Study to Determine the Efficacy and Safety of Asciminib in Pediatric Patients With Ph+ CML-CP

Status: RECRUITING

Eligibility Criteria

Sex: ALL
Age: 1 year to 18 years old
Healthy Volunteers: This study is NOT accepting healthy volunteers
Key Inclusion Criteria:

Participants eligible for inclusion in this study must meet all of the following criteria:

1. Signed informed consent must be obtained prior to participation in the study.
2. Male or female participants 1 and < 18 years of age at study enrollment
3. Diagnosis of CML-CP (Apperley et al 2025) with cytogenetic confirmation of Philadelphia positive (Ph+) chromosome
4. For participants with CML-CP newly diagnosed within 3 months of screening OR 5 For participants with CML - CP with high risk of developing resistance or intolerance to previous TKI:

    1. Unfavourable response to TKI is defined following the Apperley et al 2025 guidelines as:

* At three months after the initiation of therapy: BCR::ABL1 ratio \> 10% IS (if confirmed within 1-3 months)
* At six months after the initiation of therapy: BCR::ABL1 ratio \> 10% IS
* At twelve months after initiation of therapy: BCR::ABL1 ratio \> 1% IS
* At any time loss of previous response
* At any time emergent resistant BCR::ABL1 mutations or high-risk ACA from prior TKI treatment as per local test results

    1. Intolerance to TKI is defined as:

* Non-hematologic intolerance: participants with grade 3 or 4 toxicity while on therapy (in which case the patient is eligible whether or not there was a dose reduction); or with persistent grade 2 toxicity unresponsive to optimal management including dose adjustments (unless dose reduction is not considered in the best interest of the patient if response is already suboptimal)
* Hematologic intolerance: participants with grade 3 or 4 toxicity (absolute neutrophil count \[ANC\] or platelets) while on therapy that is recurrent after dose reduction to the lowest doses of the TKI

6. Evidence of typical BCR::ABL1 transcript [e14a2 and/or e13a2] at the time of screening which are amenable to standardized RQ-PCR quantification.

7. Performance status: Karnofsky ≥ 50% for participants ≥ 16 years of age, and Lansky ≥ 50 for participants < 16 years of age at the time of screening.

Key Exclusion Criteria:

1. Known second chronic phase (CP) of CML after previous progression to Accelerated Phase (AP)/Blast Phase (BP).
2. Previous treatment with a hematopoietic stem-cell transplantation.
3. Patient planned to undergo allogeneic hematopoietic stem cell transplantation
4. Known presence of a BCR::ABL1 mutation with known resistance to study treatment in accordance with the most recent public version of international CML clinical guidelines (e.g. NCCN CML treatment guidelines v 1.2026 and Apperley et al 2025) any time prior to study entry

Other inclusion/exclusion criteria may apply.

Conditions & Interventions

Interventions:
DRUG: Asciminib single agent
Conditions:
Chronic Myelogenous Leukemia, Leukemia, Myelogenous, Chronic, Philadelphia Chromosome Positive
Keywords:
Asciminib, ABL001, Pediatric participants, Philadelphia chromosome positive chronic myeloid leukemia in chronic phase, Ph+ CML-CP, tyrosine kinase inhibitor, TKI, Molecular Response, MR, CML, Chronic phase, T3151, Ph+

More Information

Description:
The aim of this study is to support development of asciminib in the pediatric population (1 to \< 18 years) with Ph+ CML-CP. The study will evaluate the efficacy and safety of asciminib in pediatric formulation (weigh-based dose, fed state) or adult formulation (fasted) in newly diagnosed and resistant or intolerant Ph+ CML-CP with or without T315I mutation.

Contact(s): - cancer@cchmc.org
Phase: PHASE2
System ID: NCT07354074

Thank you for choosing StudyFinder. Please visit http://studyfinder.cchmc.org to find a Study which is right for you and contact clinicalstudies@cchmc.org if you have questions or need assistance.