;

each R^6 is independently C_1-C_6-alkyl, C_2-C_6-alkenyl, C_2-C_6-alkynyl, C_1-C_6-heteroalkyl, C_1-C_6-haloalkyl, cycloalkyl, heterocyclyl, aryl, heteroaryl, halo, cyano, oxo, $–OR^A$, $–NR^BR^C$, $–NR^BC(O)R^D$, $–NO_2$, $–C(O)NR^BR^C$, $–C(O)R^D$, $–C(O)OR^D$, or $–S(O)_xR^D$, wherein each alkyl, alkenyl, alkynyl, heteroalkyl, haloalkyl, cycloalkyl, heterocyclyl, aryl, and heteroaryl is optionally substituted with one or more R^{11};

each R^A is independently hydrogen, C_1-C_6 alkyl, C_2-C_6 alkenyl, C_2-C_6 alkynyl, C_1-C_6 heteroalkyl, C_1-C_6 haloalkyl, aryl, heteroaryl, C_1-C_6 alkylene-aryl, C_1-C_6 alkylene-heteroaryl, $–C(O)R^D$, or $–S(O)_xR^D$;

each of R^B and R^C is independently hydrogen, C_1-C_6 alkyl, C_2-C_6 alkenyl, C_2-C_6 alkynyl, C_1-C_6 heteroalkyl, C_1-C_6 haloalkyl, cycloalkyl, heterocyclyl, aryl, heteroaryl, C_1-C_6 alkylene-aryl, or C_1-C_6 alkylene-heteroaryl, $–OR^A$, $–C(O)NR^BR^C$, $–C(O)R^D$, $–C(O)OR^D$, or $–S(O)_xR^D$; or

R^B and R^C together with the atom to which they are attached form a 3-7-membered heterocyclyl ring optionally substituted with one or more R^7;

each R^D is independently hydrogen, C_1-C_6 alkyl, C_2-C_6 alkenyl, C_2-C_6 alkynyl, C_1-C_6 heteroalkyl, C_1-C_6 haloalkyl, cycloalkyl, heterocyclyl, aryl, heteroaryl, C_1-C_6 alkylene-aryl, or C_1-C_6 alkylene-heteroaryl;

each R^7 is C_1-C_6-alkyl, halo, cyano, oxo, or $–OR^{A1}$;

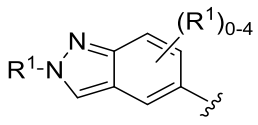
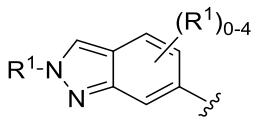
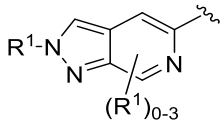
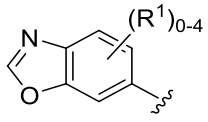
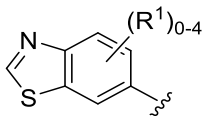
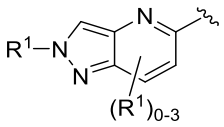
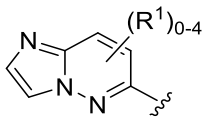
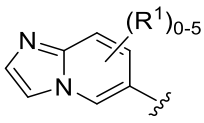
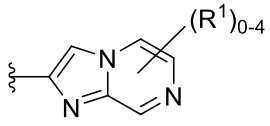
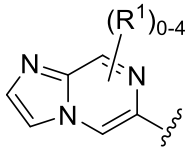
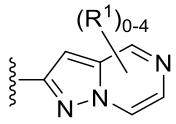
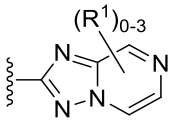
each R^{11} is independently C_1-C_6-alkyl, C_1-C_6-heteroalkyl, C_1-C_6-haloalkyl, cycloalkyl, heterocyclyl, aryl, heteroaryl, halo, cyano, oxo, or $–OR^A$;

