

# INOtuzumab Treatment Retrospective Analysis for Navigating tranSITION to CD19 CAR-T (INO-TRANSIT)

**First published:** 24/05/2024

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Study

Planned

## Administrative details

### PURI

<https://redirect.ema.europa.eu/resource/1000000118>

### EU PAS number

EUPAS1000000118

### Study ID

1000000118

### DARWIN EU® study

No

### Study countries

Spain

United Kingdom

United States

### Study description

The study will be an international site-led (approximately 13 sites) retrospective observational chart review, assessing the characteristics and clinical outcomes of R/R ALL patients who received InO as a bridging therapy to CAR-T. Sites will be based in the US, UK, and Spain. This non-interventional study is designated as a Post-Authorization Safety Study (PASS) and is conducted voluntarily by Pfizer.

## Study status

Planned

## Research institution and networks

### Institutions

#### Adelphi Real World

United Kingdom

**First published:** 22/07/2016

Last updated

06/03/2024

Institution

Non-Pharmaceutical company

ENCePP partner

#### Pfizer

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

Last updated

01/02/2024

Institution

## Contact details

### Study institution contact

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Study contact

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### Primary lead investigator

Alexander Russell-Smith

Primary lead investigator

## Study timelines

### Date when funding contract was signed

Planned:

22/08/2023

Actual:

22/08/2023

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### **Study start date**

Planned:

01/07/2024

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### **Date of final study report**

Planned:

01/03/2025

## Sources of funding

- Pharmaceutical company and other private sector

## More details on funding

Pfizer Inc

## Study protocol

[B1931044 Non Interventional Study Protocol v1.0\\_11Apr24\\_Redacted.pdf](#)(16.09 MB)

## Regulatory

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

No

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

Not applicable

## Other study registration identification numbers and links

Pfizer study number B1931044

## Methodological aspects

### Study type

### Study type list

**Study type:**

Non-interventional study

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**Scope of the study:**

Drug utilisation

Effectiveness study (incl. comparative)

Other

**If 'other', further details on the scope of the study**

Describe the demographics and clinical characteristics of R/R ALL patients treated with InO as a bridging therapy for CAR-T

**Data collection methods:**

Secondary data collection

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**Study design:**

The study will be an international site-led (approximately 13 sites) retrospective observational chart review, assessing the characteristics and clinical outcomes of R/R ALL patients who received InO as a bridging therapy to CAR-T. Sites will be based in the US, UK, and Spain.

**Main study objective:**

To describe the demographics and clinical characteristics of R/R ALL patients treated with InO as a bridging therapy for CAR-T.

## Study Design

**Non-interventional study design**

Cohort

## Study drug and medical condition

**Name of medicine**

BESPONSA 1 mg - Powder for concentrate for solution for infusion

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

INOTUZUMAB OZOGAMICIN

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**Anatomical Therapeutic Chemical (ATC) code**

(L01FX06) dinutuximab beta

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## Medical condition to be studied

Acute lymphocytic leukaemia

## Population studied

### Short description of the study population

1. Patient has a clinical diagnosis of R/R (precursor B-cell) ALL
  2. Patient is aged 18 years or older at start of InO therapy
  3. Patient received InO treatment as a bridge to CD19-directed CAR-T cell therapy (is defined as either patient received InO immediately prior to apheresis, and/or patient received InO after apheresis and before CAR-T cell infusion. In either case, InO was used with the aim of preventing uncontrolled disease progression and facilitating successful progression to CAR-T cell therapy. Such patients are eligible to be included even if CAR-T was not ultimately administered from index date (01 June 2017))
  4. Patients with data points available for a minimum of 3 months data from the first dose of InO are eligible for inclusion within the study. Permitted exceptions to the minimum 3-month follow-up requirement include:
    - a. Patients who die within the 3 months
    - b. Patients who proceed to a CD19 CAR-T clinical trial within the 3 months
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### Age groups

Adult and elderly population (>18 years)

Adults (18 to < 65 years)

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Elderly (? 65 years)

Adults (65 to < 75 years)

Adults (75 to < 85 years)

Adults (85 years and over)

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### Estimated number of subjects

66

## Study design details

### Setting

R/R ALL patients aged 18 years and above initiating InO treatment as a bridge to CD19-directed CAR-T from index date 01 June 2017 (first InO approval) will be identified within the sites based in UK, US and Spain, using the inclusion and exclusion criteria. The indexing period should allow for a large proportion of patients treated with InO prior to CAR-T at these sites to be gathered, following US Food and Drug Administration (FDA) and European Medicines Agency (EMA) approval of InO in 2017, and CAR-T approval from 2017. No formal sampling will be conducted in any country. Sites selected for chart review methodology will complete data collection for all eligible patients.

Potential sites participating in the retrospective chart review will be invited to complete a short feasibility questionnaire prior to site selection. The feasibility questionnaire will be used to confirm suitability of sites for inclusion, confirming study critical information such as InO patient caseload and data availability. Additionally, logistical information pertaining to site contracting and IRB processes will also be assessed. A number of sites across the three countries have already been assessed to provisionally confirm feasibility. Following completion of the feasibility phase of the study and the subsequent set-up activities for selected sites, eCRFs will be completed by clinicians and site staff at the sites for patients meeting the inclusion/exclusion criteria.

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## **Outcomes**

### Primary objective

- To describe the demographics and clinical characteristics of R/R ALL patients treated with InO as a bridging therapy to CAR-T.

### Secondary objectives

- To describe relevant treatment effectiveness outcomes for the study population, including response to treatment, time-to-next salvage treatment and survival.
  - To describe InO treatment patterns in the study population, including the use of InO in combination or monotherapy, timing of treatment and dosage information.
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## **Data analysis plan**

The study is not intended to test hypotheses and is primarily descriptive in nature, therefore descriptive analysis will be conducted to meet the majority of the pre-specified objectives. Frequencies and percentages will be reported for categorical variables, including the percentage of missing/unknown data, while counts, number of missing, means, medians, standard deviations (SDs), standard errors (SEs), first and third quartiles, minimum and maximum values will be reported for continuous numeric variables. Where applicable, all estimates will be described with accompanying 95% confidence intervals (CI). Outcomes such as OS will be assessed via time to event analyses, with Kaplan-Meier (KM) curves and 95% CI estimated for KM curves outputted. This will be calculated using variables in the CRF. For example, OS will be calculated from the first dose of InO to point of death. Censoring will be applied if the patient is alive at the point of data capture.

Data analysis will be aligned with data extracted/collected from all data sets. Where specific variables or outcomes cannot be assessed/described subgroup analyses could be conducted for a subset of patients from certain data sources.

All analyses will be conducted using Stata 17 software, version 17 or the latest available version (StataCorp, College Station, Texas).

Detailed methodology for summary and statistical analyses of data collected in this study will be documented in a statistical analysis plan (SAP), which will be dated, filed, and maintained by the sponsor. The SAP may modify the plans outlined in the protocol; any major modifications of primary endpoint definitions or their analyses would be reflected in a protocol amendment.

## **Data management**

### **Data sources**

## Data sources (types)

Other

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### Data sources (types), other

Data will be collected/extracted from sites based in the UK, US and Spain. The study will involve the abstraction of data from patients' medical charts by healthcare professionals involved in the care of the patients into a secure eCRF. Sites will be asked to provide eCRFs for all patients which they have access to who meet the inclusion criteria. Sites will be instructed to refer to the patients' complete medical record whilst completing the eCRF and not to answer any question from memory or that is not listed within the patients' medical records.

## 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