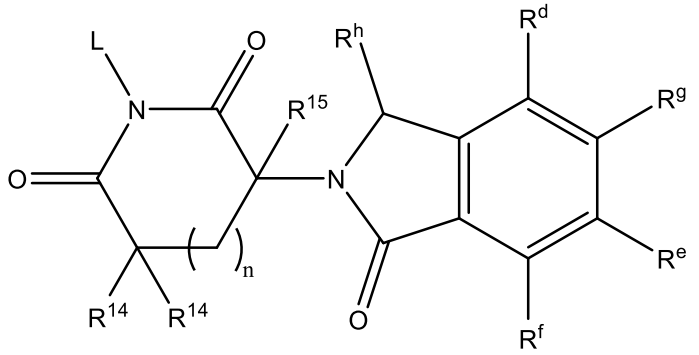
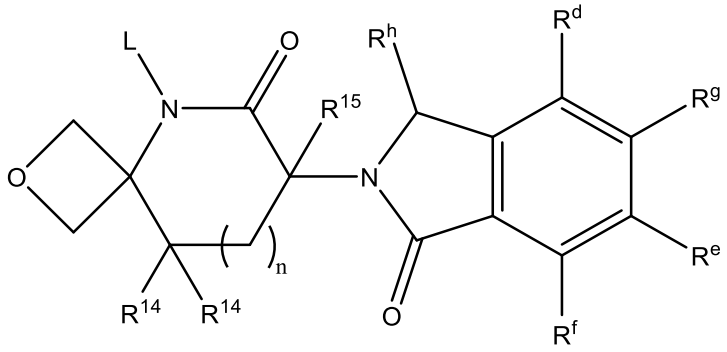
CLAIMS

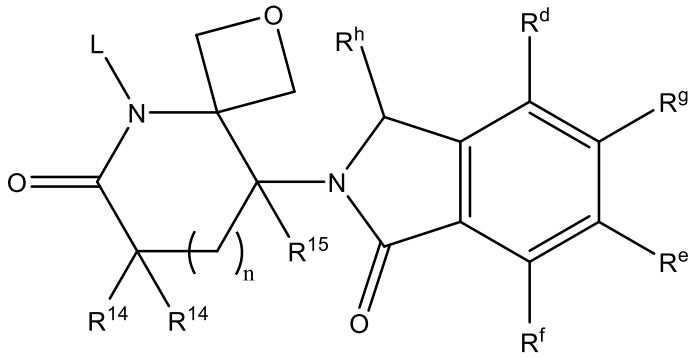
1. A compound of formula (Ia), (Ib) or (Ic):



(Ia)



(Ib)



(Ic)

or a pharmaceutically acceptable salt, ester, optically active isomer, racemate, solvate, amino acid conjugate, or prodrug thereof,

in which

L is selected from hydrogen, alkyl, alkenyl, benzyl, aryl, heteroaryl, haloalkyl, haloalkenyl, $-CH_2OC(O)^tBu$, $-CH_2C(O)OR''$, $-C(O)R''$, $-C(O)OR''$, $-C(O)NH_2$, $-C(O)NHR''$, $-C(O)NR''_2$, $-OR''$, $-NR''_2$, $-S(O)_2R''$ or P(O)(OR")(OR");

each R'' is independently selected from hydrogen, alkyl, cycloalkyl, alkenyl, cycloalkenyl, aryl, heteroaryl, or benzyl;

each R^{14} is independently selected from deuterium and hydrogen;

R^{15} is selected from hydrogen, deuterium and C_1-C_4 alkyl;

R^g is $CR^aR^bR^c$,

R^h is selected from H and C_1-C_4 alkyl;

R^a is selected from H, deuterium, and C_1-C_4 alkyl;

R^b is selected from H, deuterium, and C_1-C_4 alkyl;

R^c is selected from NR^1R^2, OH, OR^6, CH_2X, CHX_2, and CX_3;

each of R^d, R^e and R^f is independently selected from H, deuterium, X, C_1-C_4 alkyl, and NH_2;

n is 0, 1 or 2;

X is selected from F, Cl, Br and I;

R^1 is selected from H and C_1-C_3 alkyl,

R^2 is selected from H, C_1-C_3 alkyl, $–COR^3$, and $–COOR^3$,

or alternatively R^1 and R^2, together with the nitrogen atom to which they are attached, form an 5 or 6-membered heterocycle, in which the heterocycle is unsubstituted or in which one or more carbon atoms of the heterocycle form part of a carbonyl group; or R^1 and R^a, together with the carbon and nitrogen atoms to which they are attached, form a 5 or 6-membered heterocycle;

R^3 is selected from:

unsubstituted C_1-C_4 alkyl;

C_1-C_{10} alkyl substituted with one or more R^4, in which each R^4 is independently selected from NH_2, $NHC(NH)NH_2$, $NHCOR^5$, $NHCOOR^5$, -OH, OR^5, $OCOR^5$, unsubstituted 5-membered heterocyclyl, substituted or unsubstituted dioxolyl, unsubstituted 6-membered heterocyclyl, 5-membered heteroaryl, 6-membered heteroaryl, indole, and 6-membered aryl substituted with one or more substituents independently selected from C_1-C_4 alkyl, -OH, $-CH_2$-OH, -OCO(C_1-C_4 alkyl) and CH_2OCO(C_1-C_4 alkyl); and in which R^4 is not X;

C_2-C_{10} alkyl substituted with a halophenyl group;

6-membered aryl substituted with one or more substituents independently selected from CH_2-OH or $CH_2OCO(C_1$-C_4 alkyl);

unsubstituted 5- or 6-membered heterocyclyl

R^5 is unsubstituted C_1-C_6 alkyl; or C_1-C_6 alkyl substituted with one or more substituents independently selected from 5-membered heterocyclyl, 6-membered heterocyclyl, 5-membered aryl, 6-membered aryl, 5-membered heteroaryl and 6-membered heteroaryl; and

R^6 is unsubstituted cyclopentyl or cyclohexyl; or cyclopentyl or cyclohexyl substituted with one or more NH_2;

in which, in formula (Ia):

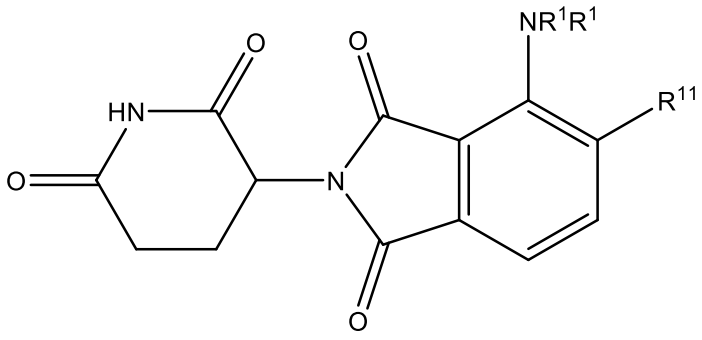
when R^a, R^b, R^1 and R^2 are each H, then n is 0 or 1;

when R^a, R^b, R^d, R^e, R^f, R^h, R^1, R^2, R^{14} and L are each H, and R^{15} is H or C_1-C_4 alkyl, then n is 0;

when R^a, R^b and R^1 are each H and R^2 is -COR^3, then n is 0 or 1; and

when R^a, R^b, R^d, R^e, R^f and R^1 are each H and R^3 is unsubstituted C_1-C_4 alkyl, then n is 0.

