Jakafi® (ruxolitinib) – New orphan indication

- On May 24, 2019, Incyte announced the FDA approval of Jakafi (ruxolitinib), for treatment of steroid-refractory acute graft-versus-host disease (GVHD) in adult and pediatric patients 12 years and older.
- Jakafi is also approved for treatment of adults with myelofibrosis and polycythemia vera.
- GVHD is a condition that can occur after an allogeneic stem cell transplant where the donated cells initiate an immune response and attack the transplant recipient's organs. There are two major forms of GVHD, acute and chronic, that can affect multiple organ systems including the skin, gastrointestinal tract, and liver.
    - Patients who develop steroid-refractory acute GVHD can progress to severe disease, with one-year mortality rates of approximately 70%.
- The approval of Jakafi's new indication was based on an open-label, single-arm study in 49 patients with steroid-refractory acute GVHD occurring after allogeneic hematopoietic stem cell transplantation. The efficacy of Jakafi was based on day-28 overall response rate (ORR) and the duration of response (DOR).
    - The ORR was 57.1% (95% CI: 42.2, 71.2). The median DOR was 16 days (95% CI: 9, 83).
- In acute GVHD, the most common hematologic adverse reactions (> 50%) with Jakafi use were anemia, thrombocytopenia, and neutropenia. The most common nonhematologic adverse reactions (> 50%) were infections and edema.
- The recommended starting dose of Jakafi for GVHD is 5 mg orally twice daily. The dose may be increased to 10 mg twice daily after at least 3 days of treatment if the absolute neutrophil count and platelet counts are not decreased by 50% or more relative to the first day of dosing with Jakafi.
    - Refer to the Jakafi drug label for dosing for its other indications.