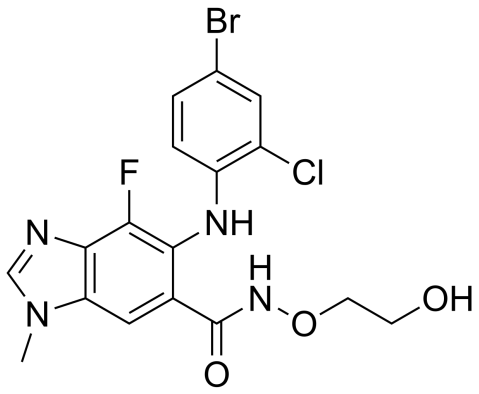
Brand Name: Koselugo
Generic: selumetinib
Type: small molecule
Year Accepted/Phase: 2020

Mechanism:

Selumetinib is a selective inhibitor of MEK1 and MEK2, which are proteins involved in the MAPK signaling pathway. Dysregulation of this pathway is associated with the development of PNs in patients with NF1. By inhibiting MEK1 and MEK2, selumetinib helps to reduce the size and symptoms of PNs.

Chemical Structure:



Indication:

Koselugo is indicated for the treatment of pediatric patients aged 2 years and older with NF1 with symptomatic, inoperable PNs.

Clinical trials:

Clinical Trials for NF1 and Plexiform Neurofibromas

Pubmed: https://pubmed.ncbi.nlm.nih.gov/32187457/
Purpose: Evaluate the efficacy and safety of selumetinib in pediatric patients with NF1 and symptomatic, inoperable PNs.
Dates: Clinical trials for Koselugo were conducted in the 2010s.
Results: These trials demonstrated that selumetinib reduced the size of PNs, improved symptoms related to PNs, and showed a favorable safety profile in pediatric patients with NF1.
Impact: The positive results from these trials supported the approval of Koselugo for the treatment of NF1 with symptomatic, inoperable PNs in pediatric patients.