2. A compound of formula (II):



(II)

or a pharmaceutically acceptable salt, ester, optically active isomer, racemate, solvate, amino acid conjugate, or prodrug thereof,

in which:

each R^1 is independently selected from H and C_1-C_4 alkyl;

R^{11} is OH or OR^{5a}; and

R^{5a} is unsubstituted C_1-C_6 alkyl; or C_1-C_6 alkyl substituted with one or more substituents independently selected from 5-membered heterocyclyl, 6-membered heterocyclyl, 5-membered heteroaryl and 6-membered heteroaryl.

3. A compound according to Claim 1, in which R^3 is selected from:

unsubstituted C_1-C_4 alkyl;

C_1-C_{10} alkyl substituted with one or more R^4, in which each R^4 is independently selected from NH_2, $NHC(NH)NH_2$, $NHCOR^5$, $NHCOOR^5$, -OH, OR^5, $OCOR^5$, unsubstituted 5-membered heterocyclyl, substituted or unsubstituted dioxolyl, unsubstituted 6-membered heterocyclyl, 5-membered heteroaryl, 6-membered heteroaryl, indole, and 6-membered aryl substituted with one or more substituents independently selected from C_1-C_4 alkyl, OH, -CH_2-OH, -OCO(C_1-C_4 alkyl) and CH_2OCO(C_1-C_4 alkyl); and in which R^4 is not X;

6-membered aryl substituted with one or more substituents independently selected from CH_2-OH or CH_2OCO(C_1-C_4 alkyl); and

unsubstituted 5- or 6-membered heterocyclyl; optionally in which when the C_1-C_{10} alkyl is substituted with indole, the C_1-C_{10} alkyl is further substituted with at least one additional R^4.

4. A compound according to Claim 2 or 3, in which R^{11} is OH.

5. A compound according to Claim 1, 2 or 3, in which NR^1R^1 is NH_2.

6. A compound according to any preceding claim, in which R^h is H or in which R^h is methyl.

7. A compound according to any preceding claim, in which:

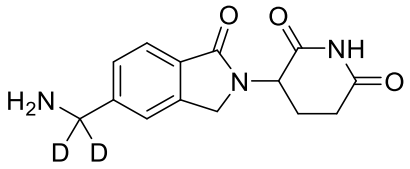
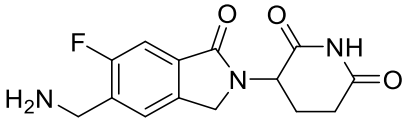
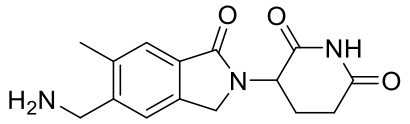
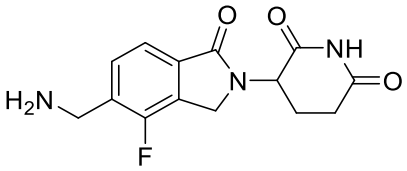
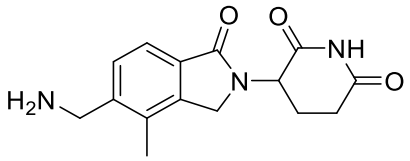
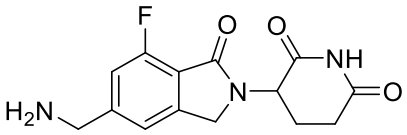
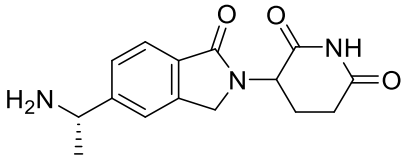
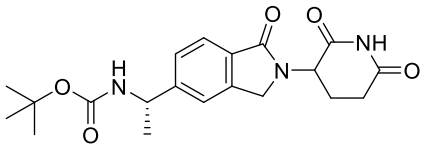
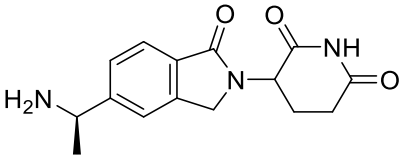
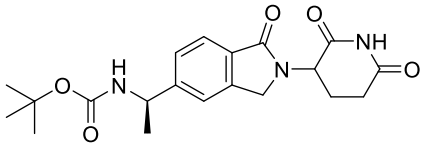
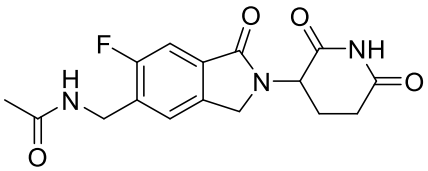
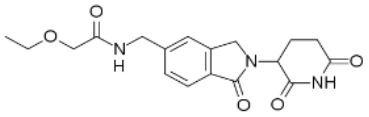
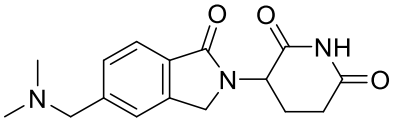
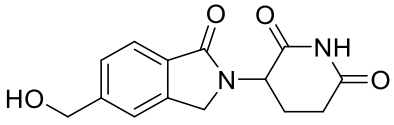
(a) R^a and R^b are each H; or

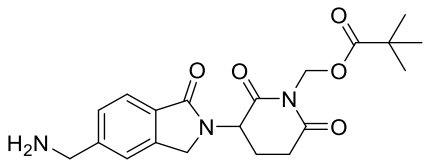
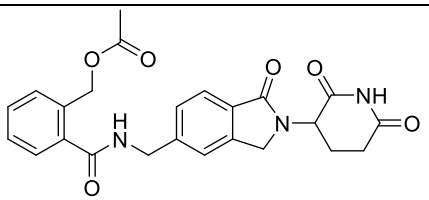
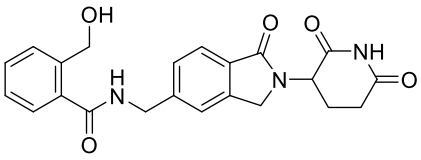
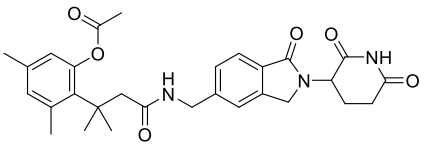
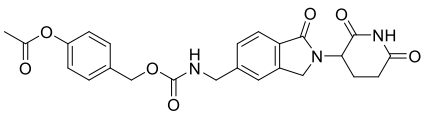
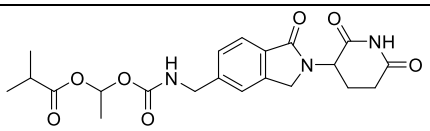
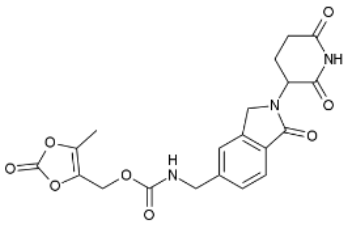
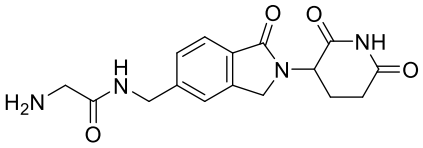
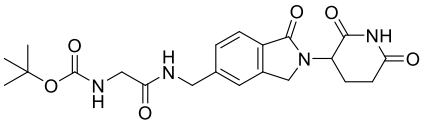
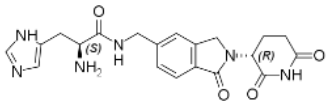
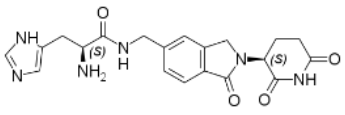
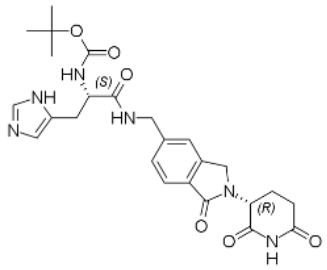
(b) R^a and R^b are each deuterium; or

