Aliqopa™ (copanlisib) – New orphan drug approval

- On September 14, 2017, the FDA announced the approval of Bayer's Aliqopa (copanlisib) for the treatment of adult patients with relapsed follicular lymphoma (FL) who have received at least two prior systemic therapies.
    - Accelerated approval was granted for this indication based on overall response rate (ORR). Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial.
- FL is a slow-growing type of non-Hodgkin lymphoma (NHL), a cancer of the lymph system. The lymph system is part of the body's immune system and is made up of lymph tissue, lymph nodes, the spleen, thymus, tonsils and bone marrow.
- The National Cancer Institute estimates that approximately 72,240 people in the U.S. will be diagnosed with some form of NHL this year; approximately 20,140 patients with NHL will die from the disease in 2017.
- Aliqopa is a kinase inhibitor that works by blocking several enzymes that promote cell growth.
- The efficacy of Aliqopa was based on data from a single-arm trial of 104 patients with follicular B-cell NHL who had relapsed disease following at least two prior treatments. Efficacy was based on ORR.
    - The ORR was 59% (95% CI: 49, 68). Fourteen percent of patients had a complete response and 44% had a partial response. The median duration of response was 12.2 months (range: 0+, 22.6).
    - The median time to response was 1.7 months (range: 1.3, 9.7).
- Warnings and precautions of Aliqopa include infections, hyperglycemia, hypertension, non-infectious pneumonitis, neutropenia, severe cutaneous reactions, and embryo-fetal toxicity.
- The most common adverse reactions (≥ 20%) with Aliqopa use were hyperglycemia, diarrhea, decreased general strength and energy, hypertension, leukopenia, neutropenia, nausea, lower respiratory tract infections, and thrombocytopenia.
- The recommended dose of Aliqopa is 60 mg administered as a 1-hour intravenous infusion on days 1, 8, and 15 of a 28-day treatment cycle on an intermittent schedule (three weeks on and one week off). Treatment is continued until disease progression or unacceptable toxicity.
- The Aliqopa™ Resource Connection (ARC™) Program, which includes a range of resources to help patients navigate the insurance process and identify sources of financial assistance, is now available. The program offers free medication to those who are uninsured or underinsured and meet the eligibility criteria, and includes a $0 co-pay program for covered patients.
- Aliqopa will be available in the U.S. market immediately. Aliqopa will be available as a 60 mg single-dose vial for reconstitution.