BMT CTN
Blood & Marrow Transplant Clinical Trials Network

PROTOCOL SUMMARY

Frequently asked questions about BMT CTN 2203, a study adding ruxolitinib to medications commonly used to prevent GVHD

Brought to you by the BMT CTN Patient and Caregiver Advocacy Committee

What is being studied?

Doctors want to find the best combination of medications for transplant patients to prevent graft-versus-host disease (GVHD), a serious complication after blood or marrow transplant. The combination they are studying is tacrolimus and methotrexate, **plus ruxolitinib (Rux**, also called Jakafi).

This is a two-step study. The first step is to find the best dose of **Rux**—either 5mg or 10mg twice daily—to be used with tacrolimus and methotrexate. This is called the "run-in" phase of this study. **You are invited to participate in this first step of the study. 50 patients will participate in this step.**

The second step is to compare 2 ways to prevent GVHD:

- A standard combination of medications (tacrolimus and methotrexate) **plus ruxolitinib (Rux)** using the best dose found in the first step
- A standard combination of medications (tacrolimus, mycophenolate mofetil and cyclophosphamide)

Why Rux?

Smaller studies have shown that **Rux** may help prevent GVHD when given with tacrolimus and methotrexate. The Food and Drug Administration (FDA) approved **Rux** to **treat** GVHD. It's **not** approved to **prevent** GVHD.

Why 5mg or 10mg doses?

In other **Rux** studies for GVHD treatment, patients got 5mg or 10mg twice daily. These doses were found to be safe and effective and led to the FDA approval for GVHD treatment. Both doses are included in this study to find the best dose for **preventing** GVHD.

What is the goal of the run-in part of the study?

To see which dose of **Rux** is best to use to prevent GVHD.

What if I don't want to be in the study?

Let your doctor know that you do not want to participate. You and your doctor can then discuss options available to you to prevent GVHD.

What is different if I join the run-in part of the study?

You will receive a standard combination of medications (tacrolimus and methotrexate) **plus Rux** for GVHD prevention. For the **Rux**, you'll take pills 2 times a day for 1 year. Then you'll take a lower dose of the pills for about 3 months.

You will be randomly assigned by a computer to get either:

- 5mg of **Rux** twice daily
- 10mg of **Rux** twice daily

Are there extra tests and doctor visits?

There are not any extra planned doctor visits. There are extra tests done before and after transplant. These include pregnancy testing (if needed), blood and urine samples, 2 EKGs, and surveys.

Are there extra costs for participating?

You or health insurance company will **not** be charged for **extra** tests or research costs for this study. Most of the doctor visits for this study are standard of care and will be billed to your health insurance company.

What are the risks?

You may have side effects during the study. They can range from mild to severe. Read more about the risks in the consent form. Then, tell your doctor about your questions or concerns.

Are there potential benefits?

Joining this study may or may not make your health better. If it works for you, you may have less severe complications after transplant.

How long would I be in the study?

For about 2 years after your transplant.

I think I want to join. What's next?

1. First, you will talk to your doctor about the study and review the consent form.
2. If you agree to join, you will sign the consent form.
3. You will have screening tests done.
4. A computer will randomly assign you to **Rux** 5mg or 10mg twice daily.
5. You will get transplant and tacrolimus and methotrexate **plus Rux** to prevent GVHD.

What if I want to leave the study after I join?

Being in this study is your choice. If at any time you are considering leaving the study, talk to your study doctor about your health and safety.

What if I have questions? Where can I go for more information?

Your transplant doctor and study team will be able to answer your questions. You can also access these resources:

Consent form

Study Information

ClinicalTrials.gov

Clinical Trial IDs: BMT CTN 2203; ClinicalTrials.gov NCT06615050

Sponsor: Blood and Marrow Transplant Clinical Trials Network (BMT CTN)

BMT CTN is a collaboration of the Center for International Blood and Marrow Transplant Research; The Medical College of Wisconsin; NMDP; and The Emmes Company.