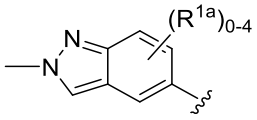
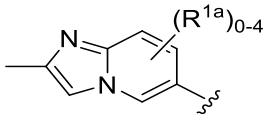
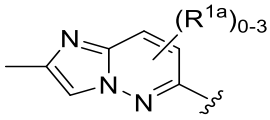
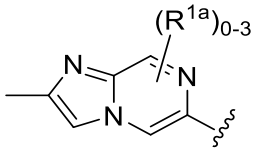
each R^{A1} is hydrogen or C_1-C_6-alkyl; and

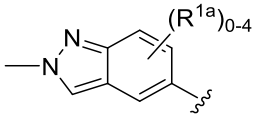
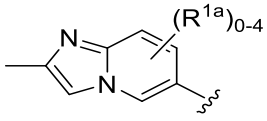
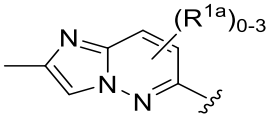
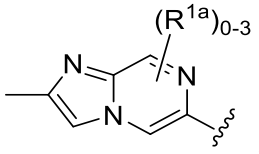
x is 0, 1, or 2.

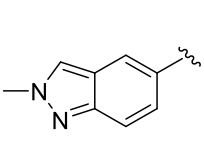
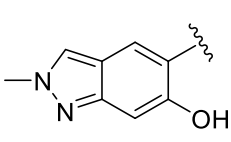
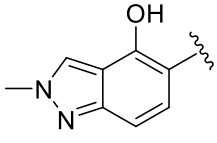
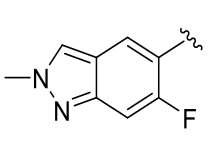
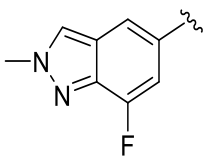
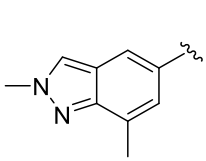
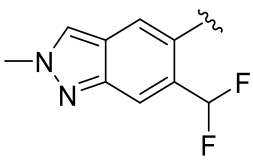
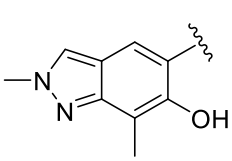
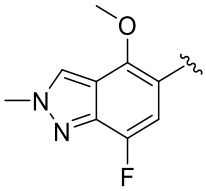
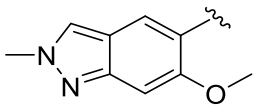
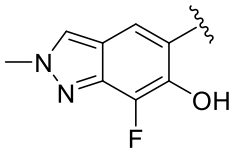
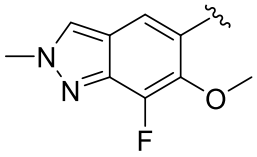
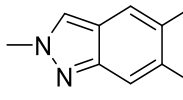
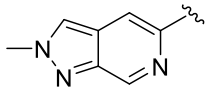
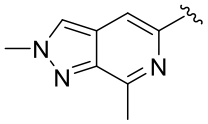
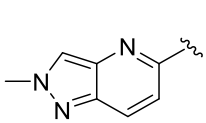
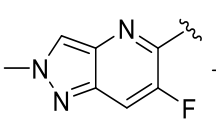
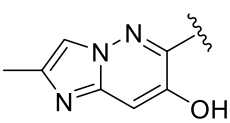
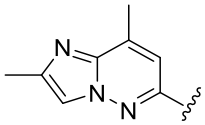
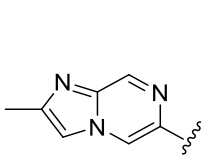
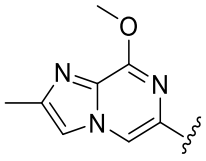
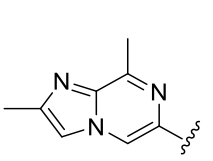
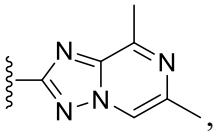
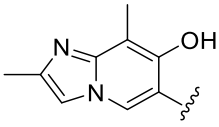
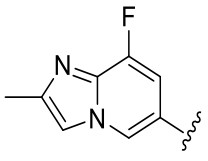
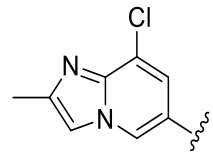
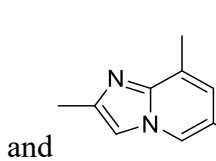
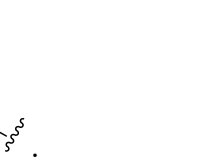
11. The compound of claim 10, wherein one of A and B is independently a monocyclic heteroaryl or bicyclic heteroaryl, each of which is optionally substituted with one or more R^1.

