Claims

1. A pharmaceutical formulation comprising (or consisting essentially of) the following components:
   i) melflufen, or a salt thereof;
   ii) propylene glycol;
   iii) optionally one or more physiologically acceptable aqueous solvent(s); and
   iv) optionally one or more additional therapeutic agent(s).

2. The pharmaceutical formulation of claim 1, wherein the melflufen, or salt thereof, is at a concentration of about 10 mg/mL to about 50 mg/mL.

3. The pharmaceutical formulation of claim 3, wherein the melflufen, or salt thereof, is at a concentration of about 15 mg/mL to about 25 mg/mL.

4. A pharmaceutical formulation comprising (or consisting essentially of) the following components:
   i) melflufen, or a salt thereof;
   ii) polyethylene glycol;
   iii) optionally one or more physiologically acceptable aqueous solvent(s); and
   iv) optionally one or more additional therapeutic agent(s).

5. The pharmaceutical formulation of claim 4, wherein the melflufen, or salt thereof, is at a concentration of about 10 mg/mL to about 50 mg/mL.

6. The pharmaceutical formulation of claim 5, wherein the melflufen, or salt thereof, is at a concentration of about 15 mg/mL to about 25 mg/mL.

7. The pharmaceutical formulation of any one of claims 4 to 6, wherein the polyethylene glycol has an average molecular weight of 150 to 650 Da, for example 150 to 250, 250 to 350, 350 to 450, 450 to 550 and 550 to 650 Da.

8. The pharmaceutical formulation of claim 7, wherein the polyethylene glycol has an average molecular weight of 250 to 350 Da.

9. The pharmaceutical formulation of claim 8, wherein the polyethylene glycol is PEG 300.

10. The pharmaceutical formulation of any one of claims 1 to 3 consisting essentially of melflufen, or a salt thereof, propylene glycol, and optionally one or more additional therapeutic agent(s).

11. The pharmaceutical formulation of any one of claims 4 to 9 consisting essentially of melflufen, or a salt thereof, polyethylene glycol, and optionally one or more additional therapeutic agent(s).

12. The pharmaceutical formulation of any one of claims 1 to 9, wherein the one or more physiologically acceptable solution(s) is a glucose solution, a saline solution, or a mixture thereof.

13. The pharmaceutical formulation of any one of claims 1 to 12, wherein at least one of the one or more additional therapeutic agent(s) is a protease inhibitor (PI), an immunomodulatory drug (IMiD), or an alkylator.

14. The pharmaceutical formulation of any one of claims 1 to 13 for use as a medicament.

15. The pharmaceutical formulation of any one of claims 1 to 13 for use in treatment or prophylaxis of a disease or condition that may be treated with conventional chemotherapy agents, for example, with an alkylator (e.g. melphalan, cyclophosphamide and bendamustine).

16. The pharmaceutical formulation of any one of claims 1 to 13 for use in the treatment or prophylaxis of cancer or use in the treatment or prophylaxis of amyloidosis.

17. The pharmaceutical formulation for use of claim 16, for use in the treatment or prophylaxis of cancer wherein the cancer is selected from multiple myeloma, breast cancer, lung cancer, ovarian cancer, leukaemias and lymphomas.

18. A method for treating a patient which comprises administering a pharmaceutically effective amount of the pharmaceutical formulation of any one of claims 1 to 13 to a subject.

19. A method for the treatment or prophylaxis of cancer, comprising administering an effective amount of the pharmaceutical formulation of any one of claims 1 to 13 to a subject.

20. The method of claim 19, wherein the pharmaceutical formulation is directly administered to the subject.

21. A method for preparing the pharmaceutical formulation of any of claims 1 to 13 comprising dissolving melflufen, or a salt thereof, in propylene glycol.

22. A method for preparing the pharmaceutical formulation of any of claims 1 to 13 comprising dissolving melflufen, or a salt thereof, in polyethylene glycol.