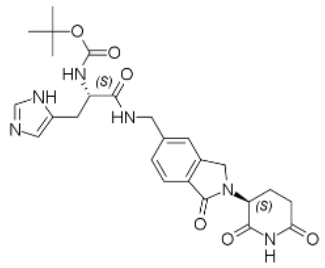
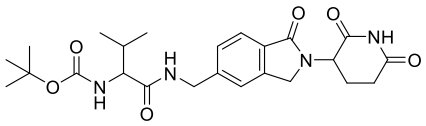
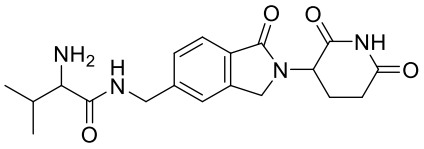
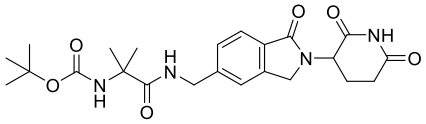
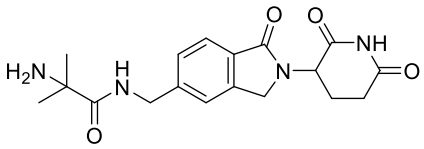
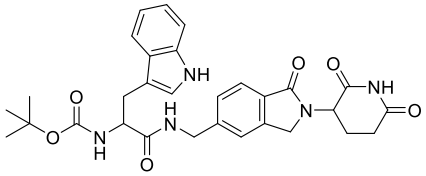
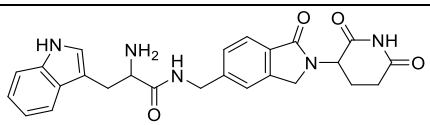
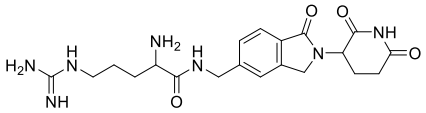
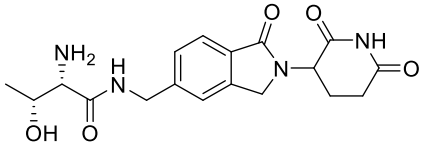
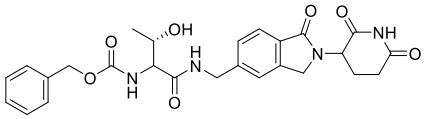
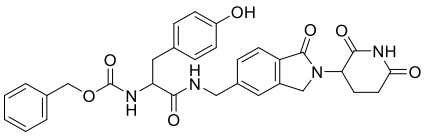
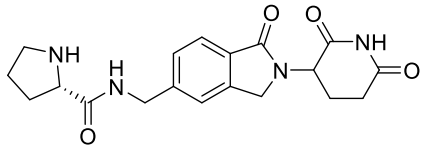
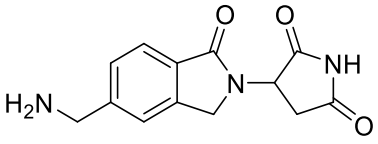
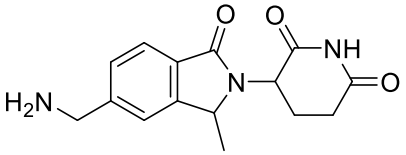
(c) R^a is H and R^b is methyl.

8. A compound according to any preceding claim, in which R^c is selected from NHR^2, and OH; optionally in which R^c is NHR^2.

9. A compound according to any preceding claim, in which the compound is selected from:

Compound ID	Structure
2	
3	
4	
5	
6	
7	
8	
9	
10	
11	
13	
14	
16	
17	

18		19	
20		21	
22		23	
24		25	
26		27	
28		29	

30		31	
32		33	
34		35	
37		38	
39		40	
41		43	
50		52	

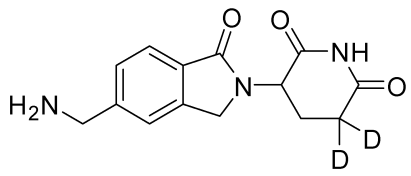
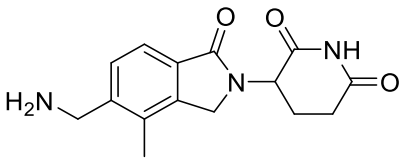
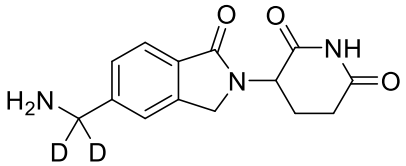
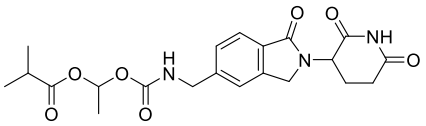
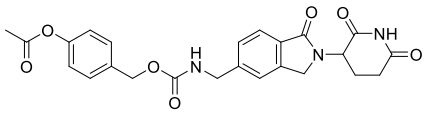
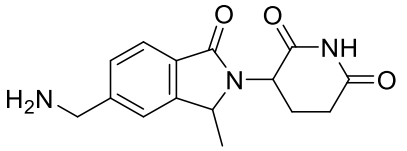
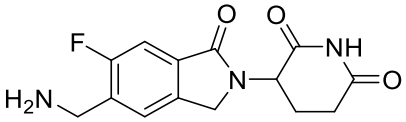
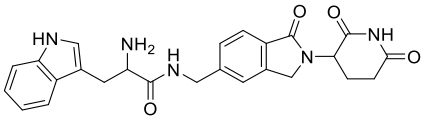
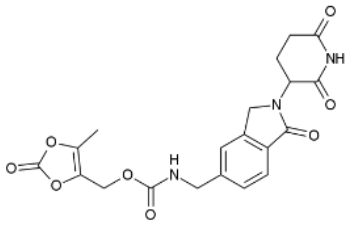
and pharmaceutically acceptable salts, esters, optically active isomers, racemates, solvates, amino acid conjugates, or prodrugs thereof.

10. A compound according to any preceding claim, in which R^2 is selected from H, $–COR^3$, and $–COOR^3$.

11. A compound according to any preceding claim, in which R^3 is C_1-C_{10} alkyl substituted with one or more R^4.

12. A compound according to any preceding claim, in which each R^4 is independently selected from NH_2, $OCOR^5$, substituted or unsubstituted dioxolyl, indole, and 6-membered aryl substituted with one or more -OCO(C_1-C_4 alkyl); in which R^4 is not X.

13. A compound according to any preceding claim, in which the compound is selected from compounds 51, 2, 22, 3, 24, 6, 23, 52, and 37.

