Non-interventional post-authorization study of belzutifan in adult patients with von Hippel-Lindau disease-associated renal cell carcinoma, pancreatic neuroendocrine tumor and/or central nervous system hemangioblastoma (MK-6482-026)

First published: 19/01/2024

Last updated: 03/01/2025

Study Ongoing

Administrative details

PURI

https://redirect.ema.europa.eu/resource/199007

EU PAS number

EUPAS108114

Study ID

199007

DARWIN EU® study

No

Study countries

☐ United States

Study description

Von Hippel-Lindau (VHL) disease is a rare autosomal dominant disease characterized by an increased prevalence of recurring benign and malignant tumors including renal cell carcinomas (RCCs), central nervous system (CNS) hemangioblastomas, and pancreatic neuroendocrine tumors (pNETs). The only systemic therapy approved for the treatment of certain patients with VHL disease-associated neoplasms is belzutifan (WELIREG®), which was initially approved by the United States (US) Food and Drug Administration (FDA) in August 2021 for the treatment of adult patients with VHL disease who require therapy for associated RCC, CNS hemangioblastoma, or pNET, not requiring immediate surgery. The primary aim of this registry is to further characterize the effectiveness of belzutifan for patients with von Hippel-Lindau (VHL) disease-associated renal cell carcinoma (RCC) and/or central nervous system (CNS) hemangioblastoma treated in real-world clinical practice. The primary effectiveness parameter includes tumor reductive procedures given the clinical importance of this parameter and association with subsequent morbidity and mortality of disease. Secondary aims are to describe potential serious adverse events (SAEs), occurrence of new VHL disease-associated tumors or tumor type, and metastasis during belzutifan use, and to evaluate treatment patterns.

Study status

Ongoing

Research institutions and networks

Institutions

Merck & Co.

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Institution

Contact details

Study institution contact

Clinical Trials Disclosure Merck Sharp & Dohme LLC

Study contact

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Primary lead investigator

Clinical Trials Disclosure Merck Sharp & Dohme LLC

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 21/07/2022

Actual: 21/07/2022

Study start date

Planned: 31/07/2024

Actual: 30/07/2024

Data analysis start date
Planned: 30/09/2029

Date of interim report, if expected
Planned: 30/06/2027

Date of final study report
Planned: 31/12/2030

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Merck Sharp & Dohme LLC

Study protocol

MK-6482-026-00-v3-with-ha-approval-date_Protocol_redaction-final.pdf(1.91 MB)

Regulatory

Was the study required by a regulatory body?

Yes

Is the study required by a Risk Management Plan (RMP)?

Non-EU RMP only

Other study registration identification numbers and links

NAT/H/0087/II/009

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Effectiveness study (incl. comparative)

Main study objective:

The study aims to evaluate the treatment effectiveness and long-term safety of patients treated with belzutifan in routine clinical practice.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Study drug International non-proprietary name (INN) or common name

BELZUTIFAN

Medical condition to be studied

Von Hippel-Lindau disease

Renal cell carcinoma

Haemangioblastoma

Pancreatic neuroendocrine tumour

Population studied

Short description of the study population

The target sample size includes approximately 40 eligible patients with VHL disease-associated RCC and approximately 40 patients with VHL disease-associated CNS hemangioblastoma requiring treatment. Enrollment of patients with each tumor type will be stopped once the target sample size is reached with overall recruitment stopped once the sample size requirement for both tumor types is met.

Age groups

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Adults (65 to < 75 years)

Adults (75 to < 85 years)

Adults (85 years and over)

Estimated number of subjects

100

Study design details

Outcomes

The primary objectives are to describe the proportion of patients who undergo at least one renal tumor reductive surgery (e.g., nephrectomy) or locally directed therapy (e.g., radiofrequency ablation) and to describe the proportion of patients who undergo at least one CNS tumor reductive surgery (e.g., craniectomy) or locally directed therapy (e.g., radiation therapy).
Secondary objectives are to describe proportion of patients with treatment emergent SAEs, evaluate treatment patterns, and for participants with VHL disease-associated RCC and, separately, VHL disease-associated CNS hemangioblastoma, to describe the proportion of patients who develop metastatic disease (for RCC only), and proportion with occurrence of new VHL tumors or tumor type.

Data analysis plan

Participant data for those who undergo surgery or other tumor reductive procedures, development of metastatic disease for RCC or VHL disease-associated study conditions will be summarized descriptively using frequency of counts or descriptive statistics such as n, mean, 95% CI of the mean, SD, median, minimum, maximum separately for VHL disease-associated RCC and VHL disease-associated CNS hemangioblastoma.

Data management

Data sources

Data sources (types)

Other

Data sources (types), other

Prospective patient-based data collection,Prospectively enrolled treatment registry

Use of a Common Data Model (CDM)

CDM mapping

No

Data quality specifications

Check conformance

Unknown

Check completeness

Unknown

Check stability

Unknown

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

No