



A Phase 3, Multi-center, Open-label, Randomized Study of Oral ABL001 Versus Bosutinib in Patients With Chronic Myelogenous Leukemia in Chronic Phase (CML-CP), Previously Treated With 2 or More Tyrosine Kinase Inhibitors

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Main Information

Primary registry identifying number

LBCTR2019010185

Protocol number

CABL001A2301

MOH registration number

49983/2017

Study registered at the country of origin

Yes

Study registered at the country of origin: Specify

Type of registration

Retrospective

Type of registration: Justify

LCTR was already initiated, original file was previously submitted

Date of registration in national regulatory agency

21/12/2017

Primary sponsor

Novartis Pharma Services Inc.

Primary sponsor: Country of origin

Novartis Pharmaceuticals

Date of registration in primary registry

02/10/2020

Date of registration in national regulatory agency

21/12/2017

Public title

A Phase 3, Multi-center, Open-label, Randomized Study of Oral ABL001 Versus Bosutinib in Patients With Chronic Myelogenous Leukemia in Chronic Phase (CML-CP), Previously Treated With 2 or More Tyrosine Kinase Inhibitors

Acronym

ASCEMBL

Scientific title

A Phase 3, Multi-center, Open-label, Randomized Study of Oral ABL001 Versus Bosutinib in Patients With Chronic Myelogenous Leukemia in Chronic Phase (CML-CP), Previously Treated With 2 or More Tyrosine Kinase Inhibitors

Acronym

Brief summary of the study: English

The purpose of this pivotal study is to compare the efficacy of ABL001 with that of bosutinib in the treatment of patients with CML-CP having previously been treated with a minimum of two prior ATP-binding site TKIs with BCR-ABL ratios $\geq 1\%$ IS at screening.

Brief summary of the study: Arabic

عن طريق الفم مقابل دواء بوسوتينيب لدى المرضى ABL001 مفتوحة اللصاقة، متعددة المراكز حول دواء 3دراسة جزائية في المرحلة المصابين بسرطان الدم النقوي المزمن في المرحلة المزمنة، المعالجين سابقاً بمثبطين أو أكثر لكيناز التيروسين

Health conditions/problem studied: Specify

Chronic Myelogenous Leukemia

Interventions: Specify

ABL001, Bosutinib



**Key inclusion and exclusion criteria: Inclusion criteria**

Male or female patients with a diagnosis of CML-CP ≥ 18 years of age

Patients must meet all of the following laboratory values at the screening visit:

- $< 15\%$ blasts in peripheral blood and bone marrow
- $< 30\%$ blasts plus promyelocytes in peripheral blood and bone marrow
- $< 20\%$ basophils in the peripheral blood
- $\geq 50 \times 10^9/L$ ($\geq 50,000/mm^3$) platelets
- Transient prior therapy related thrombocytopenia ($< 50,000/mm^3$ for ≤ 30 days prior to screening) is acceptable
- No evidence of extramedullary leukemic involvement, with the exception of hepatosplenomegaly

BCR-ABL1 ratio $\geq 1\%$ IS according to central laboratory at the screening examination

Prior treatment with a minimum of 2 prior ATP-binding site TKIs (i.e. imatinib, nilotinib, dasatinib, radotinib or ponatinib)

Failure (adapted from the 2013 ELN Guidelines Bacarrani 2013) or intolerance to the most recent TKI therapy at the time of screening

• Failure is defined for CML-CP patients (CP at the time of initiation of last therapy) as follows. Patients must meet at least 1 of the following criteria.

- Three months after the initiation of therapy: No CHR or $> 95\%$ Ph+ metaphases
- Six months after the initiation of therapy: BCR-ABL1 ratio $> 10\%$ IS and/or $> 65\%$ Ph+ metaphases
- Twelve months after initiation of therapy: BCR-ABL1 ratio $> 10\%$ IS and/or $> 35\%$ Ph+ metaphases
- At any time after the initiation of therapy, loss of CHR, CCyR or PCyR
- At any time after the initiation of therapy, the development of new BCR-ABL1 mutations which potentially cause resistance to study treatment
- At any time after the initiation of therapy, confirmed loss of MMR in 2 consecutive tests, of which one must have a BCR-ABL1 ratio $\geq 1\%$ IS
- At any time after the initiation of therapy, new clonal chromosome abnormalities in Ph+ cells: CCA/Ph+
- Intolerance is defined as:
 - Non-hematologic intolerance: Patients with grade 3 or 4 toxicity while on therapy, 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: Patients 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 recommended by manufacturer