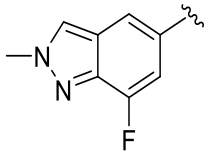
12. The compound of claim 10 or 11, wherein one of A and B is independently a nitrogen-containing heteroaryl optionally substituted with one or more R^1.

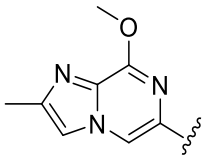
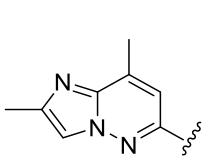
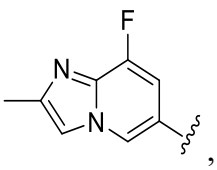
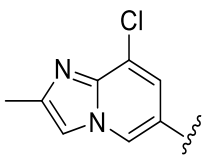
13. The compound of any one of claims 10-12, wherein one of A and B is independently selected from , , , , , , , , , , , and , wherein R^1 is as described in claim 10.

14. The compound of any one of claims 10-13, wherein one of A and B is independently selected from , , , and , wherein each R^{1a} is independently C_1-C_6-alkyl, C_1-C_6-heteroalkyl, C_1-C_6-haloalkyl, halo, cyano, or $–OR^A$, and each alkyl, heteroalkyl, and haloalkyl is optionally substituted with one or more R^7.

15. The compound of any one of claims 10-14, wherein A is selected from , , , and , wherein each R^{1a} is independently C_1-C_6-alkyl, C_1-C_6-heteroalkyl, C_1-C_6-haloalkyl, halo, cyano, or $–OR^A$, and each alkyl, heteroalkyl, and haloalkyl is optionally substituted with one or more R^7.

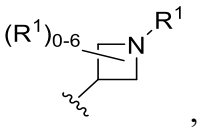
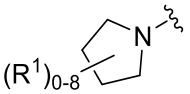
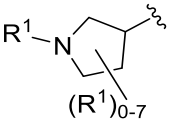
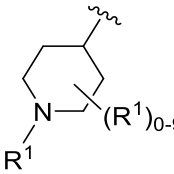
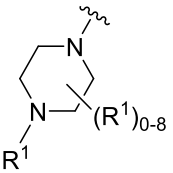
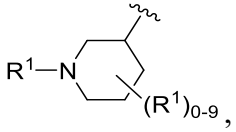
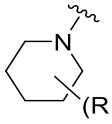
16. The compound of any one of claims 10-15, wherein one of A and B is independently selected from , , , , , , , , , , , , , , , , , , , , , , , , , , , , , , , and .

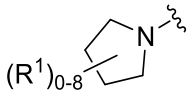
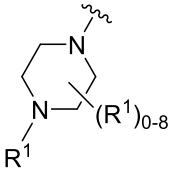
,

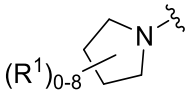
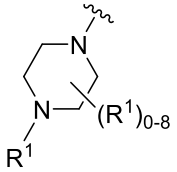
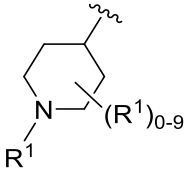
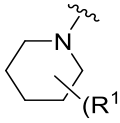
17. The compound of any one of claims 10-16, wherein A selected from , , , and .

