

# Real-World Effects and Utilisation Patterns of Elexacaftor, Tezacaftor, and Ivacaftor Combination Therapy (ELX/TEZ/IVA) in Patients with Cystic Fibrosis (CF)

**First published:** 15/09/2021

**Last updated:** 03/03/2025

Study

Ongoing

## Administrative details

### PURI

<https://redirect.ema.europa.eu/resource/45404>

### EU PAS number

EUPAS43022

### Study ID

45404

### DARWIN EU® study

No

## Study countries

☐ Germany

☐ United States

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## Study description

Cystic fibrosis (CF) is an autosomal recessive disease with serious, chronically debilitating morbidities, and high premature mortality. ELX/TEZ/IVA is currently indicated for treatment of CF in patients 12 years and older in the EU who have specified CFTR mutations. This 5-year observational post-authorisation safety study (PASS) will evaluate safety, effectiveness / CF disease progression, and pregnancy outcomes in patients with CF who are treated with ELX/TEZ/IVA, as well as its drug utilisation patterns using observational cohorts of patients receiving therapy in a real-world setting. Existing CF registries provide an established source to obtain data on long term effects in real world use for analysis. In the US Cystic Fibrosis Foundation Patient Registry (CFFPR) and German CF Registry, within-cohort evaluation of outcomes in the 5-year periods before and after treatment initiation will be performed. Evaluation of the outcome patterns and trends in the 5-year pre-treatment period will place into context the outcome patterns and trends observed in the post-treatment period. In addition, the European Cystic Fibrosis Society Patient Registry (ECFSPR) will be used to provide additional information for the evaluation of drug utilisation patterns in the European region. Information regarding the safety profile of the therapy under the real-world conditions of use will be informative to patients, caregivers, and prescribers. Existing CF registries provide an established source from which to obtain these data.

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## Study status

Ongoing

## Research institutions and networks

## Institutions

### Vertex Pharmaceuticals

**First published:** 01/02/2024

**Last updated:** 01/02/2024

Institution

### Mukoviszidose Institut

☐ Germany

**First published:** 01/02/2024

**Last updated:** 01/02/2024

Institution

Patient organisation/association

### European Cystic Fibrosis Society (ECFS)

**First published:** 01/02/2024

**Last updated:** 01/02/2024

Institution

Educational Institution

German CF Register Germany, ECFSPR European region (multiple countries), US CFF Patient

## Contact details

### Study institution contact

Vertex Pharmaceuticals Global Medical Information

Study contact

[vertexmedicalinfo@vrtx.com](mailto:vertexmedicalinfo@vrtx.com)

### Primary lead investigator

Julie Bower

Primary lead investigator

## Study timelines

### Date when funding contract was signed

Planned: 31/08/2021

Actual: 31/08/2021

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### Study start date

Planned: 31/08/2021

Actual: 31/08/2021

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### Date of interim report, if expected

Planned: 31/12/2021

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### Date of final study report

Planned: 31/12/2025

## Sources of funding

- Pharmaceutical company and other private sector

## More details on funding

Vertex Pharmaceuticals Incorporated

## Study protocol

[ELX-TEZ-IVA PASS Protocol\\_Version 2.0\\_redacted.pdf.pdf](#)(674.84 KB)

## Regulatory

**Was the study required by a regulatory body?**

Yes

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

EU RMP category 3 (required)

## Methodological aspects

### Study type

### Study type list

**Study type:**

Non-interventional study

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**Scope of the study:**

Assessment of risk minimisation measure implementation or effectiveness

Drug utilisation

Effectiveness study (incl. comparative)

**Main study objective:**

To evaluate, among patients treated with ELX/TEZ/IVA in the real-world setting:

1. Safety outcomes 2. Effectiveness outcomes / CF disease progression 3.

Safety and effectiveness outcomes/ CF disease progression in genotype

subgroups 4. Frequency and outcome of pregnancy in female patients 5. Drug

utilisation patterns and characterise potential off-label use outside of the

labelled indication

## Study Design

**Non-interventional study design**

Cohort

## Study drug and medical condition

**Name of medicine**

KAFTRIO

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**Medical condition to be studied**

Cystic fibrosis

## Population studied

## **Age groups**

Children (2 to < 12 years)

Adolescents (12 to < 18 years)

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)

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## **Special population of interest**

Hepatic impaired

Immunocompromised

Pregnant women

Renal impaired

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## **Estimated number of subjects**

21000

# Study design details

## **Outcomes**

Safety analyses: death, organ transplant, hospitalisations, pulmonary exacerbations, CF complications, respiratory microbiology, liver function tests

Disease progression analyses: Percent predicted FEV1, BMI Pregnancy analyses: pregnancy outcome, gestational age, congenital anomalies (data availability varies by registry) Drug utilization analyses: ELX/TEZ/IVA use outside of labeled indications

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## **Data analysis plan**

Data will be analysed separately for each registry over the course of the 5 year study. Results of analyses will be presented in annual study reports. Each annual report will include patient data collected through the end of the previous calendar year. Descriptive statistics will be presented for all study outcomes. Continuous variables will be summarised using the following descriptive summary statistics where appropriate: the number of observations (n), mean, SD, SE, 95% CI, median, minimum value, maximum value, and 25th and 75th percentile values. Categorical variables will be summarised using counts, percentages, and 95% CIs as appropriate. All safety outcomes, effectiveness / CF disease progression outcomes, and pregnancy outcomes will be evaluated in the ELX/TEZ/IVA Cohorts in the US CFFPR and German CF registry. In addition to these 2 registries, the ECFSPR will be used to provide additional information for the evaluation of drug utilisation patterns in the European region.

## **Data management**

### **Data sources**

#### **Data sources (types)**

[Disease registry](#)

### **Use of a Common Data Model (CDM)**

#### **CDM mapping**

No

### **Data quality specifications**

**Check conformance**

Unknown

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**Check completeness**

Unknown

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**Check stability**

Unknown

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**Check logical consistency**

Unknown

## Data characterisation

**Data characterisation conducted**

No