# Open-label Study of Asciminib for CML-CP or CML-AP Patients With T315I Mutation Who Are Resistant, Intolerant or Ineligible to Ponatinib.

Last Update: Mar 05, 2025
A Phase II, Multi-center, Prospective, Open-label Study of Asciminib in Patients With Chronic Myeloid Leukemia in Chronic Phase (CML-CP) or Accelerated Phase (CML-AP) With T315I Mutation Who Are Resistant, Intolerant or Ineligible to Ponatinib.
ClinicalTrials.gov Identifier:
NCT06514534
Novartis Reference Number:CABL001AFR05
See if you Pre-qualify
All compounds are either investigational or being studied for (a) new use(s). Efficacy and safety have not been established. There is no guarantee that they will become commercially available for the use(s) under investigation.

## Study Description

The objective of this Phase II study is to assess the potential of asciminib in managing CML-CP or CML-AP in patient carrying the T315I mutation. The presence of this mutation introduces treatment difficulties due to the limited available options. The study seeks to collect additional data on the effectiveness and safety of asciminib for these patients. By determining the drug's capacity to manage the disease and enhance patients outcomes, the study is designed to fill the unmet medical need and potentially offer a new therapeutic path for patients at a treatment deadlock. This study is a Phase II, multi-center, single-arm prospective, open-label study that aims to evaluate the efficacy and safety of oral asciminib in patients with CML-CP or CML-AP with T315I mutation and after at least one tyrosine kinase inhibitors (TKI) and are resistant, intolerant, or ineligible for treatment with ponatinib.

Patients who have not been previously treated with asciminib would be enrolled in this study. The researchers will assess the effectiveness of asciminib in these participants, as well as evaluate its safety profile. The study will consist of two phases:

* The "core phase" which aims to answer the scientific and medical objectives.
* An "extension phase" intended to provide opportunity to the participants to continue their ongoing treatment (asciminib) up to commercialization in France or decision to not commercialize asciminib for the study population (stopping development, refusal to extend marketing authorization, refusal of reimbursement).

Condition
Chronic Myeloid Leukemia (CML)
Phase
Phase2
Overall Status
Recruiting

Number of Participants
20
Start Date
Feb 18, 2025
Completion Date
Dec 01, 2028
Gender
All
Age(s)
18 Years - 99 Years (Adult, Older Adult)

# Interventions

Drug

### ABL001/Asciminib

The study treatment for this clinical trial is an investigational drug called asciminib, which is marketed under the brand name Scemblix®. Asciminib is a compound that is being evaluated for its efficacy and safety in the treatment of the target condition. The minimum dose of asciminib to be administered in this study is 200 mg, while the maximum dose is 400 mg. The dose is planned as 200 mg twice a day (BID). The drug will be administered orally, allowing for convenient and non-invasive administration.

# Eligibility Criteria

Inclusion Criteria:

* Signed informed consent must be obtained prior to participation in the study.
* Male or female participants with a diagnosis of CML-CP or CML-AP ≥ 18 years of age.
* Patients with CML-CP or CML-AP with history of documented T315I mutation after at least one TKI and are resistant, intolerant, or ineligible to ponatinib (according to Investigator judgment)
* Not already treated with asciminib or another any allosteric TKI
* Failure (adapted from the 2020 \& 2013 ELN Guidelines) or intolerance to Ponatinib at the time of Screening.
* Ineligible to ponatinib according to Investigator (based on EU ponatinib SmPC)
* Evidence of typical BCR::ABL1 transcript or atypical transcripts at the time of Screening which are amenable to standardized or non-standardized RQ-PCR quantification.

Exclusion Criteria:

* Previous hematopoietic allogeneic stem-cell transplantation
* Cardiac or cardiac repolarization abnormality
* Severe and/or uncontrolled concurrent medical disease that in the opinion of the Investigator could cause unacceptable safety risks or compromise compliance with the protocol (e.g. uncontrolled diabetes, active or uncontrolled infection, pulmonary hypertension)
* History of clinical acute pancreatitis within 1 year of study entry or past medical history of chronic pancreatitis (except if ponatinib-induced and completely resolved at time of Screening)
* History of acute or chronic liver disease (i.e., cirrhosis; liver impairment)
* Known presence of significant congenital or acquired bleeding disorder unrelated to cancer
* History of other active malignancy within 3 years prior to study entry with the exception of previous or

concomitant basal cell skin cancer and previous carcinoma in situ treated curatively
* Known history of Human Immunodeficiency Virus (HIV), chronic Hepatitis B Virus (HBV), or chronic Hepatitis C Virus (HCV) infection. Testing for Hepatitis B surface antigen (HBs Ag) and Hepatitis B core antibody (HBcAb / anti HBc) will be performed at Screening
* Impairment of gastrointestinal (GI) function or GI disease that may significantly alter the absorption of study drug (e.g. ulcerative disease, uncontrolled nausea, vomiting, diarrhea, malabsorption syndrome, small bowel resection, or gastric bypass surgery)
* Treatment with medications that meet one of the following criteria and that cannot be discontinued at least one week prior to the start of treatment with study treatment:

* Moderate or strong inducers of CYP3A
* Moderate or strong inhibitors of CYP3A
* Pregnant or nursing (lactating) women
* Women of child-bearing potential
* Compound mutant T315I resistant to asciminib monotherapy (polyclonal ABL1 mutations including T315I can be enrolled) Other protocol-defined inclusion/exclusion criteria may apply.

**France**

**Novartis Investigative Site**

Recruiting

Lyon,69373,France

# Worldwide Contacts

If the location of your choosing does not feature any contact detail, please reach out using the information below.

**Novartis Pharmaceuticals**

Phone: +41613241111
Email: novartis.email@novartis.com

**Novartis Pharmaceuticals**

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