

What is claimed is:

1. Use of at least one compound chosen from (R)-2-[3-[4-amino-3-(2-fluoro-4-phenoxy-phenyl)pyrazolo[3,4-d]pyrimidin-1-yl]piperidine-1-carbonyl]-4-methyl-4-[4-(oxetan-3-yl)piperazin-1-yl]pent-2-enenitrile and pharmaceutically acceptable salts thereof in the manufacture of a medicament for treating immune thrombocytopenia (ITP) in a human patient in need thereof, wherein the medicament is to be administered to the human patient a therapeutically effective amount twice a day for a treatment period, wherein the human patient has a history of response to at least one prior line of therapy chosen from intravenous immunoglobulin (IVIG) and corticosteroids.
2. Use of at least one compound chosen from (R)-2-[3-[4-amino-3-(2-fluoro-4-phenoxy-phenyl)pyrazolo[3,4-d]pyrimidin-1-yl]piperidine-1-carbonyl]-4-methyl-4-[4-(oxetan-3-yl)piperazin-1-yl]pent-2-enenitrile and pharmaceutically acceptable salts thereof in the manufacture of a medicament for treating immune thrombocytopenia (ITP) in a human patient in need thereof, wherein the medicament is to be administered to the human patient a therapeutically effective amount twice a day for a treatment period, wherein the human patient in need thereof has not received prior treatment with a thrombopoietin receptor agonist (TPO-RA), and has at least one characteristic chosen from:
 - a. a platelet count of $<33,000/\mu\text{L}$ on two occasions no less than 7 days apart in the 15 days prior to beginning study treatment; and
 - b. a history of response (two or more platelet counts $\geq 50,000/\mu\text{L}$ with an increase of $\geq 20,000/\mu\text{L}$) to at least one prior line of therapy.
3. Use of at least one compound chosen from (R)-2-[3-[4-amino-3-(2-fluoro-4-phenoxy-phenyl)pyrazolo[3,4-d]pyrimidin-1-yl]piperidine-1-carbonyl]-4-methyl-4-[4-(oxetan-3-yl)piperazin-1-yl]pent-2-enenitrile and pharmaceutically acceptable salts thereof in the manufacture of a medicament for treating immune thrombocytopenia (ITP) in a human patient in need thereof, wherein the medicament is to be administered to the human patient a therapeutically effective amount twice a day for a treatment period, wherein the human patient in need thereof has not received prior treatment with rituximab, and has at least one characteristic chosen from:

- a. a platelet count of $<33,000/\mu\text{L}$ on two occasions no less than 7 days apart in the 15 days prior to beginning study treatment; and
 - b. a history of response (two or more platelet counts $\geq 50,000/\mu\text{L}$ with an increase of $\geq 20,000/\mu\text{L}$) to at least one prior line of therapy.
- 4. The use of any one of claims 1-3, wherein the baseline platelet count is the mean of two platelet counts taken on two occasions no less than 7 days apart in the 15 days prior to beginning treatment and a third count taken on the first day of the study treatment, and wherein at least one of the following occurs:
 - (a) the human patient achieves at least one platelet count of at least $20,000/\mu\text{L}$ above the baseline platelet count;
 - (b) the human patient achieves at least two platelet counts of at least $20,000/\mu\text{L}$ above the baseline platelet count;
 - (c) the human patient achieves at least two consecutive platelet counts of at least $20,000/\mu\text{L}$ above the baseline platelet count;
 - (d) the human patient achieves at least two platelet counts of at least $20,000/\mu\text{L}$ above the baseline platelet count separated by at least five days or separated by seven days; and
 - (e) the human patient achieves at least two consecutive platelet counts of at least $20,000/\mu\text{L}$ above the baseline platelet count separated by at least five days or separated by seven days.
- 5. The use of any one of claims 1-4, wherein at least one of the following occurs:
 - (a) the human patient achieves a platelet count of at least $50,000/\mu\text{L}$;
 - (b) the human patient achieves at least two platelet counts of at least $50,000/\mu\text{L}$;
 - (c) the human patient achieves at least two consecutive platelet counts of at least $50,000/\mu\text{L}$;
 - (d) the human patient achieves at least two platelet counts of at least $50,000/\mu\text{L}$ separated by at least five days or separated by seven days;
 - (e) the human patient achieves at least two consecutive platelet counts of at least $50,000/\mu\text{L}$ separated by at least five days or separated by seven days; and

(f) the human patient achieves at least one platelet count of at least 50,000/ μ L within eight days of initiating treatment.

