RATIONALE FOR INCLUSION IN PA PROGRAM

Background
Besponsa is an injectable cancer agent that works as a CD22-directed antibody drug conjugate (ADC). Besponsa is indicated for the treatment of adults with relapsed or refractory B-cell precursor acute lymphoblastic leukemia (ALL). B-cell precursor ALL is a rapidly progressing type of cancer in which the bone marrow makes too many B-cell lymphocytes, an immature type of white blood cell. Besponsa is a targeted therapy that is thought to work by binding to B-cell ALL cancer cells that express the CD22 antigen, blocking the growth of cancerous cells (1).

Regulatory Status
FDA-approved indication: Besponsa is a CD22-directed antibody drug conjugate (ADC) indicated for the treatment of adult patients with relapsed or refractory B-cell precursor acute lymphoblastic leukemia (ALL) (1).

Besponsa has a boxed warning for hepatotoxicity that can include fatal and life-threatening hepatic veno-occlusive disease (VOD). Risk factors for VOD in patients treated with Besponsa include ongoing or prior liver disease, prior post-hematopoietic stem cell transplant (HSCT), increased age, later salvage lines and a greater number of Besponsa treatment cycles. If elevated liver tests are obtained, it may require the dose of Besponsa to be interrupted, reduced, or permanent discontinued. If VOD occurs in patients, permanent discontinuation of Besponsa will be necessary (1).

Patients in the clinical studies with Philadelphia chromosome-positive (Ph+) B-cell precursor ALL were required to have a failed treatment with at least 1 tyrosine kinase inhibitor and standard chemotherapy (1).

The safety and effectiveness of Besponsa has not been established in pediatric patients below 18 years of age (1).

Summary
Besponsa is indicated for the treatment of relapsed or refractory B-cell precursor acute lymphoblastic leukemia (ALL) in patients 18 years of age and older. Safety and efficacy in pediatric patients below the age of 18 have not been established (1).
References