

Research Summary

Title: Studies of: 1. normal and abnormal blood cells, 2. genetic disorders of blood cells and coagulation factors, and 3. transforming growth factor- β 1 in health and disease

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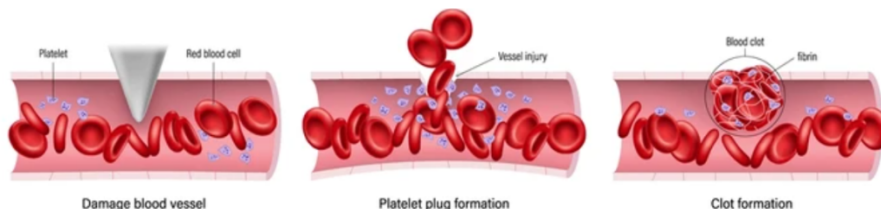
Thank you for your participation in this study.

As a participant in a clinical research study, you belong to a large community of participants around the world who help researchers answer important health questions and discover new medical treatments. Without your research partnership, clinical research would not be possible.

Here, we share a summary of the results of this study. We hope it helps you understand and feel proud of your key role in medical research and discovery.

What was this study about?

We study how blood platelets (a type of cell in blood) do their work, which primarily includes stopping bleeding from wounds. In some situations, however, platelets can contribute to producing blood clots in the wrong places, and this can lead to heart attacks, strokes, and blood clots in the veins in the legs.



In this study, we ask research volunteers to donate a few tablespoons of blood. We study their blood in the laboratory and use the information we learn about how platelets work to design drugs to treat and prevent these conditions. Sometimes the blood of patients with unusual platelets gives us important information about how platelets work, and ideas for making new drugs.

We previously invented the drug abciximab (ReoPro), which works on platelets to prevent clots that cause heart attacks. Studies of patients with a rare platelet condition was very important in the development of the abciximab. More than 5 million people have been treated with abciximab.

We have also developed methods to test whether patients are getting the best response to drugs they are taking to prevent heart attacks and strokes, including aspirin and Plavix (clopidogrel). These tests (VerifyNow) are being used in the U.S., Europe, and Asia.

Who was included in this study?

We mostly study blood from healthy volunteers, but sometimes we study blood from patients who have unusual platelets or blood clotting proteins. To date, we have studied 15 men and 11 women, ages 31 to 79, including 25 healthy volunteers and 1 patient with a platelet disorder.

What were the results of the study?

We learned more about how platelets work and used that information to identify new drugs that may help to prevent heart attacks, strokes and other abnormal clotting diseases. We tested the effect of different compounds on how platelets function and identified compounds that alter platelet function in ways that may help prevent or treat heart attacks. We then modified the compounds to make them have better properties as drugs, for example, the ability to be absorbed rapidly so that they can work fast.

Specifically, the blood samples we tested helped us develop a new drug (zalunfiban) that prevents platelets from producing clots. Zalunfiban showed benefit for treating patients who are having a heart attack who receive the drug in the ambulance or in the emergency room. The company developing zalunfiban, CeleCor Therapeutics, has submitted a request to the Food and Drug Administration to approve use of zalunfiban for patients having a heart attack.

We have also used the results of this study to identify additional drugs that may be good for preventing blood clots in the legs. Blood clots are a common problem in patients with cancer. We also identified other drugs that may be good for preventing blood clots in patients with infectious diseases, like COVID-19, and disorders of their immune systems, such as Lupus.

We have published a number of scientific papers about our research, and a few of them are listed at the end of this report.

How has this study helped patients and researchers?

This study has helped patients by finding new drugs that may improve the treatment of heart attack and stroke.

In addition, because we publish all the new knowledge we obtain, other researchers can use that information for their own studies to identify additional new therapies that may be more effective or safer.

Are there plans for further studies? Or what happens next?

We continue to perform additional experiments with the blood we obtain in these studies. If

we find new therapies that may be better than existing therapies, we will develop new studies to test them at that time.

Who paid for this study?

The research was paid for by grants from the National Institutes of Health, which is supported by American taxpayers and by Rockefeller University funds from generous donors.

Where can I find more information about this study?

This study is registered on the federal website [ClinicalTrials.gov](https://clinicaltrials.gov), and you can search that website using the registration numbers for these studies ([NCT00230165](https://clinicaltrials.gov/ct2/show/study/NCT00230165)).

For more information on zalunfiban, you can go to the CeleCor Therapeutics website (<https://www.celecor.com/>) since CeleCor is the company developing zalunfiban.

Some papers published from this study can be found [here](#).

For information about research studies at Rockefeller, go to <https://www.rucare.org>

This research was important. Thank you for helping us understand more about platelets, heart attacks, strokes, and clots in the legs. Thank you for being part of this study. Without research participants, there would be no clinical research and many fewer advances in medical therapy!

Thank you to all who participated!

If you have questions, please contact Dr. Barry Collier at collerb@rockefeller.edu.