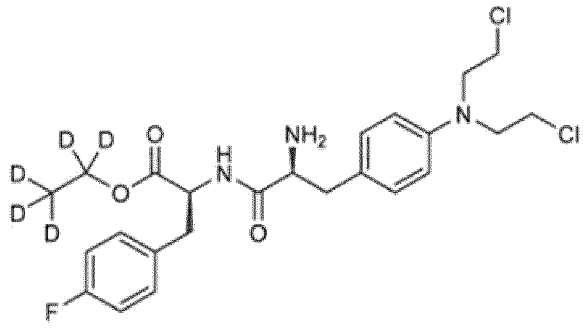
23. A method for storing melflufen, or a salt thereof, comprising the steps of preparing a pharmaceutical formulation according to any one of claims 1 to 13, and storing the pharmaceutical formulation at a temperature of about -90 to 25 °C, preferably at a temperature of about 5 °C (for example, 5 ± 3 °C) or less, for example about 0 to 4 °C, about -20 °C (for example, 20 ± 3 °C) and about -80 °C (for example, -80 ± 3 °C).

24. A kit comprising melflufen, or a salt thereof; propylene glycol; optionally one or more physiologically acceptable aqueous solvent(s); and optionally one or more additional therapeutic agent(s).

25. A kit comprising melflufen, or a salt thereof; polyethylene glycol; optionally one or more physiologically acceptable aqueous solvent(s); and optionally one or more additional therapeutic agent(s).

26. The pharmaceutical formulation of any one of claims 1 to 17, the method of any one of claims 18 to 23, or the kit of any one of claims 24 and 25, wherein the melflufen is in the form of its hydrochloride, ethanesulfonate, maleate or p-toluenesulfonate salt, preferably its hydrochloride salt.

27. The pharmaceutical formulation of any one of claims 1 to 17 or 26, the method of any one of claims 18 to 23 or 26, or the kit of any one of claims 24 to 26, wherein the melflufen is deuterated melflufen, for example the melflufen is OPD-5:


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CLAIMS

1. A pharmaceutical formulation comprising (or consisting essentially of) the following components:

(i) melflufen, or a salt thereof;

(ii) propylene glycol;

(iii) optionally one or more physiologically acceptable aqueous solvent(s); and

(iv) optionally one or more additional therapeutic agent(s)

in which the melflufen, or salt thereof, is at a concentration of about 1 mg/mL to about 50 mg/mL, and in which the formulation is substantially free from organic solvents that are not PEG or propylene glycol.

2. A pharmaceutical formulation according to Claim 1, in which the melflufen, or salt thereof, is at a concentration of about 10 mg/mL to about 50 mg/mL.

3. A pharmaceutical formulation according to Claim 2, in which the melflufen, or salt thereof, is at a concentration of about 15 mg/mL to about 25 mg/mL.

4. A pharmaceutical formulation comprising (or consisting essentially of) the following components:

(i) melflufen, or a salt thereof;

(ii) polyethylene glycol;

(iii) optionally one or more physiologically acceptable aqueous solvent(s); and

(iv) optionally one or more additional therapeutic agent(s)

in which the melflufen, or salt thereof, is at a concentration of about 1 mg/mL to about 50 mg/mL, and in which the formulation is substantially free from organic solvents that are not PEG or propylene glycol.

5. A pharmaceutical formulation according to Claim 4, in which the melflufen, or salt thereof, is at a concentration of about 10 mg/mL to about 50 mg/mL.

6. A pharmaceutical formulation according to Claim 5, in which the melflufen, or salt thereof, is at a concentration of about 15 mg/mL to about 25 mg/mL.

7. A pharmaceutical formulation according to Claim 4, 5 or 6, in which the polyethylene glycol has an average molecular weight of 150 to 650 Da, for example 150 to 250, 250 to 350, 350 to 450, 450 to 550 and 550 to 650 Da.

8. A pharmaceutical formulation according to Claim 7, in which the polyethylene glycol has an average molecular weight of 250 to 350 Da.