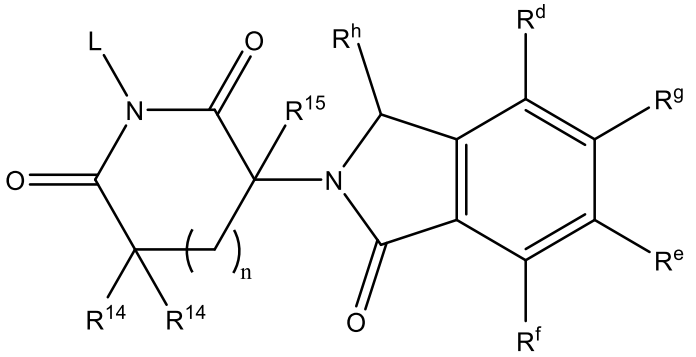
51			6	
2			23	
22			52	
3			37	
24				

and pharmaceutically acceptable salts, esters, optically active isomers, racemates, solvates, amino acid conjugates, or prodrugs thereof.

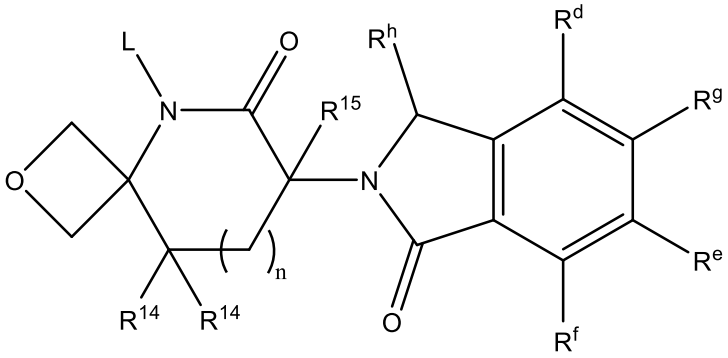
14. A pharmaceutical composition comprising a compound according to any preceding claim.

15. A compound for use in a method of treating cancer, in which the compound is to be administered to a subject in need thereof, in which the compound is:

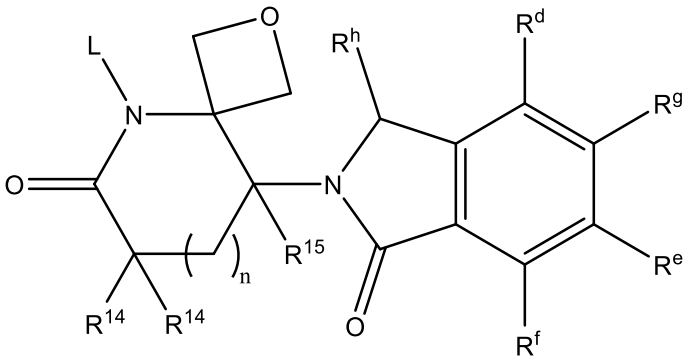
(i) a compound of formula (Ia), (Ib) or (Ic):



(Ia)



(Ib)



(Ic)

or a pharmaceutically acceptable salt, ester, optically active isomer, racemate, solvate, amino acid conjugate, or prodrug thereof,

in which

L is selected from hydrogen, alkyl, alkenyl, benzyl, aryl, heteroaryl, haloalkyl, haloalkenyl, $-CH_2OC(O)^tBu$, $-CH_2C(O)OR''$, $-C(O)R''$, $-C(O)OR''$, $-C(O)NH_2$, $-C(O)NHR''$, $-C(O)NR''_2$, $-OR''$, $-NR''_2$, $-S(O)_2R''$ or P(O)(OR")(OR");

each R'' is independently selected from hydrogen, alkyl, cycloalkyl, alkenyl, cycloalkenyl, aryl, heteroaryl, or benzyl;

each R^{14} is independently selected from deuterium and hydrogen;

R^{15} is selected from hydrogen, deuterium and C_1-C_4 alkyl;

R^g is selected from -COOH and $CR^aR^bR^c$,

R^h is selected from H and C_1-C_4 alkyl;

R^a is selected from H, deuterium, and C_1-C_4 alkyl;

R^b is selected from H, deuterium, and C_1-C_4 alkyl;

R^c is selected from NR^1R^2, OH, OR^6, CH_2X, CHX_2, and CX_3;

each of R^d, R^e and R^f is independently selected from H, deuterium, X, C_1-C_4 alkyl, and NH_2;

n is 0, 1 or 2;

X is selected from F, Cl, Br and I;

R^1 is selected from H and C_1-C_4 alkyl,

R^2 is selected from H, C_1-C_4 alkyl, $–COR^3$, and $–COOR^3$,

or alternatively R^1 and R^2, together with the nitrogen atom to which they are attached, form an 5 or 6-membered heterocycle, in which the heterocycle is unsubstituted or in which one or more carbon atoms of the heterocycle form part of a carbonyl group; or R^1 and R^a, together with the carbon and nitrogen atoms to which they are attached, form a 5 or 6-membered heterocycle;

R^3 is selected from:

unsubstituted C_1-C_4 alkyl;

C_1-C_{10} alkyl substituted with one or more R^4, in which each R^4 is independently selected from NH_2, $NHC(NH)NH_2$, $NHCOR^5$, $NHCOOR^5$, -OH, OR^5, $OCOR^5$, unsubstituted 5-membered heterocyclyl, substituted or unsubstituted dioxolyl, unsubstituted 6-membered heterocyclyl, 5-membered heteroaryl, 6-membered heteroaryl, indole, and 6-membered aryl substituted with one or more substituents independently selected from C_1-C_4 alkyl, OH, $-CH_2$-OH, $-OCO(C_1$-C_4 alkyl) and $CH_2OCO(C_1$-C_4 alkyl); and in which R^4 is not X;

C_2-C_{10} alkyl substituted with a halophenyl group;

6-membered aryl substituted with one or more substituents independently selected from CH_2-OH or $CH_2OCO(C_1$-C_4 alkyl); and

unsubstituted 5- or 6-membered heterocyclyl

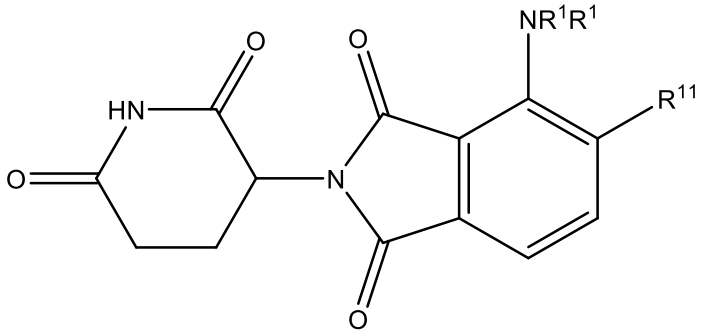
R^5 is unsubstituted C_1-C_6 alkyl; or C_1-C_6 alkyl substituted with one or more substituents independently selected from 5-membered heterocyclyl, 6-membered heterocyclyl, 5-membered aryl, 6-membered aryl, 5-membered heteroaryl and 6-membered heteroaryl; and

R^6 is unsubstituted cyclopentyl or cyclohexyl; or cyclopentyl or cyclohexyl substituted with one or more NH_2;

in which when R^a, R^b and R^1 are each H and R^2 is H or -COR^3, then n is 0 or 1;

or

(ii) a compound of formula (II):



(II)

or a pharmaceutically acceptable salt, ester, optically active isomer, racemate, solvate, amino acid conjugate, or prodrug thereof,

in which:

