A European observational registry collecting efficacy and safety data in newly diagnosed pediatric Philadelphia-positive (Ph+) Acute Lymphoblastic Leukemia (ALL) patients treated with chemotherapy + imatinib ± hematopoietic stem cell treatment (±HSCT)

First published: 29/05/2014

Last updated: 14/03/2024

Study | Finalised

Administrative details

EU PAS number

EUPAS6665

Study ID

39519

DARWIN EU® study

No

Study countries

France
Germany
Greece
Hungary
Italy
Poland
Portugal
Romania
Russian Federation
Slovakia
Slovenia
Spain
Ukraine

Study description

This study was an observational, multi-center disease registry to collect efficacy and safety data in Ph+ ALL pediatric patients treated with chemotherapy + imatinib, with or without hematopoietic stem cell treatment (±HSCT), primarily in European countries. The study concept was endorsed by the EMA as part of the post-marketing commitment (EMEA/H/C/000406/II/80) and the design was aligned with ensuing discussions, as well as routine medical practice in the treatment of newly diagnosed Ph+ ALL in pediatric patients across Europe. This was an EMA imposed non-interventional post-authorization safety study (NI-PASS), conducted per GVP module VIII. For each patient enrolled in the registry, a minimum of 5 years observational follow-up data was collected or such available data until early discontinuation, as measured from the date of diagnosis.

Study status

Finalised

Research institutions and networks

Institutions

Novartis Pharmaceuticals

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Multiple centres: 33 centres are involved in the study

Contact details

Study institution contact

Disclosure Officer Novartis Clinical

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Study contact

Trialandresults.registries@novartis.com

Primary lead investigator

Disclosure Officer Novartis Clinical

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 20/12/2013

Actual: 20/12/2013

Study start date
Planned: 30/06/2014

Actual: 14/07/2014

Data analysis start date
Actual: 01/09/2022

Date of final study report
Planned: 20/02/2023

Actual: 02/02/2023

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Novartis Pharma AG

Study protocol

STI571I2201-v02--protocol_Redacted.pdf (358.28 KB)

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 1 (imposed as condition of marketing authorisation)

Other study registration identification numbers and links

CSTI571I2201

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Effectiveness study (incl. comparative)

Data collection methods:

Combined primary data collection and secondary use of data

Main study objective:

The primary objective was to evaluate long-term clinical outcome measured by even-free survival (EFS).

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Observational, multi-center registry

Study drug and medical condition

Anatomical Therapeutic Chemical (ATC) code

(L01XE01) imatinib

imatinib

Medical condition to be studied

Philadelphia positive acute lymphocytic leukaemia

Population studied

Short description of the study population

The study population included male and female pediatric patients (aged 1 to <18 years old) diagnosed with Philadelphia chromosome positive (Ph+) acute lymphoblastic leukemia (ALL) identified from hospital discharge files, clinical records and electronic medical records.

Inclusion Criteria:

1. Male or female, pediatric patients aged greater than 1 year (≥365 days) and less than 18 years old (<17 years, 365 days) at diagnosis.
2. Documented, newly diagnosed Philadelphia Chromosome-positive (Ph+) acute lymphoblastic leukemia (ALL).

- Recorded presence of t(9;22)(q34;q11) is required e.g. determined via institutional cytogenetics or FISH and/ or of the presence of BCR-ABL fusion transcript identified by RT-PCR or FISH.

3. Enrolled into this registry within 6 months of diagnosis or enrolled in a clinical trial within
6 months of diagnosis, although no earlier than Jan-2012.
4. Previously treated or currently on treatment with any chemotherapy regimen + imatinib (of an HA-approved formulation or HA-approved Glivec generic) ± HSCT.
5. Written informed consent obtained prior to any information being entered into the registry(parent / legal guardian consent, where applicable).

- Assent from a patient enrolled as a minor by parent / legal guardian consent must be obtained wherever possible. Obvious child dissent must be respected.
- A patient enrolled as a minor by parent / legal guardian consent must be re-consented as an adult upon reaching the legal age of maturity during the course of the registry(legal age of maturity defined by local regulations).
- Patients fulfilling the inclusion criteria, but who have died prior to registry opening and without the opportunity to give consent, may still be eligible for inclusion, subject to local requirements regarding the consent process.

The registry participating physician must assess whether there are any third-

party agreements limiting the clinical trial patient’s data collection as part of the non-interventional study given that no identification of proprietary treatment information will take place.
Exclusion Criteria:
- There are no exclusion criteria for this non-interventional study.
- Patients may voluntarily withdraw from the registry at any time.</18></18>

Age groups

- Adolescents (12 to < 18 years)
- Children (2 to < 12 years)
- Infants and toddlers (28 days – 23 months)

Special population of interest

Other

Special population of interest, other

Patients with Philadelphia chromosome positive (Ph+) acute lymphoblastic leukemia (ALL)

Estimated number of subjects

65

Study design details

Outcomes

The primary outcomes relate to EFS, which is defined as time from diagnosis to relapse at any site, development of a second malignant neoplasm or death., Main secondary outcomes relate to overall survival (OS), defined as time from diagnosis to death from any cause and Safety, defined as adverse events and

serious adverse events in the observation period. Other secondary outcomes relate to hematological remission status, MRD response, duration of complete remission, evaluation of time to transplantation, assessment of growth & development.

Data analysis plan

All data analyses were performed by Novartis personnel and/ or designee. All data
summaries and analyses have descriptive purposes only. Patient efficacy, safety and
tolerability outcomes of patients treated with chemotherapy + imatinib were summarized
overall, by HSCT (yes/ no) and risk group (good risk– induction responder/ poor risk–
induction non-responder).

Documents

Study results

csti571i2201--report-body_Redacted.pdf (1.36 MB)

Data management

ENCePP Seal

The use of the ENCePP Seal has been discontinued since February 2025. The ENCePP Seal fields are retained in the display mode for transparency but are no longer maintained.

Data sources

Data sources (types)

Other

Data sources (types), other

Prospective patient-based data collection, EsPhALL study Database Network

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