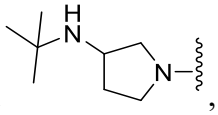
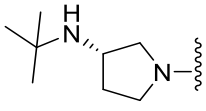
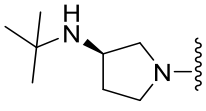
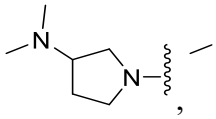
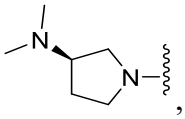
18. The compound of any one of claims 10-17, wherein one of A and B is independently a monocyclic heterocyclyl or bicyclic heterocyclyl, each of which is optionally substituted with one or more R^1.

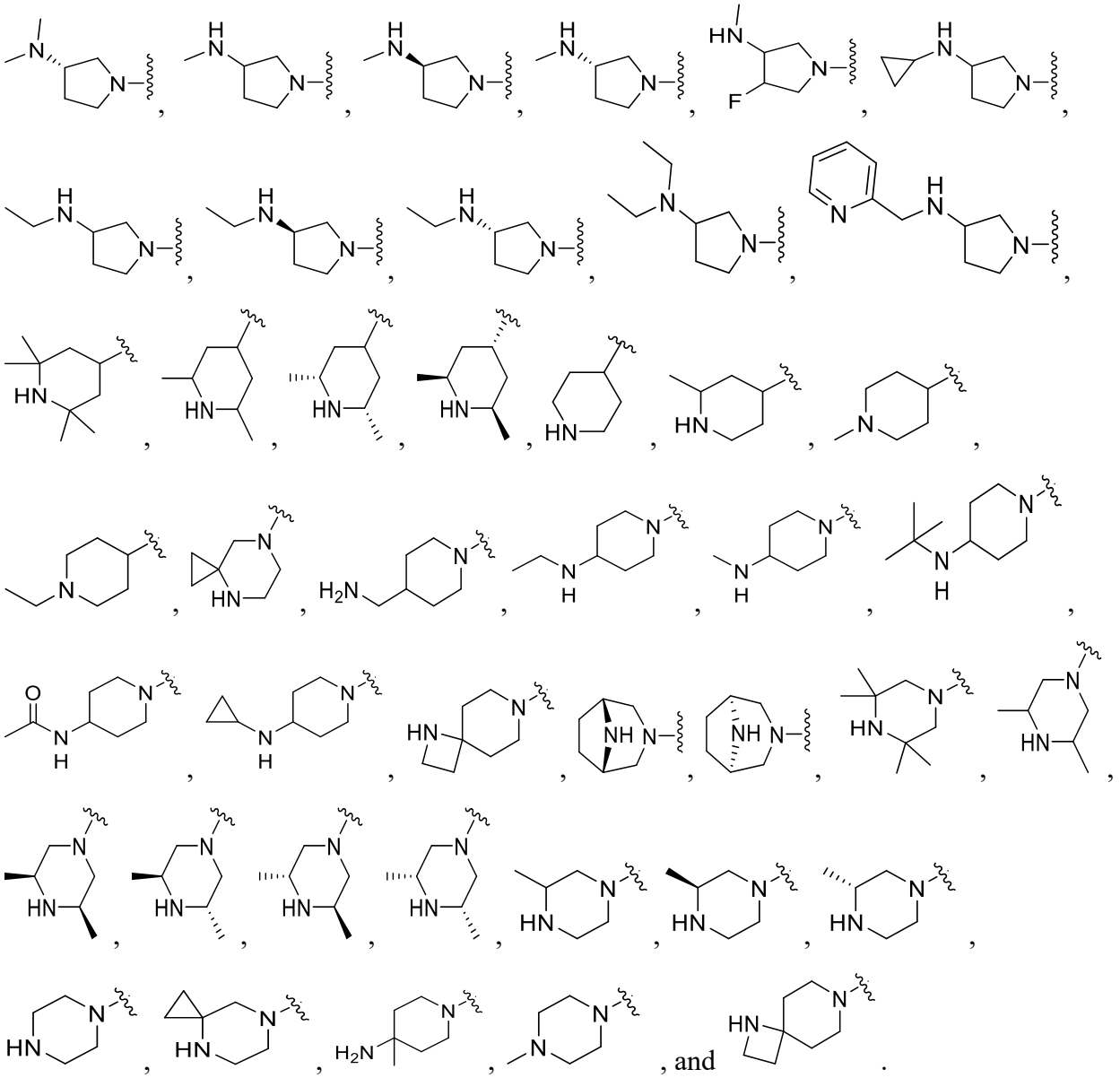
19. The compound of any one of claims 10-18, wherein one of A and B is independently a nitrogen-containing heterocyclyl optionally substituted with one or more R^1.

