OPTUMRx®

## Scemblix® (asciminib) – New orphan drug approval

- On October 29, 2021, Novartis announced the FDA approval of Scemblix (asciminib), 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). This indication is approved under accelerated approval based on major molecular response (MMR). Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).
  - Ph+ CML in CP with the T315I mutation.
- Scemblix is the first approved CML treatment that binds to the ABL myristoyl pocket. This may help address resistance in patients with CML previously treated with two or more TKIs and overcome mutations at the defective BCR-ABL gene, which is associated with the over-production of leukemic cells.
- The efficacy of Scemblix was established in a randomized, active-controlled, and open-label study in 233 patients with Ph+ CML-CP, previously treated with two or more TKIs. Patients received either Scemblix or Bosulif® (bosutinib) until unacceptable toxicity or treatment failure occurred. The primary endpoint was major molecular response (MMR).
  - At 24 weeks, the MMR rate was 25% with Scemblix vs. 13% with Bosulif (treatment difference 12, 95% CI: 2.2, 22; p = 0.029). The MMR rate at 48 weeks was 29% in patients receiving Scemblix and 13% in patients receiving Bosulif.
- The efficacy of Scemblix was also evaluated in an open-label study in 45 patients with Ph+ CML-CP with the T315I mutation. Patients received Scemblix until unacceptable toxicity or treatment failure occurred. The primary endpoint was MMR.
  - MMR was achieved by 24 weeks in 42% (95% CI: 28% to 58%) of the 45 patients treated with Scemblix. MMR was achieved by 96 weeks in 49% (95% CI: 34% to 64%) of patients.
- Warnings and precautions for Scemblix include myelosuppression, pancreatic toxicity, hypertension, hypersensitivity, cardiovascular toxicity, and embryo-fetal toxicity.
- The most common adverse reactions (≥ 20%) with Scemblix use were upper respiratory tract infections, musculoskeletal pain, fatigue, nausea, rash, and diarrhea. The most common laboratory abnormalities (≥ 20%) were decreased platelet count, increased triglycerides, decreased neutrophil count, decreased hemoglobin, increased creatine kinase, increased alanine aminotransferase, increased lipase, and increased amylase.
- For Ph+ CML-CP (previously treated with two or more TKIs), the recommended dose of Scemblix is 80 mg taken orally once daily at approximately the same time each day or 40 mg twice daily at approximately 12-hour intervals. Treatment should be continued as long as clinical benefit is observed or until unacceptable toxicity occurs.
- For Ph+ CML-CP with the T315I mutation, the recommended dose of Scemblix is 200 mg taken orally twice daily at approximately 12-hour intervals.

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- Novartis plans to launch Scemblix immediately. Scemblix will be available as 20 mg and 40 mg tablets.

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