Asciminib: Adis Evaluation

Key Points

- Selective allosteric inhibitor targeting the myristoyl pocket of BCR-ABL1 being developed by Novartis for the treatment of haematological malignancies, including Ph+ CML
- Received its first approval on 29 October 2021 in the USA
- Approved for the treatment of adult patients with Ph+ CML-CP, previously treated with ≥ 2 TKIs, and Ph+ CML-CP with the T315I mutation

Summary

Asciminib (Scemblix®) is an orally administered, small molecule, selective allosteric inhibitor that targets the myristoyl pocket of the BCR-ABL1 tyrosine kinase and is being developed by Novartis for the treatment of haematological malignancies, including Philadelphia chromosome-positive (Ph+) chronic myeloid leukaemia (CML).

The drug is active against a number of the single catalytic-site mutations, such as T315I, that confer resistance to conventional tyrosine kinase inhibitors (TKIs) that bind to the ATP-binding site of BCR-ABL1.

In October 2021, asciminib monotherapy was granted accelerated approval for the treatment of adults with Ph+ CML in chronic phase (CML-CP), previously treated with ≥ 2 TKIs, and full approval for the treatment of adults with Ph+ CML-CP with the T315I mutation. The drug is under regulatory review for use as monotherapy in CML in the EU, and is in phase 1–3 development exploring its potential in first-line, later-line and paediatric patients with CML.

Deeks, ED. Drugs. 2021. https://doi.org/10.1007/s40265-021-01662-3