SLN124-004

Phase 1/2 study with an open-label dose escalation phase followed by a randomized, double-blind phase of SLN124 in patients with Polycythemia Vera

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Trattamento: SLN124

Sindromi MIELOPROLIFERATIVE CRONICHE target: Polycythemia Vera

Principali Criteri Inclusione:

1. Male and female patients aged 18 years or older.

2. A confirmed diagnosis of PV according to the revised 2016 WHO criteria

3. Prior to dosing, patients must have had ≥ 3 phlebotomies in the last 28 weeks, or 5 or more phlebotomies in the last 12 months, with documented raised Hct and/or documented presentation of symptoms of PV.

4. Records of phlebotomies and associated Hct performed for at least 28 weeks, or if available, for up to 12 months, prior to dosing, and any of the following associated data; soluble transferrin receptor 1 [sTfR1], ferritin or transferrin saturation [TSAT], if available.

5. Patients must have Hct < 43% prior to dosing. Hct test can be repeated during the screening period.

6. Patients who are not receiving cytoreductive therapy must have been discontinued from any prior cytoreductive therapy for at least 24 weeks before dosing and have recovered from any adverse events due to cytoreductive therapy.

7. Patients receiving cytoreductive therapy with hydroxyurea, interferon, busulfan or ruxolitinib must have received a stable dose of cytoreductive therapy for at least 12 weeks before dosing and with no planned change in cytoreductive dose

13. Patients must have had a dermatological examination within 6 months prior to screening or during screening.

14. Must have an Eastern Cooperative Oncology Group score of 0, 1, or 2.

Principali Criteri Esclusione:

3. Clinically significant thrombosis (e.g., deep vein thrombosis or splenic vein thrombosis) within 12 weeks of screening.

4. History of major bleeding events and/or a requirement for blood transfusion therapy owing to bleeding in the last 6 months prior to screening.

5. Meets the criteria for post-PV myelofibrosis as defined by the International Working Group-Myeloproliferative Neoplasms Research and Treatment