

Brand Name: Welireg

Generic: belzutifan

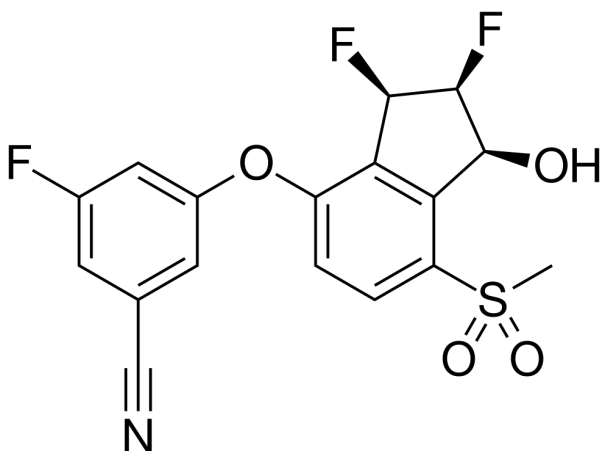
Type: small molecule

Year Accepted/Phase: 2021

Mechanism:

Belzutifan inhibits hypoxia-inducible factor-2 alpha (HIF-2 α), a transcription factor that plays a key role in the response of cancer cells to hypoxic conditions. By inhibiting HIF-2 α , belzutifan reduces the expression of genes involved in tumor growth, angiogenesis, and metabolism.

Chemical Structure:



Indication:

Welireg is indicated for the treatment of adult patients with von Hippel-Lindau (VHL) disease who require therapy for associated renal cell carcinoma (RCC), central nervous system (CNS) hemangioblastomas, or pancreatic neuroendocrine tumors (pNET), not requiring immediate surgery.

Clinical trials:

Study 004 (Phase II)

Pubmed: <https://pubmed.ncbi.nlm.nih.gov/38393723/>

Purpose: Evaluate the efficacy and safety of belzutifan in patients with von Hippel-Lindau (VHL) disease-associated renal cell carcinoma (RCC) and other VHL-associated tumors.

Dates: Conducted from 2018 to 2020.

Results: Study 004 demonstrated a high response rate in patients with VHL-associated RCC, with an overall response rate (ORR) of 49% in RCC tumors. The trial also showed responses in other VHL-associated tumors, including CNS hemangioblastomas and pancreatic neuroendocrine tumors. The median duration of response was not reached at the time of analysis, indicating sustained responses.

Impact: The results of Study 004 supported the approval of belzutifan for the treatment of VHL-associated RCC and other related tumors, providing a new therapeutic option for patients with this rare genetic disorder.