RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Venclexta is used for the treatment of adult patients with chronic lymphocytic leukemia (CLL) or small lymphocytic lymphoma (SLL). Venclexta is FDA-approved treatment that targets the B-cell lymphoma 2 (BCL-2) protein, which supports cancer cell growth (1).

Regulatory Status

FDA-approved indications: Venclexta is a BCL-2 inhibitor indicated: (1)

1. For the treatment of adult patients with chronic lymphocytic leukemia (CLL) or small lymphocytic lymphoma (SLL)
2. In combination with azacitidine or decitabine or low-dose cytarabine for the treatment of newly-diagnosed acute myeloid leukemia (AML) in adults who are 75 years or older, or who have comorbidities that preclude use of intensive induction chemotherapy

Off-Label Uses: (2-7)

1. Mantle cell lymphoma (MCL)
2. Acute myeloid leukemia (AML)
3. Waldenstrom macroglobulinemia (WM)
4. Systemic light chain amyloidosis (SLCA)

Venclexta can cause rapid reduction in tumor and thus poses a risk for Tumor Lysis Syndrome (TLS), which can occur within 6-8 hours after the first infusion. Patients with high tumor burden and/or high circulating lymphocyte count are at greater risk for TLS and should receive appropriate tumor lysis prophylaxis with anti-hyperuricemics (e.g., allopurinol) and hydration beginning 12-24 hours prior to the infusion of Venclexta. For treatment of TLS, correct electrolyte abnormalities, monitor renal function, and fluid balance, and administer supportive care, including dialysis as indicated (1).

Neutropenia may occur during Venclexta therapy. Complete blood counts (CBC) should be monitored throughout the treatment period. Dosing should be interrupted or reduced for severe neutropenia (1).

Venclexta may cause embryo-fetal harm when administered to a pregnant woman. Females of reproductive potential should be advised to avoid pregnancy during treatment. Pregnant patients

should be advised of the potential hazard to the fetus (1).

The safety and efficacy of immunization with live or attenuated viral vaccines during or following Venclexta therapy has not been studied. Immunization with live virus vaccines is not recommended during treatment and until B-cell recovery (1).

The safety and effectiveness of Venclexta in pediatric patients have not been established (1).

Summary

Venclexta is used for the treatment of adult patients with chronic lymphocytic leukemia (CLL) or small lymphocytic lymphoma (SLL). Venclexta is also approved for use in relapsed or refractory acute myeloid leukemia (AML) or newly-diagnosed AML. Venclexta may be used off-label for mantle cell lymphoma (MCL), Waldenstrom macroglobulinemia (WM), and systemic light chain amyloidosis (SLCA). Venclexta can cause rapid reduction in tumor and thus poses a risk for Tumor Lysis Syndrome (TLS), which can occur within 12-24 hours after the first infusion. The safety and efficacy of immunization with live or attenuated viral vaccines during or following Venclexta therapy has not been studied. The safety and efficacy of Venclexta in pediatric patients has not been established (1).

Prior approval is required to ensure the safe, clinically appropriate, and cost-effective use of Venclexta while maintaining optimal therapeutic outcomes.

References

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