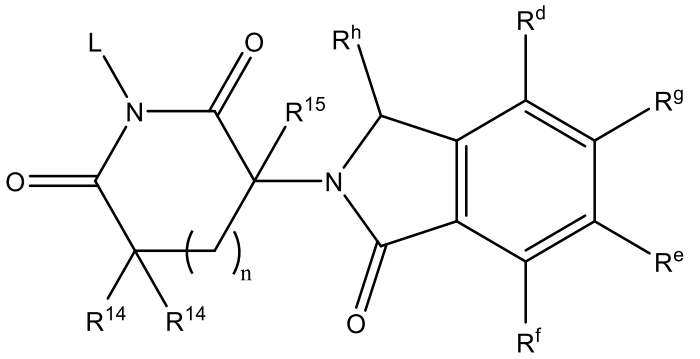
each R^1 is independently selected from H and C_1-C_4 alkyl;

R^{11} is OH or OR^{5a}; and

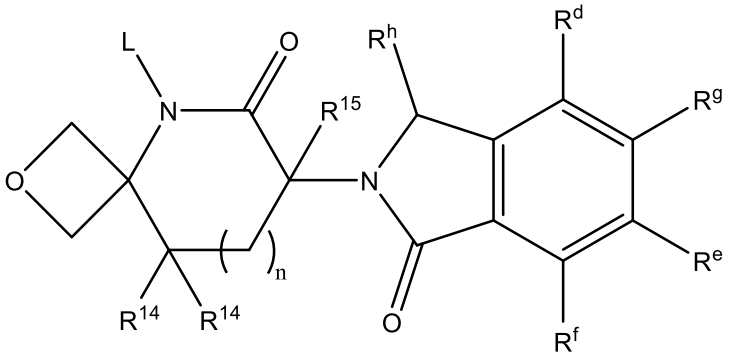
R^{5a} is unsubstituted C_1-C_6 alkyl; or C_1-C_6 alkyl substituted with one or more substituents independently selected from 5-membered heterocyclyl, 6-membered heterocyclyl, 5-membered heteroaryl and 6-membered heteroaryl.

16. A pharmaceutical composition for use in a method of treating cancer, in which the pharmaceutical composition is to be administered to a subject in need thereof, in which the pharmaceutical composition comprises:

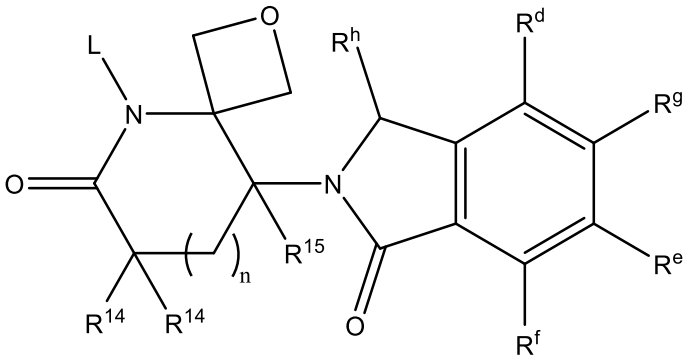
(i) a compound of formula (Ia), (Ib) or (Ic):



(Ia)



(Ib)



(Ic)

or a pharmaceutically acceptable salt, ester, optically active isomer, racemate, solvate, amino acid conjugate, or prodrug thereof,

in which

L is selected from hydrogen, alkyl, alkenyl, benzyl, aryl, heteroaryl, haloalkyl, haloalkenyl, -$CH_2OC(O)^tBu$, -$CH_2C(O)OR''$, -$C(O)R''$, -$C(O)OR''$, -$C(O)NH_2$, -$C(O)NHR''$, -$C(O)NR''_2$, -OR'', -NR''_2, -$S(O)_2R''$ or P(O)(OR")(OR");

each R'' is independently selected from hydrogen, alkyl, cycloalkyl, alkenyl, cycloalkenyl, aryl, heteroaryl, or benzyl;

each R^{14} is independently selected from deuterium and hydrogen;

R^{15} is selected from hydrogen, deuterium and C_1-C_4 alkyl;

R^g is selected from -COOH and $CR^aR^bR^c$,

R^h is selected from H and C_1-C_4 alkyl;

R^a is selected from H, deuterium, and C_1-C_4 alkyl;

R^b is selected from H, deuterium, and C_1-C_4 alkyl;

R^c is selected from NR^1R^2, OH, OR^6, CH_2X, CHX_2, and CX_3;

each of R^d, R^e and R^f is independently selected from H, deuterium, X, C_1-C_4 alkyl, and NH_2;

n is 0, 1 or 2;

X is selected from F, Cl, Br and I;

R^1 is selected from H and C_1-C_4 alkyl,

R^2 is selected from H, C_1-C_4 alkyl, –COR^3, and –$COOR^3$,

or alternatively R^1 and R^2, together with the nitrogen atom to which they are attached, form an 5 or 6-membered heterocycle, in which the heterocycle is unsubstituted or in which one or more carbon atoms of the heterocycle form part of a carbonyl group; or R^1 and R^a, together with the carbon and nitrogen atoms to which they are attached, form a 5 or 6-membered heterocycle;

R^3 is selected from:

unsubstituted C_1-C_4 alkyl;

C_1-C_{10} alkyl substituted with one or more R^4, in which each R^4 is independently selected from NH_2, $NHC(NH)NH_2$, $NHCOR^5$, $NHCOOR^5$, OH, OR^5, OCOR, unsubstituted 5-membered heterocyclyl, substituted or unsubstituted dioxolyl,

unsubstituted 6-membered heterocyclyl, 5-membered heteroaryl, 6-membered heteroaryl, indole, and 6-membered aryl substituted with one or more substituents independently selected from C_1-C_4 alkyl, -OH, -CH_2-OH, -OCO(C_1-C_4 alkyl) and $CH_2OCO(C_1$-C_4 alkyl); and in which R^4 is not X;

C_2-C_{10} alkyl substituted with a halophenyl group;

6-membered aryl substituted with one or more substituents independently selected from CH_2-OH or $CH_2OCO(C_1$-C_4 alkyl); and

unsubstituted 5- or 6-membered heterocyclyl;

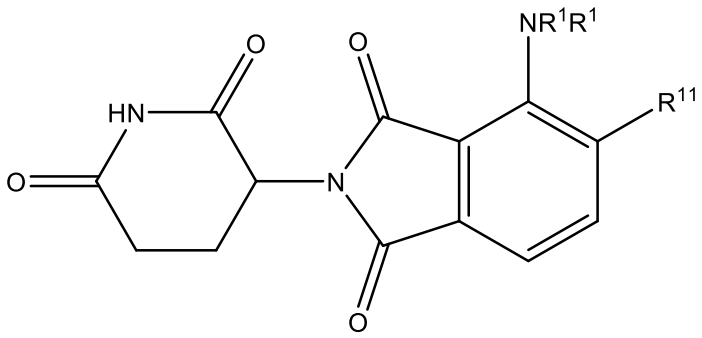
R^5 is unsubstituted C_1-C_6 alkyl; or C_1-C_6 alkyl substituted with one or more substituents independently selected from 5-membered heterocyclyl, 6-membered heterocyclyl, 5-membered aryl, 6-membered aryl, 5-membered heteroaryl and 6-membered heteroaryl; and

R^6 is unsubstituted cyclopentyl or cyclohexyl; or cyclopentyl or cyclohexyl substituted with one or more NH_2;

in which when R^a, R^b and R^1 are each H and R^2 is H or -COR^3 , then n is 0 or 1;

or

(ii) a compound of formula (II):



(II)

or a pharmaceutically acceptable salt, ester, optically active isomer, racemate, solvate, amino acid conjugate, or prodrug thereof,

in which:

each R^1 is independently selected from H and C_1-C_4 alkyl;

R^{11} is OH or OR^{5a}; and

R^{5a} is unsubstituted C_1-C_6 alkyl; or C_1-C_6 alkyl substituted with one or more substituents independently selected from 5-membered heterocyclyl, 6-membered heterocyclyl, 5-membered heteroaryl and 6-membered heteroaryl.