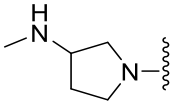
20. The compound of any one of claims 10-19, wherein one of A and B is independently selected from , , , , , , and , wherein R^1 is as described in claim 10.

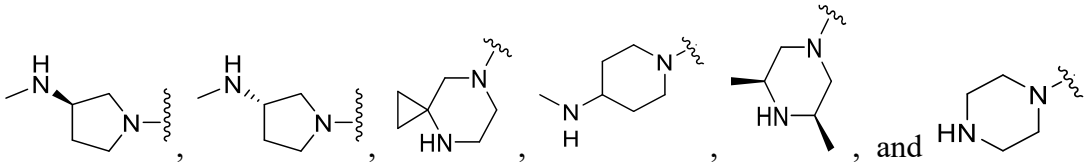
21. The compound of any one of claims 10-20, wherein one of A and B is selected from  and , wherein R^1 is as described in claim 10.

22. The compound of any one of claims 10-21, wherein B is selected from , , , and , wherein R^1 is as described in claim 10.

23. The compound of any one of claims 10-22, wherein one of A and B is independently selected from , , , , ,



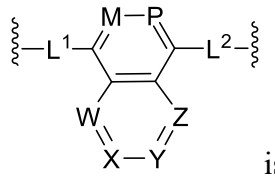
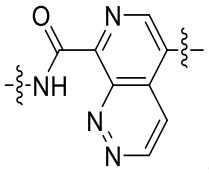
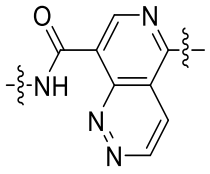
24. The compound of any one of claims 10-23, wherein B is selected from, ,



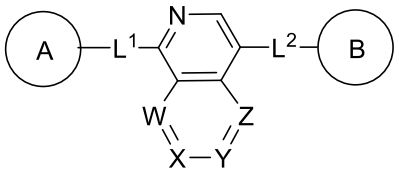
25. The compound of any one of claims 10-24, wherein L^1 is -C(O)NH- and L^2 is absent.

26. The compound of any one of claims 10-25, wherein M is $C(R^2)$ and P is N.

27. The compound of any one of claims 10-26, wherein M is N and P is $C(R^2)$.

28. The compound of any one of claims 10-27, wherein  is selected from  and .

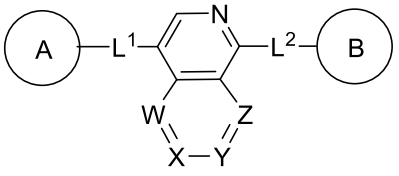
29. The compound of any one of claims 10-28, wherein the compound of Formula (II) is Formula (II-a):



(II-a)

or a pharmaceutically acceptable salt, solvate, hydrate, tautomer, or stereoisomer thereof, wherein each of A, B, W, X, Y, Z, M, P, L^1, L^2, and subvariables thereof are defined as in claim 10.

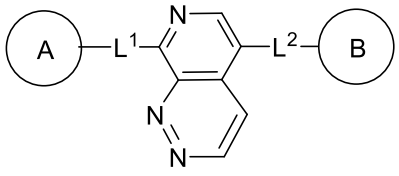
30. The compound of any one of claims 10-29, wherein the compound of Formula (II) is Formula (II-b):



(II-b)

or a pharmaceutically acceptable salt, solvate, hydrate, tautomer, or stereoisomer thereof, wherein each of A, B, W, X, Y, Z, L^1, L^2, and subvariables thereof are defined as in claim 10.

