Novartis receives FDA Breakthrough Therapy designation for Scemblix® in 1L CML

May 10, 2024

Novartis announced that the FDA has granted Breakthrough Therapy designation to Scemblix® (asciminib) for the treatment of adult patients with newly diagnosed Philadelphia chromosome-positive chronic myeloid leukemia in chronic phase (Ph+ CML-CP). This marks the third[1] Breakthrough Therapy designation for Scemblix.

- According to FDA guidelines, treatments that receive Breakthrough Therapy designation must target a serious or life-threatening disease and demonstrate a potential substantial improvement over existing therapies on one or more clinically significant endpoints.
- Breakthrough Therapy designation was granted based on positive data from the Phase III ASC4FIRST study, in which Scemblix met both primary endpoints with superior MMR rates at week 48 compared to investigator-selected TKIs (imatinib, nilotinib, dasatinib and bosutinib) and compared to imatinib alone. Scemblix also demonstrated a favorable safety and tolerability profile with fewer adverse events (AEs) and treatment discontinuations vs. standard-of-care TKIs.
- With current standard-of-care TKIs, approximately half[2] of newly diagnosed patients with CML fail to meet molecular response goals at one year, and many discontinue or change treatment due to intolerance.

Full results of the ASC4FIRST study will be presented at ASCO on Friday, May 31. Novartis is also hosting an in-person investor event in Chicago on Sunday, June 2, to delve deeper into the results and the potential commercial opportunity for Scemblix in 1L CML upon regulatory approval.

Overall, Novartis has received 30 approvals for Breakthrough Therapy designated drugs, reflecting our track record of innovation.

References:

1. Scemblix previously received FDA Breakthrough Therapy designation for the treatment of adult patients with Ph+ CML-CP previously treated with two or more TKIs, as well as adult patients with Ph+ CML-CP harboring the T315I mutation.
2. Cortes JE, et al. J Clin Oncol. 2016;34(20):2333-2340; Hochhaus A, et al. Leukemia.2016;30(5):1044-1054; Brümmendorf TH, et al. Leukemia. 2022;36:1825-1833.