



# 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

22/05/2019

### 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 اللصافة، متعددة المراكز حول دواء 3دراسة جزائية في المرحلة المصابين بسرطان الدم النقوي المزمن في المرحلة المزمنة، المعالجين سابقًا بمثبطات 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  $\geq 18$  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  $\leq 30$  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  $\geq 450$  msec (male patients),  $\geq 460$  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](http://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**

Therapy

**Trial scope: Specify scope**

N/A

**Study design: Allocation**

Randomized controlled trial

**Study design: Masking**

Open (masking not used)

**Study design: Control**

Active

**Study phase**

3

**Study design: Purpose**

Treatment

**Study design: Specify purpose**

N/A

**Study design: Assignment**

Other

**Study design: Specify assignment**

2:1

**IMP has market authorization**

No

**IMP has market authorization: Specify****Name of IMP**

ABL001

**Year of authorization****Month of authorization****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**

N/A

**Study model: Explain model**

N/A

**Study model: Specify model**

N/A

**Time perspective**

N/A

**Time perspective: Explain time perspective**

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**

1

**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**

Recruiting

**Recruitment status: Specify**

**Date of completion**

29/02/2020

**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 Bazarbachi	Beirut	Lebanon	009613612434	bazarbac@aub.edu.lb	American University of Beirut Medical Center
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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 Bazarbachi	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**