

Clinical Trial Summary

A study to provide continued treatment with freeze-dried (lyophilized) pegaspargase (S95014) in pediatric patients with Acute Lymphoblastic Leukemia (ALL).

Full scientific title: A Multicenter, roll-over study to provide continued treatment with lyophilized pegaspargase (S95014) in Pediatric Patients with Acute Lymphoblastic Leukemia (ALL).

We thank all the participants and their families who took part in the study. Clinical study participants are very important for making progress in science, for the benefit of patients.

This document is a summary of the study. It is written for a general audience.

Researchers need many studies to decide which medicines work the best and are the safest for patients. For medical science to progress, many studies involving patients are running all around the world. This summary only shows the results from this one study. Other studies, evaluating the same drug, may find different results. You should not change your current treatment based on the results of this single study. If you have any questions about this study, please talk to your doctor.

Therapeutic area:
Oncology

Disease:
Acute
lymphoblastic
leukemia

Study phase:
Phase 2

Final version
28/09/2023

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A study to provide continued treatment with freeze-dried (lyophilized) pegaspargase (S95014) in Pediatric Patients with Acute Lymphoblastic Leukemia (ALL).

1 Why was this study done?

This study was done to provide continued treatment with pegaspargase to participants with Acute Lymphoblastic Leukemia (ALL) who completed a previous study called [CL2-95014-002](#).

Leukemia is a cancer of the blood. When these cancers occur, abnormal white blood cells increase rapidly and uncontrollably. ALL is the most commonly diagnosed cancer in children. This kind of leukemia is defined by the type of the abnormal white blood cells that are involved.

The study drug is an “asparaginase” and is called pegaspargase. Asparaginases breakdown a substance in the blood called asparagine (one of the building blocks for proteins). The cancer cells in ALL need asparagine to survive. Pegaspargase has been modified to reduce the risk of allergy and to improve the ability to stay in the body. This is called freeze-dried pegaspargase.

The main aim was to provide participants of the study [CL2-95014-002](#) continued treatment with freeze-dried pegaspargase as this treatment was not yet available in Russia.

2 When and where did this study take place?

When did the study take place?

- This study started in May 2021.
- It ended in January 2023.

Where did the study take place?

The study took place in Russia.

3 Who participated in the study?

Which participants were included in the study?

To take part, participants had to:

- Complete a previous called study [CL2-95014-002](#).
- Benefit from treatment with pegaspargase in the study [CL2-95014-002](#).

How many participants took part in the study?

A total of 74 children and adolescents took part in the study: 36 girls and 38 boys.

How old were the participants?

The average age of the participants was 6 years. The youngest participant was 2 years old and the oldest was 18 years old.

4 Which treatments did the participants receive?

Each participant received pegaspargase through a needle or catheter into a vein (infusion). The infusion lasted about 1 hour. Pegaspargase was given every 2 weeks or 4 weeks for a total of 9 doses during the treatment period.

The dose of pegaspargase was determined according to the body surface area (BSA) and based on study doctor’s decision. The BSA is calculated using the participant’s height and weight. The doses were 1000, 2000 or 2500 units per square meter of BSA.

In addition, all participants received other treatments routinely used to treat this cancer.

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5 How was the study carried out?

Patients with ALL are treated over several time-periods. The aim of first period was to compare the activity of the 2 formulations of pegaspargase (one formulation was freeze-dried and the other was a liquid formulation). It is described in the Lay summary of a previous study [CL2-95014-002](#).

The present study focused on the second period of treatment, which lasted around 7 months.

All participants received the freeze-dried formulation of pegaspargase.

The participants visited the doctors regularly. During the visits, the doctors collected information about the participants' health.

6 What were the side effects?

Side effects are unwanted medical events that the doctors think may be caused by the treatments in the study.

In this summary, we describe unwanted medical events thought to be caused by pegaspargase. The results may be presented differently in other documents related to the study.







The table below shows the number of participants who had side effects.

	Pegaspargase (out of 74 participants)
Participants who had side effect(s)	74 (100%)
Participants who had serious* side effect(s)	16 (22%)
Participants who stopped the treatment because of side effect(s)	18 (24%)

*See definition of serious side effects below

What were the types of side effects?

The table below shows the most common side effects reported in the study (reported by at least 20% of participants).

	Pegaspargase (out of 74 participants)
Decrease of a protein called Antithrombin III (needed to prevent blood clotting)	53  (72%)
Decrease of a protein called fibrinogen (needed for blood clotting)	48  (65%)
Low level of neutrophils, a type of white blood cells	37  (50%)
Allergic reaction	23  (31%)
Decrease in the number of red blood cells	20  (27%)
Low level of white blood cells	17  (23%)

 = participants

What were the serious side effects?







A side effect is considered serious when:

- the participant needs to be hospitalised,
- it causes lasting damage or death,
- the participant's life is in danger or,
- it is medically important in the doctor's opinion.

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The table below shows the serious side effects thought to be caused by pegaspargase in the study.

	Pegaspargase (out of 74 participants)
Allergic reaction	11  (15%)
Sudden, severe allergic reaction	1  (1%)
Drug allergic reaction	1  (1%)
Liver injury	1  (1%)
Low level of neutrophils, a type of white blood cells	1  (1%)
Type of sudden inflammation of the pancreas	1  (1%)

No participant died during the study.

7 What were the study results?

The study was completed as planned.

The participants were able to continue their treatment with pegaspargase (some participants could not receive all the doses due to allergic reactions). The study provided additional safety information on the use of pegaspargase (see section 6).

8 How has this study helped research?

The study helped participants with ALL in getting continued treatment with pegaspargase. This study also helped researchers find out more on the safety of pegaspargase in children and adolescents.

This summary shows only the main results from this one study. Other studies, evaluating the same drug, may find different results.

9 Are there plans for further studies?

No other studies with pegaspargase are planned since this treatment has already been approved in several countries.

10 Further information

What are the identification numbers of the study?

- Protocol code: CL2-95014-003
- EudraCT number: 2020-004895-17
- US NCT number: 04956666

Who did the study?

The company that organised and funded the research, called the “sponsor”, is the Institut de Recherches Internationales Servier based in Suresnes, France.

How can you contact the sponsor?

Contact us on the Servier website
<https://servier.com/en/>.

Where can you learn more about this study?

You can find more information about this study on these websites:

- <https://clinicaltrials.servier.com/find-clinical-trials>
- www.clinicaltrialsregister.eu/ctr-search
- <http://www.clinicaltrials.gov/>

In this document we translated medical terms into lay terms. You can find the corresponding medical terms in the Servier glossary at <https://clinicaltrials.servier.com/glossary/>

You can find general information about clinical trials on <https://clinicaltrials.servier.com/>