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MEDIA & INVESTOR RELEASE

Novartis receives positive CHMP opinion for Scemblix®, a novel treatment for adult patients with chronic myeloid leukemia

- *With unique STAMP mechanism of action, Scemblix could provide a new option for patients in Europe with chronic myeloid leukemia (CML) who have suffered intolerance or inadequate response with at least two prior tyrosine kinase inhibitor treatments*[1]

- *Positive opinion based on data from pivotal Phase III ASCEMBL trial, showing a near doubling in major molecular response rate for patients treated with Scemblix® (asciminib) vs. Bosulif®* (bosutinib) (25.5% vs. 13.2%) and more than three times lower discontinuation rate due to adverse reactions (5.8% vs 21.1%) at 24 weeks*[1]

- *CHMP recommendation comes after the approval of Scemblix by the US FDA and other countries' regulatory authorities, potentially broadening access for more patients to Novartis transformative therapies in CML*

Basel, June 24, 2022 — Novartis today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has adopted a positive opinion and recommended granting marketing authorization for Scemblix® (asciminib) for the treatment of 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). If approved, Scemblix will be the first CML treatment in Europe that works by specifically targeting the ABL myristoyl pocket (also known as a STAMP inhibitor in scientific literature), representing an important therapeutic advancement for patients who experience intolerance and/or resistance to currently available TKI therapies[1].

It is estimated that, every year, more than 6,300 people will be diagnosed with CML in Europe[2]. While many patients will benefit from available TKI therapies, a significant proportion may experience intolerance or resistance to these treatments[3-10]. In an analysis of patients with CML treated with two prior TKIs, approximately 55% reported intolerance to previous treatment; and a pooled analysis of patients in the second-line setting revealed that up to 70% are unable to achieve major molecular response (MMR) within two years of follow-up[11-14].

"Although CML treatments have advanced over the last 20 years, many patients continue to experience side effects and resistance to treatment, affecting their quality of life and putting them at risk of disease progression or even death," says Dr. Andreas Hochhaus, Head of the Department of Hematology and Medical Oncology at Jena University Hospital in Germany. "If approved, the novel mechanism of action of Scemblix brings us another option to combat these challenges faced by patients — offering new hope in the management of their disease."

The positive CHMP opinion for Scemblix is based on results from the pivotal Phase III ASCEMBL trial, which showed a near doubling of MMR rate for patients treated with Scemblix vs. Bosulif®* (bosutinib) (25.5% vs. 13.2%) at 24-weeks, with a more than three times lower discontinuation rate due to adverse reactions (5.8% vs. 21.1%)[1]. The most common (incidence ≥ 20%) adverse reactions reported in this analysis were thrombocytopenia (29.5%) and neutropenia (23.1%) in the Scemblix arm; and diarrhea (71.1%), nausea (46.1%), increased ALT (28.9%), vomiting (26.3%), rash (23.7%), increased AST (21.1%) and neutropenia (21.1%) in the Bosulif arm[1].

These results were confirmed in longer-term follow-up, where the MMR rate at week 96 was more than double with Scemblix (37.6%, 95% CI: 29.99-45.65) compared with Bosulif (15.8%, 95% CI: 8.43-25.96). This data was shared at oral presentations during the American Society for Clinical Oncology (ASCO) and the European Hematology Association (EHA) annual meetings in June 2022[15,16].

“We are pleased with the recommendation of Scemblix and hope to offer patients living with CML in Europe timely access to this innovative therapy, if approved,” said Haseeb Ahmad, President, Europe, Novartis. “We’ve worked relentlessly to improve CML care over the past two decades, and must seize this opportunity to help patients in need achieve better outcomes. With the strong clinical results seen to-date, we believe we have the potential to transform the standard of care in CML yet again with Scemblix.”

The CHMP recommended approval of Scemblix in CML will be referred to the European Commission (EC). The EC will review the CHMP recommendations and deliver a final decision in the coming months.

About Scemblix® (asciminib)
Scemblix is the first CML treatment that acts as a STAMP inhibitor, specifically targeting the ABL myristoyl pocket[1]. This novel mechanism of action may help address resistance in patients with CML previously treated with two or more TKIs and overcome mutations at the defective BCR::ABL1 gene, which is associated with the over-production of leukemic cells[1,17-23].

Scemblix represents an important development for patients who experience resistance and/or intolerance to currently available TKI therapies, and it is being studied across multiple treatment lines for CML-CP, both as a monotherapy and in combination[1,17-31]. Specifically, the ASC4FIRST Phase III study (NCT04971226) evaluates Scemblix in newly diagnosed adult patients with Ph+ CML-CP vs. an investigator-selected TKI, with recruitment proceeding ahead of plan[25].

Novartis has initiated regulatory filings for Scemblix in multiple countries and regions across the globe. In October 2021, the US FDA granted accelerated approval of Scemblix for adult patients with Ph+ CML-CP, previously treated with two or more TKIs based on MMR rate at 24 weeks, and full approval for adult patients with Ph+ CML-CP with the T315I mutation. In accordance with the Accelerated Approval Program, continued approval for the first indication may be contingent upon verification and description of clinical benefit from confirmatory evidence. Further data has been shared with the FDA for evaluation [32]. Scemblix has received approval in several countries outside the US for adult patients with Ph+ CML-CP with resistance or intolerance to at least two or more previous therapies.

About Novartis Commitment to CML
Novartis has a long-standing scientific commitment to patients living with CML. For more than 20 years, our bold science has helped transform CML into a chronic disease for many patients. Despite these advancements, we’re not standing still. We continue to research ways to target the disease, seeking to address the challenges with treatment resistance and/or intolerance that many patients face. Novartis also continues to reimagine CML care through

its commitment to sustainable access for patients and collaboration with the global CML community.

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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 108,000 people of more than 140 nationalities work at Novartis around the world. Find out more at https://www.novartis.com.

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