

MEDIA & INVESTOR RELEASE

Novartis investigational novel STAMP inhibitor asciminib (ABL001) meets primary endpoint of Phase III chronic myeloid leukemia study

- *At primary analysis, ASCEMBL met its primary endpoint of significant superiority in major molecular response rate at 24 weeks for asciminib (ABL001) vs. bosutinib in patients previously treated with two or more tyrosine-kinase inhibitors (TKIs)¹*
- *Despite advances in chronic myeloid leukemia (CML) care, many patients are at risk of disease progression, and sequential TKI therapy may be associated with increased resistance and intolerance²⁻⁷*
- *By specifically targeting the ABL myristoyl pocket, STAMP inhibition has the potential to help address resistance and intolerance in later treatment lines for CML⁸⁻¹⁴; additional studies seek to evaluate asciminib in earlier lines of therapy¹⁵*
- *ASCSEMBL results will be submitted for presentation at an upcoming medical meeting and shared with regulatory authorities; FDA has granted asciminib Fast Track designation*

Basel, August 26, 2020 — Novartis announced today that, at primary analysis, the Phase III ASCSEMBL study met its primary endpoint of statistically significant superiority in major molecular response (MMR) rate at 24 weeks for asciminib (ABL001) vs. bosutinib¹. The study evaluates asciminib – a novel investigational treatment specifically targeting the ABL myristoyl pocket (STAMP) – in adult patients with Philadelphia chromosome-positive chronic myeloid leukemia in chronic phase (Ph+ CML-CP) previously treated with two or more tyrosine-kinase inhibitors (TKIs)¹. Patients with failure or intolerance to the most recently administered TKI therapy were included in the trial¹.

“Our ability to treat patients with TKIs changed CML care forever. However, the risk of disease progression is a reality for many patients – especially those who experience resistance to sequential TKI therapy or those who cannot adhere to treatment due to the daily impact of intolerable side effects¹⁶,” said John Tsai, MD, Head of Global Drug Development and Chief Medical Officer, Novartis. “We are incredibly grateful to the patients and investigators around the world who participated in this study. These results with asciminib are a testament to our commitment to further transform CML care – this time through STAMP inhibition, by exploiting a natural regulatory mechanism of the ABL kinase.”

Data from the ASCEMBL trial will be submitted for presentation at an upcoming medical meeting, and results will be shared with regulatory authorities. The U.S. Food and Drug Administration (FDA) has granted Fast Track designation for asciminib.

Findings from earlier studies of asciminib were presented during past annual meetings of the European Hematology Association, and some details can be found [here](#)^{13,14}.

About asciminib (ABL001)

Asciminib (ABL001) is an investigational treatment specifically targeting the ABL myristoyl pocket (STAMP). As a STAMP inhibitor, asciminib may help address tyrosine-kinase inhibitor (TKI)-resistance and intolerance in later treatment lines of chronic myeloid leukemia (CML)⁸⁻¹⁴, and it is being studied in several clinical trials in hopes of helping patients across multiple treatment lines of CML^{7,10,11,13-15,17}.

About ASCEMBL

ASCEMBL is a Phase III, multicenter, open-label, randomized study comparing the oral investigational treatment asciminib (ABL001) versus bosutinib in patients with Philadelphia chromosome-positive chronic myeloid leukemia in chronic phase (Ph+ CML-CP) previously treated with two or more tyrosine-kinase inhibitors (TKIs)¹. Patients with failure or intolerance to the most recently administered TKI therapy were included in the trial¹.

About Novartis Commitment to CML

Our ongoing research in Philadelphia chromosome-positive chronic myeloid leukemia (Ph+ CML) has helped transform the disease from a fatal leukemia to a chronic condition in most patients. Novartis maintains an unwavering commitment to scientific innovation and access to care for patients worldwide. As an organization committed to patients, Novartis continues to reimagine CML care by pursuing ambitious goals with courage, passion and commitment for the global CML community.

Disclaimer

This press release contains forward-looking statements within the meaning of the United States Private Securities Litigation Reform Act of 1995. Forward-looking statements can generally be identified by words such as “potential,” “can,” “will,” “may,” “could,” “committed,” “commitment,” “investigational,” “continues,” or similar terms, or by express or implied discussions regarding potential marketing approvals, new indications or labeling for asciminib, or regarding potential future revenues from asciminib. You should not place undue reliance on these statements. Such forward-looking statements are based on our current beliefs and expectations regarding future events, and are subject to significant known and unknown risks and uncertainties. Should one or more of these risks or uncertainties materialize, or should underlying assumptions prove incorrect, actual results may vary materially from those set forth in the forward-looking statements. There can be no guarantee that asciminib will be submitted or approved for sale or for any additional indications or labeling in any market, or at any particular time. Nor can there be any guarantee that asciminib will be commercially successful in the future. In particular, our expectations regarding asciminib could be affected by, among other things, the uncertainties inherent in research and development, including clinical trial results and additional analysis of existing clinical data; regulatory actions or delays or government regulation generally; global trends toward health care cost containment, including government, payor and general public pricing and reimbursement pressures and requirements for increased pricing transparency; our ability to obtain or maintain proprietary intellectual property protection; the particular prescribing preferences of physicians and patients; general political, economic and business conditions, including the effects of and efforts to mitigate pandemic diseases such as COVID-19; safety, quality, data integrity or manufacturing issues; potential or actual data security and data privacy breaches, or disruptions of our information technology systems, and other risks and factors referred to in Novartis AG’s current Form 20-F on file with the US Securities and Exchange Commission. Novartis is providing the information in this press release as of this date and does not undertake any obligation to update any forward-looking statements contained in this press release as a result of new information, future events or otherwise.

About Novartis

Novartis is reimagining medicine to improve and extend people's lives. As a leading global medicines company, we use innovative science and digital technologies to create transformative treatments in areas of great medical need. In our quest to find new medicines, we consistently rank among the world's top companies investing in research and development. Novartis products reach nearly 800 million people globally and we are finding innovative ways to expand access to our latest treatments. About 109,000 people of more than 140 nationalities work at Novartis around the world. Find out more at <https://www.novartis.com>.

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