17. A compound or pharmaceutical composition for use according to Claim 15 or 16, in which R^3 is selected from:

unsubstituted C_1-C_4 alkyl;

C_1-C_{10} alkyl substituted with one or more R^4, in which each R^4 is independently selected from NH_2, $NHC(NH)NH_2$, $NHCOR^5$, $NHCOOR^5$, -OH, OR^5, $OCOR^5$, unsubstituted 5-membered heterocyclyl, substituted or unsubstituted dioxolyl, unsubstituted 6-membered heterocyclyl, 5-membered heteroaryl, 6-membered heteroaryl, indole, and 6-membered aryl substituted with one or more substituents independently selected from C_1-C_4 alkyl, OH, -CH_2-OH, -OCO(C_1-C_4 alkyl) and CH_2OCO(C_1-C_4 alkyl); and in which R^4 is not X;

6-membered aryl substituted with one or more substituents independently selected from CH_2-OH or CH_2OCO(C_1-C_4 alkyl); and

unsubstituted 5- or 6-membered heterocyclyl; optionally in which when the C_1-C_{10} alkyl is substituted with indole, the C_1-C_{10} alkyl is further substituted with at least one additional R^4.

18. A compound or pharmaceutical composition for use according to Claim 15, 16 or 17, in which R^{11} is OH.

19. A compound or pharmaceutical composition for use according to any of Claims 15 to 18, in which NR^1R^1 is NH_2.

20. A compound or pharmaceutical composition for use according to any of Claims 15 to 19, in which R^h is H.

21. A compound or pharmaceutical composition for use according to any of Claims 15 to 20, in which:

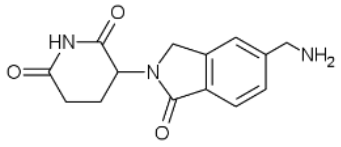
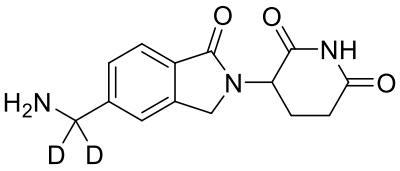
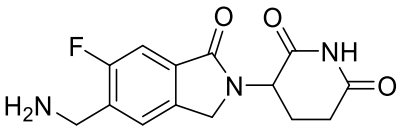
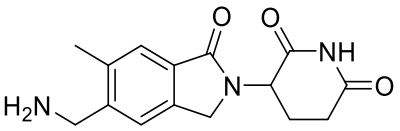
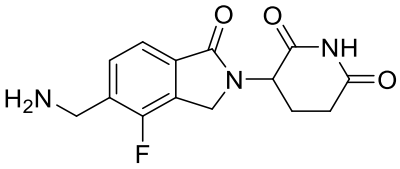
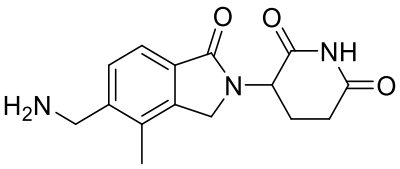
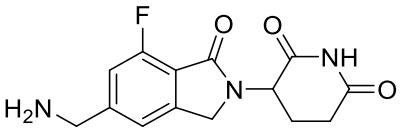
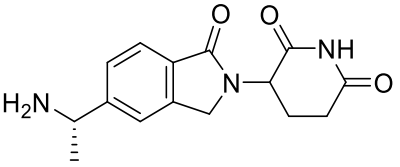
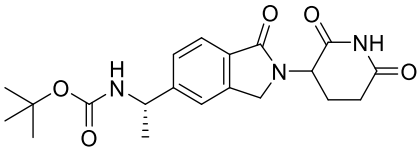
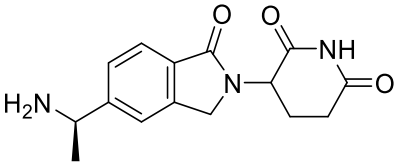
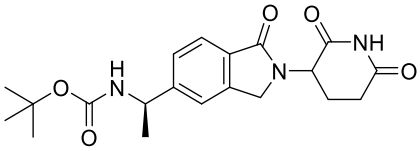
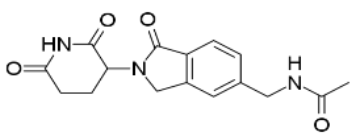
(a) R^a and R^b are each H; or

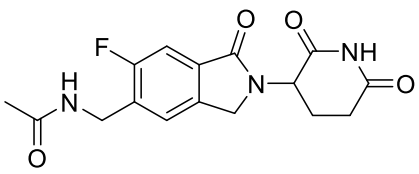
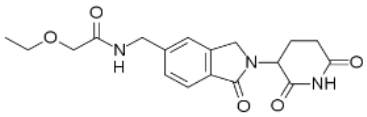
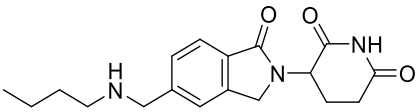
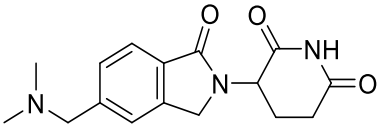
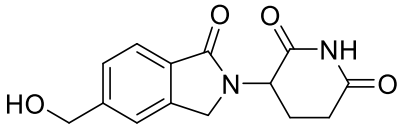
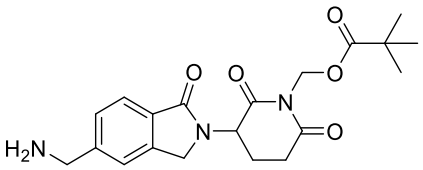
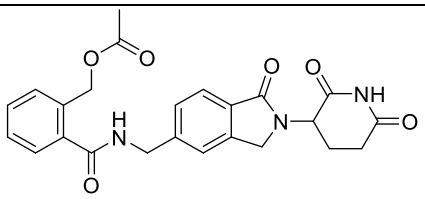
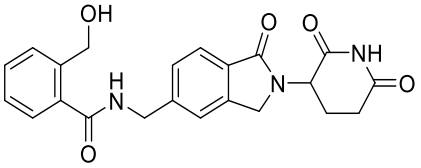
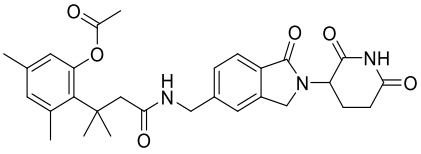
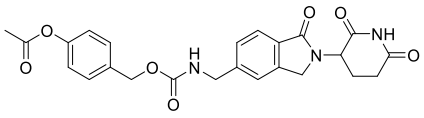
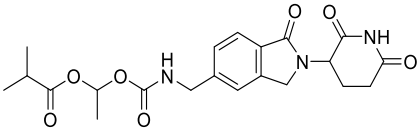
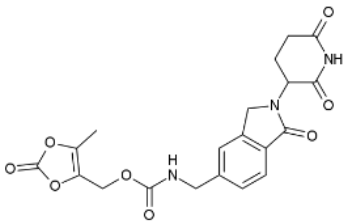
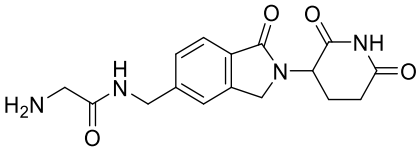
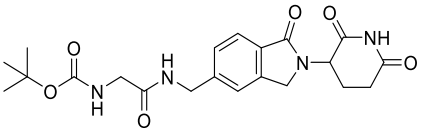
(b) R^a and R^b are each deuterium; or

