Lay summary

# Why was this study done?

People with blood cancers often develop low blood cell counts either as a consequence of the disease or its treatment (chemotherapy or a stem cell transplant). Platelet transfusions are commonly given to raise these low platelet counts and reduce the risk of bleeding (prophylaxis) or to stop bleeding (therapy).

However, recent studies have indicated that many patients continue to experience bleeding despite the use of platelet transfusions.

## What is tranexamic acid?

Tranexamic acid is an antifibrinolytic drug which reduces the breakdown of clots formed in response to bleeding. Antifibrinolytics have been used to decrease blood loss and the use of red cell transfusions in planned and emergency surgery.

## What was the purpose of this study?

The purpose of this study was to compare tranexamic acid plus best supportive care to best supportive care alone to see if tranexamic acid reduced the risk of significant bleeding or death and the need for platelet transfusions in participants with blood cancers.

## What were the main questions asked in this study?

* Does tranexamic acid reduce the risk of significant bleeding or death?
* Does tranexamic acid reduce the number of platelet transfusions or need for any platelet transfusions?
* Does tranexamic acid reduce the number of red cell transfusions or need for any red cell transfusions?
* Does tranexamic acid improve quality of life?
* Does tranexamic acid increase the risk of developing a blood clot?
* Does tranexamic acid increase the risk of a serious adverse event?

# What happened during the study?

## How was the study done?

There were 1736 patients who were screened by a doctor or a nurse for the study. Of these, 636 patients agreed to take part in the study after being told about what the study involved (consented to take part in the study). 616 participants were randomly assigned (by chance) to one of the following treatment groups:

* Tranexamic acid as a tablet or injection
* Matching placebo as a tablet or injection

310 participants were assigned to tranexamic acid and 306 participants were assigned to the matching placebo.

During the trial, participants, their physicians and the researchers did not know whether the participants were taking tranexamic acid or a placebo.

During the trial, when the participants were on the hospital ward, one of the research team helping with this study came and reviewed the participant each morning to ask questions about signs and symptoms of bleeding. If participants went home they were given a simple diary card to fill in with details of any signs or symptoms of bleeding.

Information about bleeding was collected for 30 days after the start of the study treatment.

## Where did this study take place?

This study took place at 27 specialist haematology centres in Australia and the UK, 11 sites in Australia and 16 sites in the UK.

## When did this study take place?

It began in June 2015 and ended in June 2022.

## Who took part?

The study included adults with blood cancers who were having chemotherapy or a stem cell transplant and were expected to have a very low platelet count for at least 5 days.

380 men and 236 women participated.

Participants had a variety of different blood cancers. A total of 264 had acute myeloid leukaemia, 132 had non-Hodgkin lymphoma, 71 had myeloma, and 149 had other blood cancers.

Out of the 616, 585 participants received at least one dose of the trial treatment. The trial treatment was continued until one of the following occurred:

* The participant’s platelet count had recovered so that they were no longer at high risk of bleeding or needing a platelet transfusion: 322 participants (55%)
* The participant no longer wanted to take the trial treatment: 127 participants (22%)
* It had been 30 days since the trial treatment had started: 62 participants (11%)
* The participant’s doctor no longer wanted the participant to take the trial treatment: 49 participants (8%)
* Other reasons: 25 participants (4%)

## How long did the study last?

Each participant remained in the study for up to 6 months. The whole study lasted for 7 years.

What were the results of the study?

In adults with blood cancers who are having chemotherapy or a stem cell transplant, and whose platelet count was very low:

* Tranexamic acid did not decrease the risk of clinically significant bleeding or dying
	+ In this trial, for those participants who did not receive tranexamic acid the risk of significant bleeding or dying was 34%. Of these nearly all had a bleeding episode (33%) rather than dying (1%).
	+ Giving tranexamic acid would mean 2% fewer participants would have bled or died. This estimate was uncertain and there could be between 9% fewer participants to 5% more participants who bled or died.
* Tranexamic acid did not decrease the number of participants who needed a platelet transfusion. On average, all participants received 2 platelet transfusions during the first 30 days of the study.
* Fewer participants needed a red cell transfusion if they received tranexamic acid. However, if they needed a red cell transfusion, tranexamic acid did not decrease the number of red blood cell transfusions participants received over the 30 days.
* Tranexamic acid did not improve quality of life.
* Tranexamic acid did not increase the risk of a blood clot.
* Tranexamic acid did not increase the risk of a serious adverse event.