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IR & MEDIA UPDATE

Novartis receives FDA Breakthrough Therapy designations for investigational STAMP inhibitor asciminib (ABL001) in chronic myeloid leukemia

- *Designation in patients with chronic myeloid leukemia (CML) resistant or intolerant to prior treatments based on positive data from pivotal Phase III ASCEMBL trial evaluating asciminib, an investigational treatment specifically targeting the ABL myristoyl pocket (STAMP)*[1,2]

- *Despite advances in CML care, many patients are at risk of disease progression, and sequential TKI therapy may be associated with increased resistance and intolerance*[3-9]

- *Breakthrough Therapy designation is granted to medicines being evaluated for serious conditions where early clinical evidence indicates the potential for substantial improvement over available therapy*[10]

- *Asciminib is in development across multiple treatment lines of CML; first regulatory filing in pre-treated patients anticipated in first half of 2021 under the US FDA Real-Time Oncology Review program*[11-17]

Basel, February 8, 2020 — Novartis today announced that asciminib – a novel investigational treatment specifically targeting the ABL myristoyl pocket (STAMP) – has been granted Breakthrough Therapy designation (BTD) by the US Food and Drug Administration (FDA) for the treatment of adult patients with Philadelphia chromosome-positive chronic myeloid leukemia (Ph+ CML) in chronic phase (CP), previously treated with two or more tyrosine kinase inhibitors (TKIs). Asciminib was also granted BTD for the treatment of adult patients with Ph+ CML in CP harboring the T315I mutation.

Despite tremendous advances in CML treatment over the past few decades, some of these pre-treated patients struggle to meet treatment goals due to resistance and intolerance[18-23]. With few remaining treatment options, patients in later lines of care may be at risk of progression[3-9].

These FDA designations, which may allow for an expedited development and review of asciminib, were based on:

- The pivotal, Phase III ASCEMBL trial, where asciminib was compared to Bosulif® (bosutinib)* in patients with Ph+ CML in CP previously treated with two or more TKIs[1,2]

- A Phase I trial that included patients with Ph+ CML, some of them harboring the T315I mutation[24]

Data from these trials were shared at the 2020 Annual Meeting of the American Society of Hematology (ASH), and details on positive findings can be found here.

The FDA previously granted Fast Track designation to asciminib, and Novartis plans for a submission in the first half of 2021 for review under the FDA Oncology Center of Excellence Real-Time Oncology Review program.

About asciminib (ABL001)
Asciminib (ABL001) is an investigational treatment specifically targeting the ABL myristoyl pocket (STAMP)[11-17]. As a STAMP inhibitor, asciminib is being studied in patients with chronic myeloid leukemia (CML) who experience resistance or intolerance to two or more tyrosine-kinase inhibitors (TKIs), and in several clinical trials in hopes of helping patients across multiple treatment lines of CML[11-17, 25-32].

About ASCEMBL
ASCEMBL is the first head-to-head clinical trial in chronic myeloid leukemia using a second-generation tyrosine-kinase inhibitor (TKI) as a comparator. As a Phase III, multicenter, open-label, randomized study, ASCEMBL was designed to evaluate superiority in major molecular response rate at 24 weeks of the oral investigational treatment asciminib (ABL001) versus bosutinib in patients with Philadelphia-chromosome positive CML in chronic phase previously treated with two or more TKIs[2]. Patients with failure or intolerance to the most recently administered TKI therapy were included in the trial[2].

Disclaimer
This media update 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,” “plan,” “may,” “could,” “would,” “expect,” “anticipate,” “look forward,” “believe,” “committed,” “investigational,” “pipeline,” “launch,” or similar terms, or by express or implied discussions regarding potential marketing approvals, new indications or labeling for the investigational or approved products described in this media update, or regarding potential future revenues from such products. 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 the investigational or approved products described in this media update 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 such products will be commercially successful in the future. In particular, our expectations regarding such products 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 media update as of this date and does not

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*Bosulif is a registered trademark of Pfizer.

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