Key inclusion and exclusion criteria: Gender

Both

Key inclusion and exclusion criteria: Specify gender**Key inclusion and exclusion criteria: Age minimum**

18

Key inclusion and exclusion criteria: Age maximum

99

Key inclusion and exclusion criteria: Exclusion criteria

Known presence of the T315I or V299L mutation at 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, including any of the following:

- History within 6 months prior to starting study treatment of myocardial infarction (MI), angina pectoris, coronary artery bypass graft (CABG)
- Clinically significant cardiac arrhythmias
- QTcF at screening ≥ 450 msec (male patients), ≥ 460 msec (female patients)
- Long QT syndrome, family history of idiopathic sudden death or congenital long QT syndrome, or any of the following:
 - Risk factors for Torsades de Pointes (TdP)
 - Concomitant medication(s) with a known risk of Torsades de Pointes per www.qtdrugs.org that cannot be discontinued or replaced 7 days prior to starting study drug by safe alternative medication.
 - Inability to determine the QTcF interval
 - Severe and/or uncontrolled concurrent medical disease
- History of acute pancreatitis within 1 year of study entry or past medical history of chronic pancreatitis
- History of acute or chronic liver disease
- 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 and/or P-gp
- Women of child-bearing potential, unless they are using highly effective methods of contraception during dosing and for 3 days after last dose of ABL001.
- Sexually active males unless they use a condom during intercourse while taking the drug during treatment and for 3 days after stopping treatment and should not father a child in this period. A condom is required to be used also by vasectomized men as well as during intercourse with a male partner in order to prevent delivery of the drug via semen.

Type of study

Interventional

Type of intervention

Pharmaceutical

Type of intervention: Specify type

N/A

| | | |
|--|---|-------------------------------|
| Trial scope | Trial scope: Specify scope | |
| Therapy | N/A | |
| Study design: Allocation | Study design: Masking | |
| Randomized controlled trial | Open (masking not used) | |
| Study design: Control | Study phase | |
| Active | 3 | |
| Study design: Purpose | Study design: Specify purpose | |
| Treatment | N/A | |
| Study design: Assignment | Study design: Specify assignment | |
| Other | 2:1 | |
| IMP has market authorization | IMP has market authorization: Specify | |
| No | | |
| Name of IMP | Year of authorization | Month of authorization |
| ABL001 | | |
| Type of IMP | | |
| Cell therapy | | |
| Pharmaceutical class | | |
| orally bioavailable specific BCR-ABL inhibitor with a novel mechanism of action. | | |
| Therapeutic indication | | |
| patients with Chronic Myelogenous Leukemia-CP who had prior treatment with two or more ATP binding site TKIs | | |
| Therapeutic benefit | | |
| increase OS & PFS | | |
| Study model | Study model: Explain model | |
| N/A | N/A | |
| Study model: Specify model | | |
| N/A | | |
| Time perspective | Time perspective: Explain time perspective | |
| N/A | N/A | |
| Time perspective: Specify perspective | | |
| N/A | | |
| Target follow-up duration | Target follow-up duration: Unit | |
| | | |
| Number of groups/cohorts | | |
| | | |
| Biospecimen retention | Biospecimen description | |
| | | |

Samples without DNA

Bone marrow aspirate samples, Hematology , chemistry , coagulation, hepatitis , Liver function tests , are sent to Covance central laboratory, Navigate biopharma, molecular MD and Histogene X .

Target sample size

5

Actual enrollment target size

3

Date of first enrollment: Type

Actual

Date of first enrollment: Date

05/09/2018

Date of study closure: Type

Actual

Date of study closure: Date

21/12/2022

Recruitment status

Complete

Recruitment status: Specify

Date of completion

31/10/2019

IPD sharing statement plan

Yes

IPD sharing statement description

Novartis is committed to sharing with qualified external researchers, access to patient-level data and supporting clinical documents from eligible studies. These requests are reviewed and approved by an independent review panel on the basis of scientific merit. All data provided is anonymized to respect the privacy of patients who have participated in the trial in line with applicable laws and regulations.