9. A pharmaceutical formulation according to Claim 8, in which the polyethylene glycol is PEG 300.

10. A pharmaceutical formulation according to Claim 1, 2 or 3, consisting essentially of melflufen, or a salt thereof, propylene glycol, and optionally one or more additional therapeutic agent(s).

11. A pharmaceutical formulation according to any of Claims 4 to 9 consisting essentially of melflufen, or a salt thereof, polyethylene glycol, and optionally one or more additional therapeutic agent(s).

12. A pharmaceutical formulation according to any of Claims 1 to 9, in which the one or more physiologically acceptable solution(s) is a glucose solution, a saline solution, or a mixture thereof.

13. A pharmaceutical formulation according to any preceding claim, in which at least one of the one or more additional therapeutic agent(s) is a protease inhibitor (PI), an immunomodulatory drug (IMiD), or an alkylator.

14. A pharmaceutical formulation according to any preceding claim for use as a medicament.

15. A pharmaceutical formulation according to any of Claims 1 to 13 for use in treatment or prophylaxis of a disease or condition that may be treated with conventional chemotherapy agents, for example, with an alkylator (e.g. melphalan, cyclophosphamide and bendamustine).

16. A pharmaceutical formulation according to any of Claims 1 to 13 for use in the treatment or prophylaxis of cancer or use in the treatment or prophylaxis of amyloidosis.

17. A pharmaceutical formulation according to Claim 16, for use in the treatment or prophylaxis of cancer in which the cancer is selected from multiple myeloma, breast cancer, lung cancer, ovarian cancer, leukaemias and lymphomas.

18. A method for preparing a pharmaceutical formulation according to any of Claims 1 to 13 comprising dissolving melflufen, or a salt thereof, in propylene glycol.

19. A method for preparing a pharmaceutical formulation according to any of Claims 1 to 13 comprising dissolving melflufen, or a salt thereof, in polyethylene glycol.

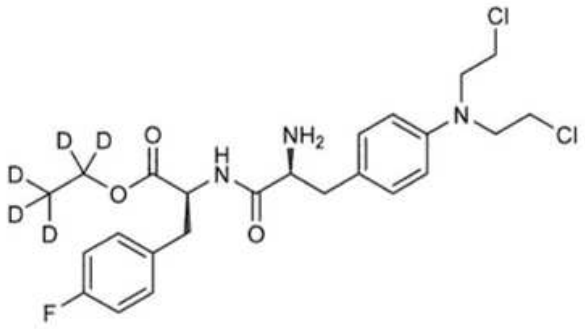
20. A method for storing melflufen, or a salt thereof, comprising the steps of preparing a pharmaceutical formulation according to any of Claims 1 to 13, and storing the pharmaceutical formulation at a temperature of about -90 to 25 ºC, preferably at a temperature of about 5 ºC (for example, 5 ± 3 ºC) or less, for example about 0 to 4 ºC, about -20 ºC (for example, 20 ± 3 ºC) and about -80 ºC (for example, -80 ± 3 ºC).

21. A kit comprising melflufen, or a salt thereof; propylene glycol; optionally one or more physiologically acceptable aqueous solvent(s); and optionally one or more additional therapeutic agent(s).

22. A kit comprising melflufen, or a salt thereof; polyethylene glycol; optionally one or more physiologically acceptable aqueous solvent(s); and optionally one or more additional therapeutic agent(s).

23. A pharmaceutical formulation according to any of Claims 1 to 17, a method according to any of Claims 16 to 20, or a kit according to any of Claims 21 and 22, in which the melflufen is in the form of its hydrochloride, ethanesulfonate, maleate or p-toluenesulfonate salt, preferably its hydrochloride salt.

24. A pharmaceutical formulation according to any of Claims 1 to 17 or 23, a method of any of Claims 16 to 18 or 21, or a kit according to Claim 19, 20 or 21, in which the melflufen is deuterated melflufen, for example the melflufen is OPD-5:


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