Study to Determine the Dose and Safety of Asciminib in Pediatric Patients With Chronic Myeloid Leukemia

Last Update: Jun 22, 2025
A Multi-center, Open-label Study to Determine the Dose and Safety of Oral Asciminib in Pediatric Patients With Philadelphia Chromosome Positive Chronic Myeloid Leukemia in Chronic Phase (Ph+ CML-CP), Previously Treated With One or More Tyrosine Kinase Inhibitors
ClinicalTrials.gov Identifier:
NCT04925479
Novartis Reference Number:CABL001I12201
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 aim of this study is to support development of asciminib in the pediatric population (1 to \<18 years) previously treated with one or more TKIs. Full extrapolation of the efficacy of asciminib from adult to pediatric patients will be conducted. Full extrapolation is based on the concept that CML in the pediatric population has the same pathogenesis, similar clinical characteristics and progression pattern as in adults. The aim of this study is to support development of asciminib in the pediatric population (1 to \<18 years) with Philadelphia chromosome positive chronic myeloid leukemia in chronic phase (PH+ CML-CP) previously treated with one or more Tyrosine kinase inhibitor (TKIs).

The primary objective of this study is to characterize the pharmacokinetic (PK) profile of asciminib in pediatric patients with the goal of identifying the pediatric formulation dose (fed) leading to asciminib exposure comparable to 40 mg BID in adult patients (fasted).

The pediatric formulation group will include at least 15 participants in each of the following two age categories: 1 to \<12 years and 12 to \<18 years; leading to at least 30 participants enrolled treated with the pediatric formulation. It will consist of a dose determination part (Part 1) and a cohort expansion (Part 2 BID regimen and Part 3 QD regimen).

In Part 1, 4-6 participants will be enrolled in order to obtain at least 4 participants evaluable for PK (these participants may be from either of the age categories described above). The initial starting dose will be based on body weight and will be administered BID with food.

Once the body weight adjusted dose has been determined in Part 1 of the study, the patients will be enrolled in Part 2 until at least 20 participants, including those who were included in Part 1, have been enrolled (10 per age group) in the pediatric formulation group. Once the interim safety and PK analysis 2 is completed for one of the age groups, the Part 3 QD regimen will open for the respective age group to enroll 10 patients (5 patients by age group).

Due to the fact that the pediatric formulation was in development and was not available, this study started with the recruitment of adolescent patients. These participants aged 14 to \<18 years, weighing at least 40 kg receive the adult formulation at a flat dose of 40 mg BID under fasted conditions.

The total duration of the treatment period of the study will be 5 years (260 weeks). Participants who, according to Investigator's judgement, are benefiting from study treatment will remain on treatment up to the completion of the treatment period (Week 260/5 years). The primary analysis for the BID regimen is planned after all participants in Part 1 and 2 have completed at least 52 weeks of study treatment or discontinued earlier.

The primary analysis for combined regimen (BID+QD) is planned after all participants in Part 1, 2 and 3 have completed at least 52 weeks of study treatment or discontinued earlier.

Condition
Myeloid Leukemia, Philadelphia Positive
Phase
Phase1, Phase2
Overall Status
Recruiting
Number of Participants
44
Start Date
Dec 27, 2021
Completion Date
Nov 01, 2031
Gender
All
Age(s)
1 Year - 17 Years (Child)

Interventions

Drug

Asciminib Adult formulation group

Asciminib Adult formulation group: 40 mg tablets BID, taken orally. 20 mg tablets BID, taken orally.
Drug

Asciminib Pediatric formulation group

Asciminib Pediatric formulation group: Mini-tablets will be supplied as size 0 capsules containing 1 mg mini-tablets, taken orally: 10 mg (10x 1 mg tablets in capsule) 15 mg (15x 1 mg tablets in capsule) 20 mg (20x 1 mg tablets in capsule) 30 mg (30x 1 mg tablets in capsule)

Eligibility Criteria

Inclusion Criteria:

- Male or female participants: Pediatric formulation group: ≥ 1 and less than 18 years of age at study entry. Adult formulation group: ≥ 14 and less than 18 years of age and body weight of ≥ 40 kg at study entry.

* Participants with Ph+ CML-CP must meet all of the following laboratory values at the screening visit. In the case where bone marrow blast and promyelocyte counts are available, these will be accepted if done within 56 days prior to the screening visit, to avoid unnecessary repetition of this test.

1. \< 15% blasts in peripheral blood and bone marrow
2. \< 30% combined blasts plus promyelocytes in peripheral blood and bone marrow
3. \< 20% basophils in the peripheral blood
4. Neutrophils ≥ 1.5 x 10\^9/L (or WBC ≥ 3 x 10\^9/L if neutrophils are not available) and platelet count ≥ 100 x 10\^9/L
5. No evidence of extramedullary leukemic involvement, with the exception of hepatosplenomegaly

* Prior treatment with a minimum of one TKI
* Failure (adapted from the 2020 European Leukemia Net (ELN) Guidelines Hochhaus et al 2020 and 2013 ELN Guidelines Baccarani et al 2013) or intolerance to the most recent TKI therapy at the time of screening.
* Performance status: Karnofsky ≥ 50% for patients ≥ 16 years of age, and Lansky ≥ 50 for patients \< 16 years of age at the time of screening
* Participants must have adequate renal, hepatic, pancreatic and cardiac function
* Participants must have electrolyte values within normal limits or corrected to be within normal limits with supplements prior to first dose of study medication:
* Evidence of typical BCR-ABL1 transcript \[e14a2 and/or e13a2\] at the time of screening which are amenable to standardized RQ-PCR quantification.