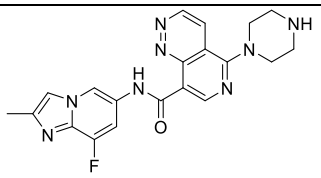
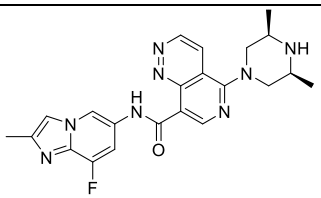
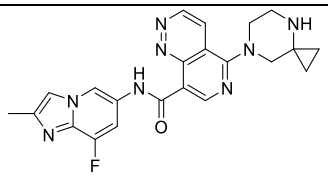
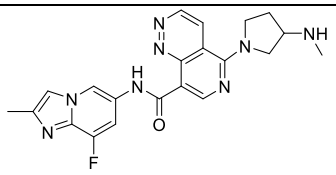
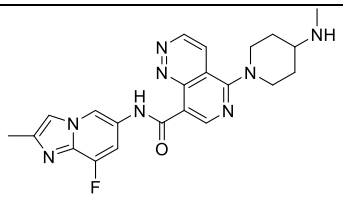
31. The compound of any one of claims 10-30, wherein the compound of Formula (II) is Formula (II-d):

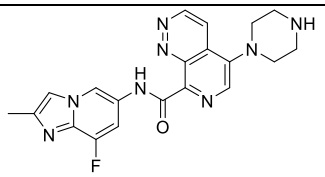
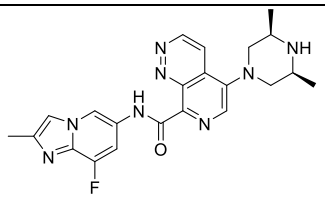
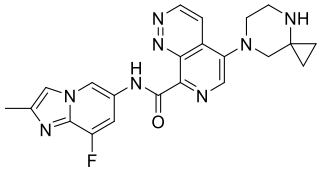
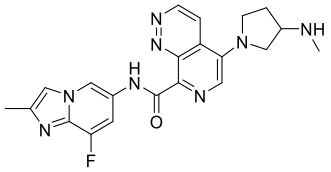
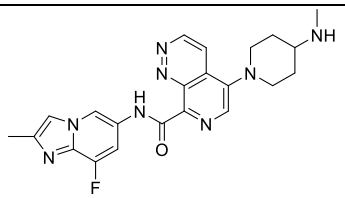
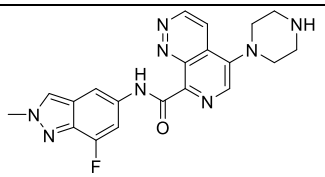
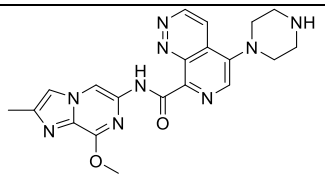
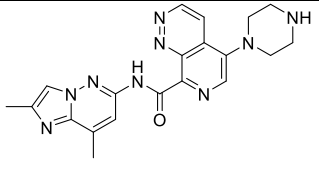


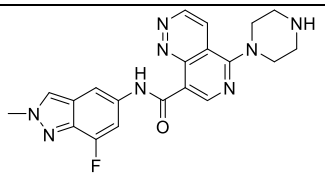
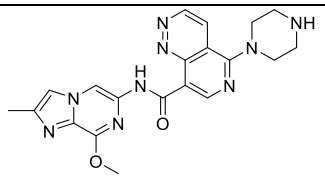
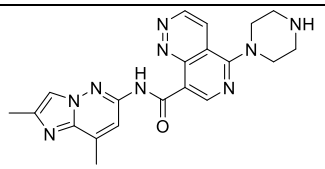
(II-d)

or a pharmaceutically acceptable salt, solvate, hydrate, tautomer, or stereoisomer thereof, wherein each of A, B, L^1, L^2, and subvariables thereof are defined as in claim 10.

