Tyrosine kinase inhibitors (TKIs) that inhibit BCR-ABL1 are the standard of care for adult and pediatric patients with chronic myeloid leukemia in chronic phase (CML-CP) 

- 5 TKIs are approved in the US for adults: imatinib, dasatinib, nilotinib, bosutinib, and asciminib.

Novel Mechanism of Action
- Asciminib is a potent and specific allosteric inhibitor of BCR-ABL1 that works by specifically Targeting the ABL Myristoyl Pocket (STAMP) (Figure 1). 

Asciminib Efficacy and Safety in 3L+ in Adults
- Asciminib has shown superior efficacy and safety in adults with CML-CP compared to bosutinib in the Phase II ASCEND study, in which it was given in the fasted state (i.e., without food 2 hours prior and 1 hour after taking asciminib) (Figure 2).

STUDY DESIGN AND METHODS
- Asciminib is a multicenter, open-label, phase II/III study of asciminib in the pediatric population previously treated with ≥1 TKIs (Figures 3a and 3b).

CONCLUSIONS
- Recruitment to ASC4KIDS, a phase II/II study, is underway. This study will support the development of asciminib in the pediatric population (1 to <18 years old) with Ph+ CML-CP previously treated with TKI.

END POINTS TO ACHIEVE THIS GOAL
- Assessments of TEAEs and other safety data, including growth and sexual development of pediatric patients.

- Questionnaire on acceptability and palatability after first dose, 4 weeks, and 52 weeks.

- Biomarkers of efficacy including MCyR, MR, and MMR.

- Molecular responses.

- Other endpoints to be determined by Medical Dictionary for Regulatory Activities version 23.0 and Common Terminology Criteria for Adverse Events version 4.03.

Figure 4. Study Objectives and Endpoints

Table 1. Overview of Eligibility Criteria

- Pediatric patients (≥1 to <18 years of age).
- Previous TKI therapy at the time of screening.
- Evidence of typical BCR-ABL1 transcript and presence of BCR-ABL1 transcript after recent TKI therapy.
- Known presence of the T315I mutation prior to study entry.
- Prior treatment with ≥1 TKI.
- Previous hematopoietic stem cell transplant.
- Carbamazepine: limited to 50 mg/kg/day or dose that may not significantly alter the absorption of asciminib.

Figure 5. Participating Countries

ASC MIN AS HIST COMPLEMENTS
- Asciminib is now open to enrollment.
- The estimated primary completion date is November 2025, and the estimated study completion date is November 2029.

- 36 study sites in the 14 countries shown in Figure 5 are participating.