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Real-world outcomes of patients with HER2-positive biliary tract cancer treated with zanidatamab through an Early Access Programme

Submission date
10/08/2026

Recruitment status
No longer recruiting

Registration date
10/08/2026

Overall study status
Completed

Last Edited
10/08/2026

Condition category
Cancer

[_] Prospectively registered
[_] Protocol
[_] Statistical analysis plan
[_] Results
[_] Individual participant data
[X] Record updated in last year

Plain English summary of protocol

Background and study aims
Biliary tract cancer (BTC) is a rare and aggressive cancer affecting the gallbladder and bile ducts. Many patients are diagnosed when the disease is advanced and treatment options are limited. Zanidatamab is a medicine that targets HER2-positive cancers and has shown promising results in clinical trials. Before becoming widely available, some patients in the United Kingdom, Spain and Italy received zanidatamab through an Early Access Programme. This study aims to understand how zanidatamab is used and how effective it is in routine clinical practice by analysing information collected during normal patient care.

Who can participate?
Adults aged 18 years or older with previously treated unresectable locally advanced or metastatic HER2-positive biliary tract cancer who received at least one dose of zanidatamab through the Early Access Programme in the United Kingdom, Spain or Italy.

What does the study involve?
This is a retrospective observational study using existing medical records. No additional treatments, tests, procedures or study visits are required. Researchers will collect pseudonymised information from patient medical records, including demographic characteristics, medical history, cancer characteristics, details of zanidatamab treatment, tumour response, disease progression, subsequent treatments and survival outcomes. The information will be used to evaluate the real-world effectiveness of zanidatamab and to better understand how it is used in clinical practice.

What are the possible benefits and risks of participating?
Participants will not receive any direct medical benefit from taking part because no additional treatment or assessments are involved. However, the findings may improve understanding of zanidatamab and help inform future treatment decisions for patients with HER2-positive biliary tract cancer. The main risk is a potential loss of confidentiality. To minimise this risk, only

pseudonymised data will be used for research purposes and identifiable information will remain securely protected at participating sites.

Where is the study run from?
The study is sponsored by Jazz Pharmaceuticals UK Ltd and is being conducted at participating cancer centres in the United Kingdom, Spain and Italy.

When is the study starting and how long is it expected to run for?
August 2026 to October 2026

Who is funding the study?
Jazz Pharmaceuticals UK Ltd

Who is the main contact?
Prof. John Bridgewater, j.bridgewater@ucl.ac.uk

Plain English summary under review with external organisation

Contact information

Type(s)
Principal investigator

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Type(s)
Scientific, Public

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Additional identifiers

Integrated Research Application System (IRAS)
364497

Central Portfolio Management System (CPMS)
70928

Sponsor Protocol ID
JZP598-527

Study information

Scientific Title
Multinational real-world study of patients with HER2-positive biliary tract cancer treated with zanidatamab in an Early Access Program (RealiZe)

Acronym
RealiZe

Study objectives
1. To evaluate the real-world effectiveness of zanidatamab in patients with previously treated unresectable locally advanced or metastatic HER2-positive biliary tract cancer who received treatment through an Early Access Programme.
2. To characterise treatment patterns and utilisation of zanidatamab in routine oncology practice, including treatment duration, dose modifications, treatment interruptions and reasons for discontinuation.
3. To describe baseline demographic and clinical characteristics of patients receiving zanidatamab through the Early Access Programme, including disease characteristics, HER2 status, prior treatments and performance status.
4. To describe clinical outcomes following zanidatamab treatment, including tumour response, progression-free survival, overall survival, duration of response, time to next treatment and outcomes of subsequent anticancer therapies.

Ethics approval required
Ethics approval required

Ethics approval(s)
1. Approved 23/03/2026, Health and Social Care Research Ethics Committee B (HSC REC B) (Office for Research Ethics Committees Northern Ireland (ORECNI) 2nd Floor James House, 2-4 Cromac Avenue, BELFAST, BT7 2JA, United Kingdom; +44 (0)28 95 361400; PRS@hscni.net), ref: 26/NI/0041

2. Approved 26/04/2026, Comité de Ética de la Investigación de la Fundación Jiménez Díaz (CEIm) (C/ del Maestro Ángel Llorca, 6 Bajo B, Edificio Alto, Madrid, 28003, Spain; +34 (0)91 544 37 20; ceic@fjd.es), ref: EO093-26_FJD

3. Submitted 14/07/2026, Comitato Etico Territoriale Lombardia 5 (Via Alessandro Manzoni 56, Rozzano (MI), 20089, Italy; +39 (0)2 8224 7216; cetlombardia5@humanitas.it), ref: 436/26

Primary study design
Observational

Secondary study design
Cohort study

Study type(s)

Health condition(s) or problem(s) studied
HER2-positive biliary tract cancer (BTC), including unresectable locally advanced or metastatic disease

Interventions
This is a multinational, retrospective, longitudinal, observational cohort study conducted in the United Kingdom, Spain and Italy. Patients with previously treated unresectable locally advanced or metastatic HER2-positive biliary tract cancer who received at least one dose of zanidatamab through an Early Access Programme will be included.

No treatments, interventions, study visits, tests, questionnaires or biological samples will be required as part of the study. Pseudonymised data will be collected retrospectively from existing medical records at participating cancer centres and entered into a standardised electronic case report form. Medical records covering medical history, zanidatamab treatment and subsequent follow-up will be reviewed.