32. The compound of any one of claims 10-31, wherein the compound is selected from one of the following:

Compound No.	Structure
550	
551	
552	
553	
554	

555	
556	
557	
558	
559	
560	
561	
562	

563	
564	
565	

or a pharmaceutically acceptable salt, solvate, hydrate, tautomer, or stereoisomer thereof.

33. A pharmaceutical composition comprising a compound of any one of the preceding claims and a pharmaceutically acceptable excipient.

34. The compound of any one of claims 1-32, or the pharmaceutical composition of claim 33, wherein the compound:

(i) alters a target nucleic acid;

(ii) binds to a target nucleic acid; or

(iii) stabilizes a target nucleic acid.

35. The compound of any one of claims 1-32, or the pharmaceutical composition of claim 33, wherein the compound:

(i) increases splicing at splice site on a target nucleic acid , by about 0.5%, 1%, 2%, 3%, 4%, 5%, 6%, 7%, 8%, 9%, 10%, 15%, 20%, 25%, 30%, 35%, 40%, 45%, 50%, 55%, 60%, 65%, 70%, 75%, 80%, 85%, 90%, 95%, or more; or

(ii) decreases splicing at splice site on a target nucleic acid, by about 0.5%, 1%, 2%, 3%, 4%, 5%, 6%, 7%, 8%, 9%, 10%, 15%, 20%, 25%, 30%, 35%, 40%, 45%, 50%, 55%, 60%, 65%, 70%, 75%, 80%, 85%, 90%, 95%, or more.

36. A method of forming a complex comprising a component of a spliceosome, a nucleic acid, and a compound of any one of claims 1-32, described herein:

comprising contacting the nucleic acid with a compound of any one of claims 1 to 32.

37. The method of claim 36, wherein the component of a spliceosome is recruited to the nucleic acid in the presence of the compound of any one of claims 1-32 or a pharmaceutically acceptable salt or a composition thereof.

38. A method of altering the conformation of a nucleic acid comprising contacting the nucleic acid with a compound of any one of claims 1-32 or a pharmaceutically acceptable salt or a composition thereof.

39. The method of claim 38, wherein the altering comprises:

(i) forming a bulge in the nucleic acid;

(ii) stabilizing a bulge in the nucleic acid; or

(iii) reducing a bulge in the nucleic acid.

40. The method of any one of claims 38-39, wherein the nucleic acid comprises a splice site.

41. Use of a compound of any one of claims 1-32 or a pharmaceutically acceptable salt or a composition thereof in the manufacture of a medicament for treating a disease or disorder in a subject, wherein the disease or disorder is selected from the group consisting of a proliferative disease, a neurological disease or disorder, autoimmune disease or disorder, immunodeficiency disease or disorder, lysosomal storage disease or disorder, cardiovascular disease or disorder, metabolic disease or disorder, respiratory disease or disorder, renal disease or disorder, or infectious disease.

42. The use of claim 41, wherein the disease or disorder comprises a proliferative disease.

43. The use of any one of clams 41-42, wherein the proliferative disease is cancer.

44. The use of any one of claims 41-43, wherein the proliferative disease comprises adenoid cystic carcinoma, colorectal cancer, leukemia, lung cancer, prostate cancer, breast cancer, or ovarian cancer

45. The use of claim 41, wherein the disease or disorder comprises a neurological disease or disorder, autoimmune disease or disorder, immunodeficiency disease or disorder, lysosomal storage disease or disorder, cardiovascular disease or disorder, metabolic disease or disorder, respiratory disease or disorder, renal disease or disorder, or infectious disease.

46. The use of claim 45, wherein the disease or disorder comprises neurological disease or disorder.

47. The use of claim 45, wherein the disease or disorder comprises Huntington's disease.

48. A compound of any one of claims 1-32 or a pharmaceutically acceptable salt or a composition thereof for use in therapy.