M13-833: Venetoclax in Pediatric and Young Adult Patients with Relapsed or Refractory Malignancies

Phase 1
Pediatric and Young Adult (<25 years of age) with Relapsed / Refractory Malignancies

Part 1 Dose Determination
N = 10 pediatric patients < 18 years of age (maximum 20 patients, including young adults)

Part 2 Cohort Expansion
N = 44-125
Five tumor cohorts:
- ALL
- AML
- NHL
- Neuroblastoma
- Other tumors with BCL-2 expression

Key Inclusion Criteria
- Age < 25 years of age at enrollment
- Adequate hepatic and renal function; normal creatinine for age
- Lansky ≥ 50 (≤ 16 years of age) or Karnofsky ≥ 50 (>16 years of age)

In Part 1:
- Patients must have relapsed or refractory cancer
- Patients with solid tumors (excluding neuroblastoma) must have adequate bone marrow function

In Part 2:
- Patients must have histologically confirmed relapsed or refractory ALL, AML, NHL, neuroblastoma, or other tumor with BCL-2 expression

Key Exclusion Criteria
- Primary brain tumors or disease metastatic to the brain
- For patients with leukemias, overt CNS disease (CNS 3 status)

Endpoints
- Primary: safety of venetoclax monotherapy (DLTs, RPTD), PK of venetoclax monotherapy
- Secondary: preliminary efficacy of venetoclax monotherapy, safety and preliminary efficacy of venetoclax in combination with chemotherapy
- Exploratory: pharmacodynamic and predictive biomarkers; MRD status in the peripheral blood and bone marrow

Venetoclax in combination with chemotherapy is allowed after 21 days of monotherapy for patients with hematologic malignancies and after 8 weeks of monotherapy for patients with solid tumors.

Venetoclax is being investigated for indications that have not been approved by Regulatory Agencies. Safety and Efficacy have not been established in unapproved indications.

To learn more about these studies, please visit https://ClinicalTrials.gov or email abbbvieclinicaltrials@abbvie.com


Venetoclax is being codeveloped by AbbVie and Genentech, a member of the Roche Group.