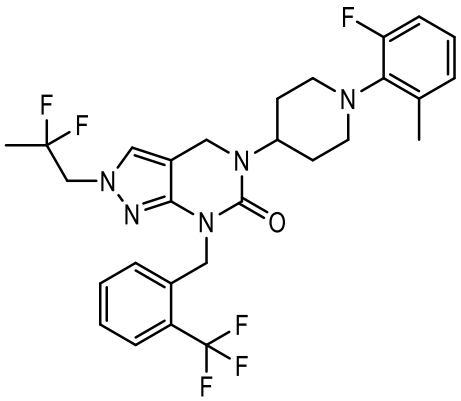
CLAIMS

1. A pharmaceutical composition which is a self-emulsifying drug delivery system (SEDDS), a self-microemulsifying drug delivery system (SMEDDS), or a self-nanoemulsifying drug delivery system (SNEDDS); said pharmaceutical composition comprising the compound 2-(2,2-difluoro-propyl)-5-[1-(2-fluoro-6-methyl-phenyl)-piperidin-4-yl]-7-(2-trifluoromethyl-benzyl)-2,4,5,7-tetrahydro-pyrazolo[3,4-d]pyrimidin-6-one:



;

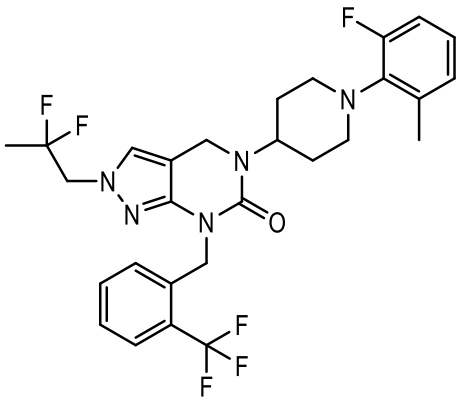
wherein said compound is in free base form, or in a pharmaceutically acceptable salt form;

wherein said pharmaceutical composition comprises a mixture of excipients comprising

- one or more lipophilic excipient(s);
- one or more hydrophilic surfactant(s); and
- optionally one or more hydrophilic co-solvent(s).

2. A pharmaceutical composition according to claim 1; comprising

- the compound 2-(2,2-Difluoro-propyl)-5-[1-(2-fluoro-6-methyl-phenyl)-piperidin-4-yl]-7-(2-trifluoromethyl-benzyl)-2,4,5,7-tetrahydro-pyrazolo[3,4-d]pyrimidin-6-one:



;

wherein said compound is in free base form, or in a pharmaceutically acceptable salt form;

and

- a mixture of excipients comprising:
  - a total of about 20 to 50 ww% of one or more lipophilic excipient(s), wherein said lipophilic excipient(s) is/are independently selected from
    - hydrophobic surfactants selected from 1,2-propandiol medium chain mono-fatty acid esters and glycerin medium chain mono-/di-fatty acid esters; and/or
    - oil-like excipients selected from medium chain triglyceride oils and 1,2-propandiol medium chain di-fatty acid esters;
  - a total of about 30 to 80 ww% of one or more hydrophilic surfactant(s), wherein said hydrophilic surfactant(s) is/are independently selected from polyethyleneglycol derivatized long chain lipids and polyethyleneglycol derivatized glycerin medium chain mono-/di-fatty acid esters; and
  - a total of about 0 to 25 ww% of one or more hydrophilic co-solvents;

  wherein the total ww% of said mixture of excipients is 100.