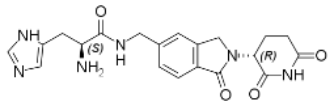
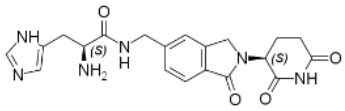
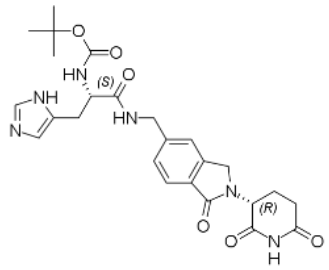
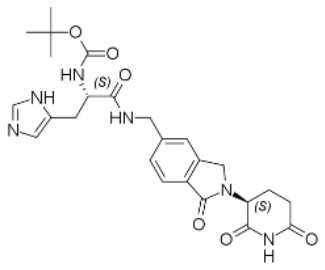
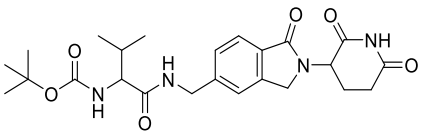
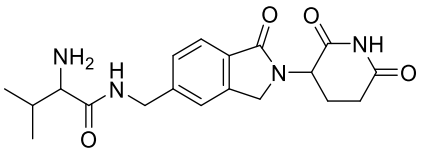
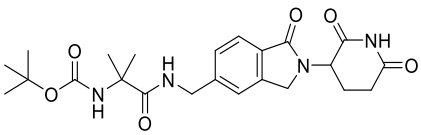
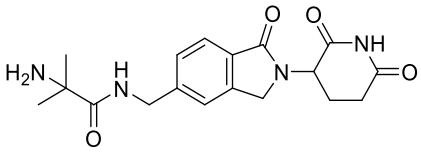
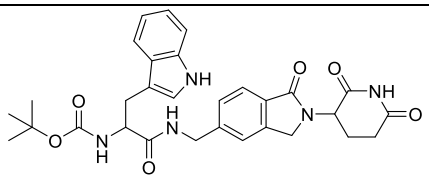
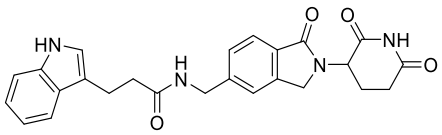
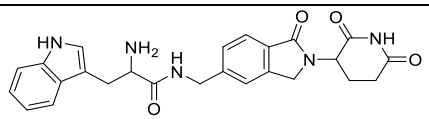
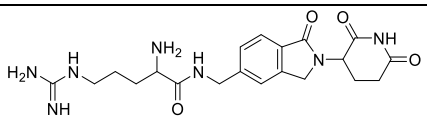
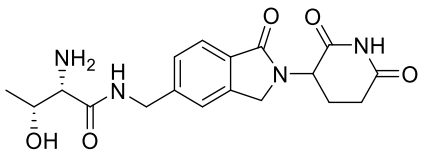
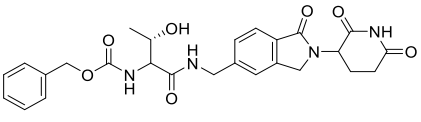
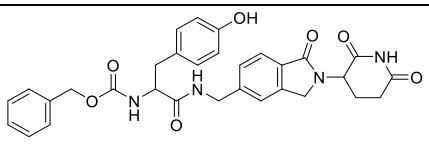
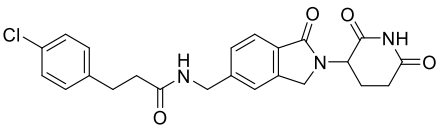
(c) R^a is H and R^b is methyl.

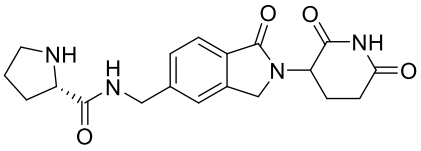
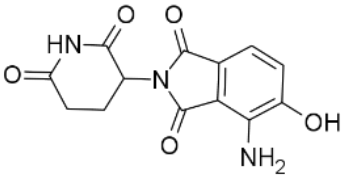
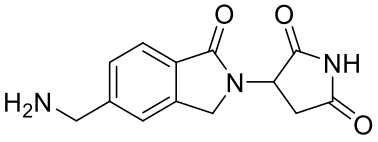
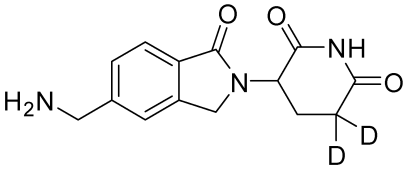
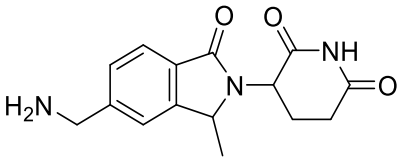
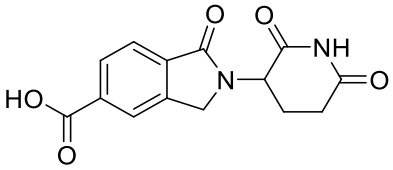
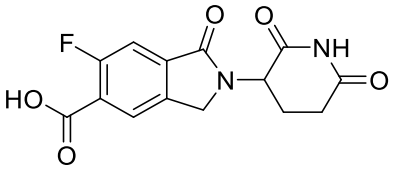
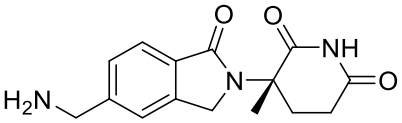
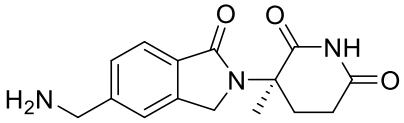
22. A compound or pharmaceutical composition for use according to any of Claims 15 to 21, in which R^c is selected from NHR^2 and OH, optionally in which R^c is NHR^2.

