Tavalisse™ (fostamatinib) – New drug approval

- On April 17, 2018, Rigel Pharmaceuticals announced the FDA approval of Tavalisse (fostamatinib) for the treatment of thrombocytopenia in adult patients with chronic immune thrombocytopenia (ITP) who have had an insufficient response to previous treatment.

- ITP is a heterogeneous autoimmune bleeding disorder characterized by a low platelet count. Common symptoms of ITP include excessive bruising, bleeding and fatigue. People suffering with chronic ITP may live with an increased risk of severe bleeding events that can result in serious medical complications or even death.

- Tavalisse is an oral spleen tyrosine kinase inhibitor that targets the underlying autoimmune cause of the disease by impeding platelet destruction. This is a novel mechanism of action for the treatment of chronic ITP.

- The safety and efficacy of Tavalisse were supported by data from two placebo-controlled studies, referred to as FIT-1 and FIT-2, enrolling 150 patients for 24 weeks and one extension study, referred to as FIT-3 (enrolled 123 patients from FIT-1 and FIT-2). The efficacy of Tavalisse was based on stable platelet response.

    - For the FIT-1 study, 18% of Tavalisse patients vs. 0% of placebo patients achieved stable platelet response ($p = 0.03$).
    - For the FIT-2 study, 16% of Tavalisse patients vs. 4% of placebo patients achieved stable platelet response (p value was not significant).
    - For the FIT-3 study, 44 patients originally treated with placebo were switched to Tavalisse and evaluated for stable response. Of these, 23% met the criteria for stable response.

- Warnings and precautions for Tavalisse include hypertension, hepatotoxicity, diarrhea, neutropenia, and embryo-fetal toxicity.

- The most common adverse reactions ($\geq$ 5% and more than placebo) with Tavalisse use were diarrhea, hypertension, nausea, respiratory infection, dizziness, increased alanine transaminase/aspartate transaminase, rash, abdominal pain, fatigue, chest pain, and neutropenia.

- The recommended dose of Tavalisse is 100 mg orally twice daily with or without food. After 4 weeks, the dose is increased to 150 mg twice daily, if needed, to achieve a platelet count at least 50 x 10^9/L as necessary to reduce the risk of bleeding.

    - Discontinue Tavalisse after 12 weeks of treatment if the platelet count does not increase to a level sufficient to avoid clinically important bleeding.

- Rigel Pharmaceuticals plans to launch Tavalisse in late May 2018. Tavalisse will be available in 100 mg and 150 mg tablets.