3. A pharmaceutical composition according to claim 1, comprising

- a total amount of about 0.05 to 5 ww% of 2-(2,2-Difluoro-propyl)-5-[1-(2-fluoro-6-methyl-phenyl)-piperidin-4-yl]-7-(2-trifluoromethyl-benzyl)-2,4,5,7-tetrahydro-pyrazolo[3,4-d]pyrimidin-6-one in free base form; and
- a total amount of at least about 80 ww% of a mixture of excipients; wherein said mixture of excipients comprises:
  - a total of about 20 to 50 ww% of a lipophilic excipient, wherein said lipophilic excipient is a 1,2-propandiol medium chain mono-fatty acid ester;
    a total of about 30 to 80 ww% of a hydrophilic surfactant, wherein said hydrophilic surfactant is a polyethyleneglycol derivatized hydrogenated castor oil; and
    a total of about 0 to 25 ww% of one or two hydrophilic co-solvents;
  - a total of about 20 to 50 ww% of a lipophilic excipient, wherein said lipophilic excipient is a 1,2-propandiol medium chain mono-fatty acid ester;
    a total of about 30 to 80 ww% of a hydrophilic surfactant, wherein said hydrophilic surfactant is a polyethyleneglycol derivatized castor oil; and
    a total of about 0 to 25 ww% of one or two hydrophilic co-solvents;
  - a total of about 20 to 50 ww% of a lipophilic excipient, wherein said lipophilic excipient is a 1,2-propandiol medium chain mono-fatty acid ester;
    a total of about 30 to 80 ww% of a hydrophilic surfactant, wherein said hydrophilic surfactant is a polyethyleneglycol derivatized glycerin medium chain mono-/di-fatty acid ester; and
    a total of about 0 to 25 ww% of one or two hydrophilic co-solvents;
  - a total of about 20 to 50 ww% of a lipophilic excipient, wherein said lipophilic excipient is a glycerin medium chain tri-fatty acid ester;

a total of about 30 to 80 ww% of a hydrophilic surfactant, wherein said hydrophilic surfactant is a polyethyleneglycol derivatized hydrogenated castor oil; and

a total of about 0 to 25 ww% of one or two hydrophilic co-solvents;

- a total of about 20 to 50 ww% of a lipophilic excipient, wherein said lipophilic excipient is a glycerin medium chain tri-fatty acid ester;

  a total of about 30 to 80 ww% of a hydrophilic surfactant, wherein said hydrophilic surfactant is a polyethyleneglycol derivatized castor oil; and

  a total of about 0 to 25 ww% of one or two hydrophilic co-solvents; or
- a total of about 20 to 50 ww% of a lipophilic excipient, wherein said lipophilic excipient is 1,2-propandiol medium chain di-fatty acid ester;

  a total of about 30 to 80 ww% of a hydrophilic surfactant, wherein said hydrophilic surfactant is a polyethyleneglycol derivatized castor oil; and

  a total of about 0 to 25 ww% of one or two hydrophilic co-solvents;

wherein the total ww% of said mixture of excipients is 100; and

wherein the total ww% of the pharmaceutical composition is 100.

4. A pharmaceutical composition according to claim 1, comprising

- a total amount of about 0.075 to 4.5 ww% of 2-(2,2-Difluoro-propyl)-5-[1-(2-fluoro-6-methyl-phenyl)-piperidin-4-yl]-7-(2-trifluoromethyl-benzyl)-2,4,5,7-tetrahydro-pyrazolo[3,4-d]pyrimidin-6-one in free base form; and
- a total amount of at least about 80 ww% of a mixture of excipients; wherein said mixture of excipients comprises:
  - a total of about 20 to 40 ww% of a lipophilic excipient, wherein said lipophilic excipient is a 1,2-propandiol medium chain mono-fatty acid ester;
  - a total of about 40 to 70 ww% of a hydrophilic surfactant, wherein said hydrophilic surfactant is a polyethyleneglycol derivatized hydrogenated castor oil; and
  - no hydrophilic co-solvent; or a total of about 10 to 20 ww% of one or two hydrophilic co-solvents selected from triethyl citrate, ethanol, and diethylene glycol monoethylether;

  wherein the total ww% of said mixture of excipients is 100; and

wherein the total ww% of the pharmaceutical composition is 100.

5. A pharmaceutical composition according to claim 1, consisting essentially of:

- a total amount of about 0.075 to 4.5 ww% of 2-(2,2-Difluoro-propyl)-5-[1-(2-fluoro-6-methyl-phenyl)-piperidin-4-yl]-7-(2-trifluoromethyl-benzyl)-2,4,5,7-tetrahydro-pyrazolo[3,4-d]pyrimidin-6-one in free base form; and
- a total amount of at least about 90 ww% based on the total weight of the pharmaceutical composition of a mixture of excipients; wherein said mixture of excipients comprises:
  - a total of about 20 to 40 ww% of a lipophilic excipient, wherein said lipophilic excipient is a 1,2-propandiol medium chain mono-fatty acid ester;
  - a total of about 40 to 70 ww% of a hydrophilic surfactant, wherein said hydrophilic surfactant is a polyethyleneglycol derivatized hydrogenated castor oil; and
  - no hydrophilic co-solvent; or a total of about 10 to 20 ww% of one or two hydrophilic co-solvents selected from triethyl citrate, ethanol, and diethylene glycol monoethylether;