Exclusion Criteria:

* Known presence of the T315I mutation prior to study entry or a BCR::ABL mutation with known resistance to study treatment any time prior to study entry.
* Known second chronic phase of CML after previous progression to AP/BC.
* Previous treatment with a hematopoietic stem-cell transplantation.
* Patient planning to undergo allogeneic hematopoietic 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
* History of acute pancreatitis within 1 year of study entry or past medical history of chronic pancreatitis.
* History of acute or chronic liver disease.
* Impairment of gastrointestinal (GI) function or GI disease that may significantly alter the absorption of study drug
* Pregnant or nursing (lactating) females.

Other protocol-defined inclusion/exclusion may apply.

China

Novartis Investigative Site

Recruiting

Hangzhou,Zhejiang,310052,China

Novartis Investigative Site

Recruiting

Beijing,100044,China

Novartis Investigative Site

Recruiting

Shanghai,200127,China

Novartis Investigative Site

Recruiting

Tianjin,300020,China

France

Novartis Investigative Site

Recruiting

Bordeaux Cedex,33076,France

Novartis Investigative Site

Recruiting

Lille,59000,France

Novartis Investigative Site

Recruiting

Paris,75019,France

Novartis Investigative Site

Recruiting

Poitiers,86021,France

Germany

Novartis Investigative Site

Recruiting

Hamburg,20246,Germany

Novartis Investigative Site

Recruiting

Erlangen,91054,Germany

Novartis Investigative Site

Recruiting

Essen,45147,Germany

Greece

Novartis Investigative Site

Recruiting

Athens,115 27,Greece

Hungary

Novartis Investigative Site

Recruiting

Budapest,H-1083,Hungary

Italy

Novartis Investigative Site

Recruiting

Roma,RM,00165,Italy

Novartis Investigative Site

Recruiting

Genova,GE,16147,Italy

Novartis Investigative Site

Recruiting

Monza,MB,20900,Italy

Novartis Investigative Site

Recruiting

Torino,TO,10126,Italy

Japan

Novartis Investigative Site

Recruiting

Shinjuku-ku,Tokyo,160 8582,Japan

Novartis Investigative Site

Recruiting

Osaka,534-0021,Japan

Novartis Investigative Site

Recruiting

Yokohama,Kanagawa,232-8555,Japan

Korea, Republic of

Novartis Investigative Site

Recruiting

Seoul,03080,Korea, Republic of

Novartis Investigative Site

Recruiting

Seoul,05505,Korea, Republic of

Netherlands

Novartis Investigative Site

Recruiting

Utrecht,CS,3584,Netherlands

Poland

Novartis Investigative Site

Recruiting

Wroclaw,50367,Poland

Russian Federation

Novartis Investigative Site

Recruiting

Moscow,117198,Russian Federation

Novartis Investigative Site

Recruiting

Saint Petersburg,197022,Russian Federation

Thailand

Novartis Investigative Site

Recruiting

Chiang Mai,50200,Thailand

Novartis Investigative Site

Recruiting

Khon Kaen,THA,40002,Thailand

Novartis Investigative Site

Recruiting

Bangkok,10400,Thailand

Turkey

Novartis Investigative Site

Recruiting

Bursa,Gorukle,16059,Turkey

Novartis Investigative Site

Recruiting

Istanbul,34093,Turkey

United States

University Of Utah

Recruiting

Salt Lake City,Utah,84132,United States

Mallorie Heneghan

Priscilla Rosen

Phone: 801-213-3599
Email: priscilla.rosen@hsc.utah.edu

Cinn Children Hosp Medical Center

Recruiting

Cincinnati,Ohio,45229-3039,United States

Angela Kyntchev

Phone: 800-344-2462
Email: Angela.kyntchev@cchmc.org

Benjamin Mizukawa

University of Mississippi Medical Center

Recruiting

Jackson,Mississippi,39216-4505,United States

Dereck B Davis

Maggie Goad

Phone: 601-984-5220
Email: mgoad@umc.edu

Indiana UH Riley H for CIU

Recruiting

Indianapolis,Indiana,46202-5167,United States

Jennifer Pencek

Email: jpencek@iupui.edu

Rachael Schulte

Columbia University Medical Center New York Presbyterian

Recruiting

New York,New York,10032,United States

Phone: 212-305-9770

Nobuko Hijiya

Childrens Hospital of Philadelphia

Recruiting

Philadelphia,Pennsylvania,19104 4399,United States

Phone: 919-590-5106

Sarah Tasian

Uni Of Texas MD Anderson Cancer Ctr

Recruiting

Houston,Texas,77024,United States

David McCall

Thinh Nguyen

Email: TQNguyen1@mdanderson.org

Dana Farber Cancer Institute

Recruiting

Boston,Massachusetts,02215,United States

Jessica Pollard

Sara Galinko

Phone: 617-632-6644
Email: sara_galinko@dfci.harvard.edu

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 Pharmaceuticals

Phone: 1-888-669-6682
Email: novartis.email@novartis.com

Source URL: *https://prod1.novartis.com/clinicaltrials/study/nct04925479*

List of links present in page

1. https://clinicaltrials.gov/ct2/show/NCT04925479
2. #trial-eligibility
3. tel:801-213-3599
4. mailto:priscilla.rosen@hsc.utah.edu
5. tel:800-344-2462
6. mailto:Angela.kyntchev@cchmc.org
7. tel:601-984-5220
8. mailto:mgoad@umc.edu

9. mailto:jpencek@iupui.edu
10. tel:212-305-9770
11. tel:919-590-5106
12. mailto:TQNguyen1@mdanderson.org
13. tel:617-632-6644
14. mailto:sara_galinko@dfci.harvard.edu
15. tel:+41613241111
16. mailto:
17. tel:1-888-669-6682
18. mailto:novartis.email@novartis.com