Data collected will include demographic and clinical characteristics, biliary tract cancer subtype, HER2 test results, ECOG performance status, relevant comorbidities, previous anticancer therapies, zanidatamab treatment details (including treatment dates, dose modifications, interruptions and reasons for discontinuation), concomitant medications, tumour response, disease progression, subsequent anticancer treatments and survival status.

Real-world effectiveness outcomes assessed from medical records will include real-world time on treatment (rwToT), real-world progression-free survival (rwPFS), real-world subsequent-line progression-free survival (rwPFS2), real-world overall survival (rwOS), real-world duration of response (rwDOR), and real-world objective response rate (rwORR). Patients will be followed from initiation of zanidatamab treatment until treatment discontinuation, death, or the end of available follow-up.

Intervention Type
Drug

Phase
Not Applicable

Drug/device/biological/vaccine name(s)

Zanidatamab

Primary outcome(s)
1. Real-world time on treatment (rwToT) measured using the time from zanidatamab treatment initiation to treatment discontinuation for any reason, including physician-assessed disease progression, treatment-related toxicity, clinical decision, or death, derived from patient medical records and recorded in the electronic case report form (eCRF) at the end of the available follow-up

2. Real-world progression-free survival (rwPFS) measured using the time from zanidatamab treatment initiation to physician-assessed disease progression or death from any cause, whichever occurs first, derived from patient medical records and recorded in the eCRF at the end of the available follow-up

3. Real-world subsequent-line progression-free survival (rwPFS2) measured using the time from zanidatamab treatment initiation to physician-assessed disease progression following subsequent-line treatment or death from any cause, whichever occurs first, derived from patient medical records and recorded in the eCRF at the end of the available follow-up

4. Real-world overall survival (rwOS) measured using the time from zanidatamab treatment initiation to death from any cause, derived from patient medical records and recorded in the eCRF at the end of the available follow-up

5. Real-world duration of response (rwDOR) measured using the time from first documented physician-assessed partial response or complete response to disease progression or death, derived from patient medical records and recorded in the eCRF at the end of the available follow-up

6. Real-world objective response rate (rwORR) measured using the proportion of patients achieving a physician-assessed complete response or partial response to zanidatamab treatment, based on medical record review and recorded in the eCRF at the end of the available follow-up

Key secondary outcome(s)
1. Patient demographics and relevant medical history measured using demographic and clinical characteristics abstracted from medical records, including country of treatment, age at zanidatamab initiation, sex at birth, relevant comorbidities, date of biliary tract cancer diagnosis, HER2 test results, previous surgery, biliary tract cancer subtype, previous lines of treatment, ECOG performance status and participation in other interventional clinical trials at baseline (zanidatamab treatment initiation)

2. Dates of zanidatamab treatment initiation and discontinuation measured using patient medical records and recorded in the eCRF at the end of the available follow-up

3. Zanidatamab dose, dose modifications and treatment interruptions measured using patient medical records and recorded in the eCRF at the end of the available follow-up

Completion date
31/10/2026

Eligibility

Key inclusion criteria
Patients with previously treated unresectable locally advanced or metastatic HER2-positive biliary tract cancer who received at least one dose of zanidatamab through the Early Access Programme (no upper age limit).

In Spain and Italy, living patients must provide informed consent where required by local regulations. In the United Kingdom, eligible patients may be included using the approved opt-out process.

Healthy volunteers allowed
No

Age group
Mixed

Lower age limit
18 Years

Upper age limit
99 Years

Sex
All

Total final enrolment
0

Key exclusion criteria
United Kingdom only: Patients with an NHS National Data Opt-Out and patients who choose to opt out of participation.

Spain and Italy: Patients who do not provide informed consent where consent is required by local regulations.

Date of first enrolment
03/08/2026

Date of final enrolment
30/09/2026

Locations

Countries of recruitment
United Kingdom

England

Italy

Spain

Study participating centre
University College London Hospitals NHS Foundation Trust
250 Euston Road
London
England
NW1 2PG

Study participating centre
Humanitas Cancer Center – IRCCS Humanitas Research Hospital
Via Manzoni 56
Rozzano (Milan)
Italy
20089

Study participating centre
IRCCS Candiolo Cancer Institute
Strada Provinciale 142 Km 3.95
Candiolo (Turin)
Italy
10060

Study participating centre
Fondazione IRCCS Istituto Nazionale dei Tumori di Milano
Via Giacomo Venezian 1
Milan
Italy
20133

Study participating centre
Hospital Universitario Fundación Jiménez Díaz
Avenida de los Reyes Católicos 2
Madrid
Spain
28040

Study participating centre
Vall d'Hebron Institute of Oncology
Calle Natzaret 115-117
Barcelona

Spain
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Study participating centre
Hospital Universitario 12 de Octubre
Avenida de Córdoba s/n
Madrid
Spain
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Sponsor information

Organisation
Jazz Pharmaceuticals Ltd

Funder(s)

Funder type

Funder Name
Jazz Pharmaceuticals

Alternative Name(s)
Jazz Pharmaceuticals plc, Greenwich Biosciences, Jazz Pharmaceuticals, Inc.

Funding Body Type
Government organisation

Funding Body Subtype
For-profit companies (industry)

Location
Ireland

Results and Publications

Individual participant data (IPD) sharing plan

IPD sharing plan summary

Not expected to be made available