  wherein the total ww% of said mixture of excipients is 100; and
- optionally one or two conventional ingredients or additives selected from one or two antioxidants selected from
  - one oxygen scavenger in an amount of below about 2 ww% based on the total weight of the pharmaceutical composition, and/or
  - one chain terminator in an amount of below about 0.3 ww% based on the total weight of the pharmaceutical composition;

wherein the total ww% of the pharmaceutical composition is 100.

6. A pharmaceutical composition according to claim 5, wherein said oxygen scavenger is present with respect to the total weight of the pharmaceutical composition in an amount of about 0.1 to 1 ww%.

7. A pharmaceutical composition according to claims 5 or 6, wherein said chain terminator is present with respect to the total weight of the pharmaceutical composition in an amount of about 0.05 to 0.2 ww%.

8. A pharmaceutical composition according any one of claims 1 to 7, wherein said pharmaceutical composition is filled into soft gelatine capsules.

9. A pharmaceutical composition according to any one of claims 1 to 8, wherein 2-(2,2-Difluoro-propyl)-5-[1-(2-fluoro-6-methyl-phenyl)-piperidin-4-yl]-7-(2-trifluoromethyl-benzyl)-2,4,5,7-tetrahydro-pyrazolo[3,4-d]pyrimidin-6-one in crystalline form is used for the preparation of said composition.

10. A crystalline form of 2-(2,2-Difluoro-propyl)-5-[1-(2-fluoro-6-methyl-phenyl)-piperidin-4-yl]-7-(2-trifluoromethyl-benzyl)-2,4,5,7-tetrahydro-pyrazolo[3,4-d]pyrimidin-6-one, characterized by the presence of peaks in the X-ray powder diffraction diagram at the following angles of refraction 2θ: 6.2°, 9.5°, and 14.4°; wherein said X-ray powder diffraction diagram is obtained by using combined Cu Kα1 and Kα2 radiation, without Kα2 stripping; and the accuracy of the 2θ values is in the range of 2θ +/- 0.2°.

11. A crystalline form of 2-(2,2-Difluoro-propyl)-5-[1-(2-fluoro-6-methyl-phenyl)-piperidin-4-yl]-7-(2-trifluoromethyl-benzyl)-2,4,5,7-tetrahydro-pyrazolo[3,4-d]pyrimidin-6-one according to claim 10, characterized by the presence of peaks in the X-ray powder diffraction diagram at the following angles of refraction 2θ: 6.2°, 9.5°, 14.4°, 15.7°, and 18.6°; wherein said X-ray powder diffraction diagram is obtained by using combined Cu Kα1 and Kα2 radiation, without Kα2 stripping; and the accuracy of the 2θ values is in the range of 2θ +/- 0.2°.

12. A crystalline form of 2-(2,2-Difluoro-propyl)-5-[1-(2-fluoro-6-methyl-phenyl)-piperidin-4-yl]-7-(2-trifluoromethyl-benzyl)-2,4,5,7-tetrahydro-pyrazolo[3,4-d]pyrimidin-6-one according to claim 11, which has a melting point of about 163°C as determined by differential scanning calorimetry.

13. A pharmaceutical composition according to any one of claims 1 to 9 for use as a medicament.

14. Use of a pharmaceutical composition according to any one of claims 1 to 9 in the manufacture of a medicament for the prevention / prophylaxis or treatment of diseases and disorders related to pathogenic events associated with elevated levels of C5a and/or with C5aR activation.

15. Use of a pharmaceutical composition according to any one of claims 1 to 9 in the manufacture of a medicament for the prevention / prophylaxis or treatment of diseases and disorders selected from vasculitic diseases or disorders, inflammatory diseases or disorders involving intravascular microvesicle release, immune complex (IC) diseases or disorders, neurodegenerative diseases or disorders, complement related inflammatory diseases or disorders, bullous diseases or disorders, diseases or disorders related to ischemia and/or ischemic reperfusion injury, inflammatory bowel diseases or disorders, autoimmune diseases or disorders, and cancer.