Rezurock™ (belumosudil) – New orphan drug approval

- On July 16, 2021, Kadmon Pharmaceuticals announced the FDA approval of Rezurock (belumosudil), for the treatment of adult and pediatric patients 12 years and older with chronic graft-versus-host disease (chronic GvHD) after failure of at least two prior lines of systemic therapy.

- Chronic GvHD is a complication that can occur following allogeneic stem cell transplantation. In GvHD, transplanted immune cells (graft) attack the patient's cells (host), leading to inflammation and fibrosis in multiple tissues, including skin, mouth, eye, joints, liver, lung, and gastrointestinal tract.

    — Approximately 14,000 patients in the U.S. are living with chronic GvHD.

- Rezurock is the first approved therapy targeting Rho-associated coiled-coil kinase 2 (ROCK2), a signaling pathway that modulates inflammatory response and fibrotic processes.

- The efficacy of Rezurock was established in a randomized, open-label study in 65 patients with chronic GvHD who had received 2 to 5 prior lines of systemic therapy and required additional treatment. Patients were treated with Rezurock and concomitant treatment with supportive care therapies for chronic GvHD was permitted. The primary endpoint was overall response rate (ORR) through cycle 7 day 1 where overall response included complete response or partial response according to the 2014 NIH Response Criteria.

    — The ORR was 75% (95% CI: 63, 85).
    — The median duration of response, calculated from first response to progression, death, or new systemic therapies for chronic GvHD, was 1.9 months (95% CI: 1.2, 2.9).
    — In patients who achieved response, no death or new systemic therapy initiation occurred in 62% (95% CI: 46, 74) of patients for at least 12 months since response.

- A warning and precaution for Rezurock is embryo-fetal toxicity.

- The most common adverse reactions (≥ 20%) with Rezurock use were infections, asthenia, nausea, diarrhea, dyspnea, cough, edema, hemorrhage, abdominal pain, musculoskeletal pain, headache, decreased phosphate, increased gamma glutamyl transferase, decreased lymphocytes, and hypertension.

- The recommended dose of Rezurock is 200 mg given orally once daily until progression of chronic GvHD that requires new systemic therapy.

    — Treatment with Rezurock has not been studied in patients with pre-existing severe renal or hepatic impairment. For patients with pre-existing severe renal or hepatic impairment, consider the risks and potential benefits before initiating treatment.

- Kadmon Pharmaceuticals plans to launch Rezurock by late August 2021. Rezurock will be available as a 200 mg tablet.