23. A compound or pharmaceutical composition for use according to any of Claims 15 to 22, in which the compound is selected from:

Compound ID	Structure	Compound ID	Structure
1		2	
3		4	
5		6	
7		8	
9		10	
11		12	

13		14	
15		16	
17		18	
19		20	
21		22	
23		24	
25		26	

27		28	
29		30	
31		32	
33		34	
35		36	
37		38	
39		40	
41		42	

43		44	
50		51	
52		53	
54		48	
49			

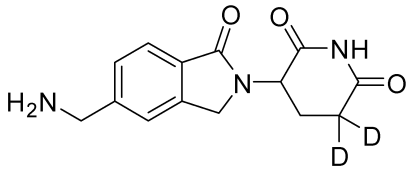
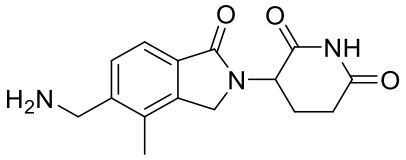
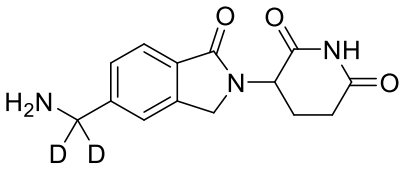
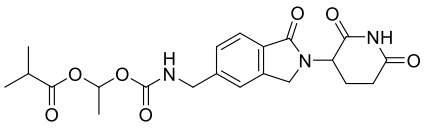
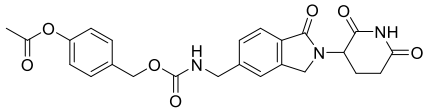
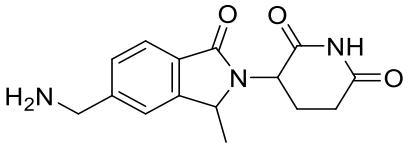
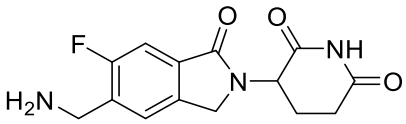
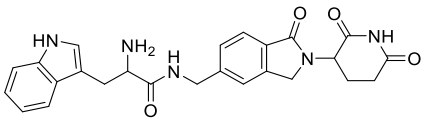
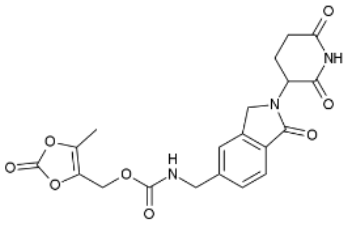
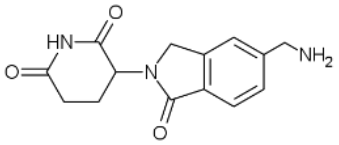
and pharmaceutically acceptable salts, esters, optically active isomers, racemates, solvates, amino acid conjugates, or prodrugs thereof.

24. A compound or pharmaceutical composition for use according to any of Claims 15 to 23 in which R^2 is selected from H, –COR^3, and –$COOR^3$.

25. A compound or pharmaceutical composition for use according to any of Claims 15 to 24, in which R^3 is C_1-C_{10} alkyl substituted with one or more R^4.

26. A compound or pharmaceutical composition for use according to any of Claims 15 to 25, in which each R^4 is independently selected from NH_2, $OCOR^5$, indole, and 6-membered aryl substituted with one or more -OCO(C_1-C_4 alkyl); in which R^4 is not X.

27. A compound or pharmaceutical composition for use according to any of Claims 15 to 26, in which the compound is selected from compounds 51, 2, 22, 3, 24, 6, 23, 52, 37, and 1:

51			6	
2			23	
22			52	
3			37	
24			1	

28. A compound or pharmaceutical composition for use according to any of Claims 15 to 27, in which the cancer is associated with one or more proteins selected from the group consisting of SALL4 or GSPT1.

29. A compound or pharmaceutical composition for use according to any of Claims 15 to 28, in which the cancer is hepatocellular carcinoma, neuroblastoma, leukemia, acute myeloid leukemia (AML), acute promyelocytic leukemia (APL), multiple myeloma, breast cancer, prostate cancer , bladder cancer, kidney cancer, muscle cancer, ovary cancer, skin cancer, pancreas cancer, breast cancer, colon cancer, hematological cancer, cancer of a connective tissue, placenta cancer, bone cancer, uterus cancer, cervical cancer, choriocarcinoma, endometrial cancer, gastric cancer, or lung cancer; optionally in which the cancer is hepatocellular carcinoma, neuroblastoma, leukemia, prostate cancer, or multiple myeloma.

30. A compound or pharmaceutical composition for use according to Claim 29, in which the cancer is:

(a) hepatocellular carcinoma; optionally in which the compound is:

(i) selected from compounds 6, 3, 36, 42, 26, 23, 24, 1, 52, 28, 27, 37, 39, 38, and 5; or

(ii) selected from compounds 6, 3, 36, 42, 26, 23, 24, 1, and 52;

or

(b) neuroblastoma; optionally in which the compound is selected from compounds 3, 36, 42, 37, 28, 27, and 1; or

(c) leukemia; optionally in which the compound is selected from compounds 3, 36, 42, 37, 28, 27, 24, and 1.

31. A compound or pharmaceutical composition for use according to any of Claims 15 to 30, in which a second cancer therapy is to be administered to the subject;

optionally in which the second cancer therapy is chemotherapy, radiotherapy or immunotherapy;

further optionally in which the second agent is selected from a therapeutic antibody that specifically binds to a cancer antigen, a hematopoietic growth factor, a cytokine, anti-cancer agent, an antibiotic, a cox-2 inhibitor, an immunomodulatory agent, an immunosuppressive agent, a corticosteroid or a pharmacologically active mutant or derivative thereof.

32. A compound of pharmaceutical composition for use according to any of Claims 15 to 31, in which the compound or the pharmaceutical composition are to be orally administered to the subject.

33. A compound or pharmaceutical composition according to any of Claims 1 to 14, or a compound or pharmaceutical composition for use according to any of Claims 15 to 32, in which:

(a) the compound is of formula (Ia) or formula (II);

(b) the compound is of formula (Ib);

(c) the compound is of formula (Ic);

(d) the compound is of formula (Ia) or formula (Ic), or the compound is of formula (II).

34. A compound, pharmaceutical composition, compound for use or pharmaceutical composition for use according to any preceding claim, in which L is selected from hydrogen, alkyl, alkenyl, benzyl, aryl, heteroaryl, haloalkyl, haloalkenyl, -$CH_2OC(O)^tBu$, -$CH_2C(O)OR''$, -$C(O)R''$, -$C(O)OR''$, -$C(O)NH_2$, -$C(O)NHR''$, -$C(O)NR''_2$, -OR'', -NR''_2, -$S(O)_2R''$; optionally in which L is alkyl, benzyl, -$CH_2OC(O)Me$, or -$CH_2OC(O)^tBu$, or in which L is hydrogen.

35. A compound, pharmaceutical composition, compound for use or pharmaceutical composition for use according to any preceding claim, in which:

(a) n is 1; or

(b) n is 0.

36. A compound, pharmaceutical composition, compound for use or pharmaceutical composition for use according to any preceding claim, in which:

(a) each R^{14} is deuterium; or

(b) each R^{14} is hydrogen.

37. A compound, pharmaceutical composition, compound for use or pharmaceutical composition for use according to any preceding claim, in which:

(a) R^{15} is deuterium; or

(b) R^{15} is hydrogen.

38. A compound, pharmaceutical composition, compound for use or pharmaceutical composition for use according to any preceding claim, in which R^e is X.

39. A compound, pharmaceutical composition, compound for use or pharmaceutical composition for use according to any preceding claim, in which R^1 is selected from H and methyl.

40. A compound, pharmaceutical composition, compound for use or pharmaceutical composition for use according to any preceding claim, in which R^2 is selected from H, methyl, $–COR^3$, and $–COOR^3$.

41. A compound, pharmaceutical composition, compound for use or pharmaceutical composition for use according to any preceding claim, in which administration of the compound or pharmaceutical composition to a subject reduces levels of a target protein in the subject; optionally in which the target protein is selected from SALL-4 or GSPT1; optionally in which administration of the compound or pharmaceutical composition to the subject induces minimal reduction or substantially no reduction of IKZF1 or IKZF3 protein levels.