Additional data URL

<https://clinicaltrials.gov/ct2/show/record/NCT03106779?id=cab1001a2301&rank=1>

Admin comments

Trial status

Approved

Secondary Identifying Numbers

| Full name of issuing authority | Secondary identifying number |
|--------------------------------|------------------------------|
| Clinical Trials.Gov | NCT03106779 |

Sources of Monetary or Material Support

| Name |
|-------------------------------|
| Novartis Pharma Services Inc. |



Secondary Sponsors

Name

NA

Contact for Public/Scientific Queries

| Contact type | Contact full name | Address | Country | Telephone | Email | Affiliation |
|--------------|-------------------|------------|---------|------------------------|-------------------------------|--|
| Public | Ali Bazarbach | Beirut | Lebanon | 009613612434 | bazarbac@aub.edu.lb | American University of Beirut Medical Center |
| Scientific | Hind Khairallah | Beirut | Lebanon | +961 1 512002 Ext. 271 | Hind.Khairallah@fattal.com.lb | Khalil Fattal et Fils s.a.l. |
| Public | Joseph Kattan | Beirut | Lebanon | 009613635913 | jkattan62@hotmail.com | Hotel Dieu De France |
| Public | Dany Abi Gerges | Mansourieh | Lebanon | 009613341960 | abgerges@idm.net.lb | Bellevue Medical Center |

Centers/Hospitals Involved in the Study

| Center/Hospital name | Name of principles investigator | Principles investigator speciality | Ethical approval |
|--|---------------------------------|------------------------------------|------------------|
| Bellevue Medical Center | Dr Dany Abi Gerges | Hematology Oncology | Approved |
| American University of Beirut Medical Center | Dr. Ali Bazarbach | Hematology Oncology | Approved |
| Hotel Dieu De France | Dr Joseph Kattan | Hematology Oncology | Approved |

Ethics Review

| Ethics approval obtained | Approval date | Contact name | Contact email | Contact phone |
|--|---------------|-----------------|-----------------------|-----------------------------|
| American University of Beirut Medical Center | 05/06/2018 | Fuad Ziyadeh | fz05@aub.edu.lb | +961 (0) 1 350 000 ext:5445 |
| Hotel Dieu de France | 02/10/2017 | Nancy Alam | nancy.alam@usj.edu.lb | +961 1421000 ext 2335 |
| Bellevue Medical Center | 23/11/2017 | Ghassan Maalouf | gmaalouf@bmc.com.lb | +961 1 682666 ext 7600 |



Countries of Recruitment

| Name |
|--------------------------|
| Lebanon |
| Argentina |
| Australia |
| Belgium |
| Bulgaria |
| Canada |
| Czech Republic |
| France |
| Germany |
| Hungary |
| Italy |
| Japan |
| Republic of Korea |
| Netherlands |
| Turkey |
| United States of America |
| Saudi Arabia |
| United Kingdom |

Health Conditions or Problems Studied

| Condition | Code | Keyword |
|------------------------------|--------------------------------|---------|
| Chronic Myelogenous Leukemia | Leukaemia, unspecified (C95.9) | CML |



Interventions

| Intervention | Description | Keyword |
|---|---|--------------------|
| Physical examination, Vital Sign, Height and weight, ECOG performance status, Laboratory chemistry and hematology, Serology, Electrocardiogram (ECG), Echocardiogram, Pulmonary function tests, PK sampling (full/sparse), Bone Marrow Biopsy, Patient Report Outcomes (MDASI-CML, PGIC, WPAI, EQ-5D-5L, resource | ICF, Lab tests, physical examination, ECG | Lab, ECG, ICF, BMA |

Primary Outcomes

| Name | Time Points | Measure |
|-------------------------------------|-------------|---------|
| Major Molecular Response (MMR) rate | 24 weeks | 24 wks |

Key Secondary Outcomes

| Name | Time Points | Measure |
|-------------------------------------|---|---------------------------|
| Major Molecular Response (MMR) rate | 96 weeks after the last patient received the first study dose | 96 weeks after first dose |
| Complete Cytogenetic response rate | 24,48,96 weeks | 24,48,96 weeks |



Trial Results

Summary results

Study results globally

Date of posting of results summaries

Date of first journal publication of results

Results URL link

Baseline characteristics

Participant flow

Adverse events

Outcome measures

URL to protocol files