6. The use of any one of claims 1-5, wherein the human patient has had ITP for six or less years four or less years.
7. The use of any one of claims 1-6, wherein at least one of the following occurs:
 - (a) the human patient has a history of taking at least one, two, three, four, or five prior ITP therapy prior to the start of the treatment period;
 - (b) the human patient has platelet counts between 2,000/ μ L and 33,000/ μ L on two occasions no less than 7 days apart and within 15 days prior to the start of the treatment period;
 - (c) the human patient had a splenectomy prior to the start of the treatment period.
8. The use of any one of claims 3-7, wherein at least one of the following occurs:
 - (a) the human patient has a history of taking at least one TPO-RA prior to the start of the treatment period; and
 - (b) the human patient has a history of taking at least one TPO-RA selected from rTPO, romiplostim, eltrombopag, and avatrombopag prior to the start of the treatment period.
9. The use of any one of claims 2-8, wherein at least one of the following occurs:
 - (a) the human patient has a history of response to at least one prior line of therapy chosen from a splenectomy, intravenous immunoglobulin (IVIG), corticosteroids, anti-D immunoglobulin therapy, and immunosuppressive drugs;
 - (b) the human patient has a history of response to at least one prior line of therapy and the response to the at least one prior therapy comprised a platelet count of $\geq 50,000/\mu\text{L}$; and
 - (c) the human patient has a history of taking at least one of intravenous immunoglobulin (IVIG), corticosteroids, anti-D immunoglobulin therapy, at least one immunosuppressive drug, and at least one immunosuppressive drug selected from

fostamatinib, mycophenolate mofetil (MMF), and cyclosporine, prior to the start of the treatment period.

10. The use of any one of claims 1-9, wherein at least one of the following occurs:

- (a) the human patient has primary ITP;
- (b) the human patient has secondary ITP;
- (c) the human patient does not have chronic ITP;
- (d) the human patient has persistent ITP;
- (e) the human patient has chronic ITP;
- (f) the human patient has relapsing ITP; and
- (g) the human patient has refractory ITP.

11. The use of any one of claims 1-10, wherein the treatment period is at least 8 days, at least 28 days, at least 84 days, or at least 169 days.

12. The use of any one of claims 1-11, wherein the human patient is to be administered 400 mg of at least one compound chosen from (R)-2-[3-[4-amino-3-(2-fluoro-4-phenoxy-phenyl)pyrazolo[3,4-d]pyrimidin-1-yl]piperidine-1-carbonyl]-4-methyl-4-[4-(oxetan-3-yl)piperazin-1-yl]pent-2-enenitrile and pharmaceutically acceptable salts thereof twice a day.

13. The use of claim 12, wherein:

- (a) the at least one compound consists of at least one compound chosen from the (E) isomer of (R)-2-[3-[4-amino-3-(2-fluoro-4-phenoxy-phenyl)pyrazolo[3,4-d]pyrimidin-1-yl]piperidine-1-carbonyl]-4-methyl-4-[4-(oxetan-3-yl)piperazin-1-yl]pent-2-enenitrile and pharmaceutically acceptable salts thereof;
- (b) the at least one compound consists of at least one compound chosen from the (Z) isomer of (R)-2-[3-[4-amino-3-(2-fluoro-4-phenoxy-phenyl)pyrazolo[3,4-d]pyrimidin-1-yl]piperidine-1-carbonyl]-4-methyl-4-[4-(oxetan-3-yl)piperazin-1-yl]pent-2-enenitrile and pharmaceutically acceptable salts thereof; or

(c) the at least one compound consists of at least one compound chosen from a mixture of (E) and (Z) isomers of (R)-2-[3-[4-amino-3-(2-fluoro-4-phenoxy-phenyl)pyrazolo[3,4-d]pyrimidin-1-yl]piperidine-1-carbonyl]-4-methyl-4-[4-(oxetan-3-yl)piperazin-1-yl]pent-2-enenitrile and pharmaceutically acceptable salt thereof.

14. The use of any one of claims 1-13, wherein the human patient is to be administered 400 mg of (R)-2-[3-[4-amino-3-(2-fluoro-4-phenoxy-phenyl)pyrazolo[3,4-d]pyrimidin-1-yl]piperidine-1-carbonyl]-4-methyl-4-[4-(oxetan-3-yl)piperazin-1-yl]pent-2-enenitrile twice a day.

15. The use of claim 14, wherein:

(a) the at least one compound is the (E) isomer of (R)-2-[3-[4-amino-3-(2-fluoro-4-phenoxy-phenyl)pyrazolo[3,4-d]pyrimidin-1-yl]piperidine-1-carbonyl]-4-methyl-4-[4-(oxetan-3-yl)piperazin-1-yl]pent-2-enenitrile;

(b) the at least one compound is the (Z) isomer of (R)-2-[3-[4-amino-3-(2-fluoro-4-phenoxy-phenyl)pyrazolo[3,4-d]pyrimidin-1-yl]piperidine-1-carbonyl]-4-methyl-4-[4-(oxetan-3-yl)piperazin-1-yl]pent-2-enenitrile; or

(c) the at least one compound consists of a mixture of (E) and (Z) isomers of (R)-2-[3-[4-amino-3-(2-fluoro-4-phenoxy-phenyl)pyrazolo[3,4-d]pyrimidin-1-yl]piperidine-1-carbonyl]-4-methyl-4-[4-(oxetan-3-yl)piperazin-1-yl]pent-2-enenitrile.