What this study is about

This study (CALGB 100104) compared a new drug treatment to no additional treatment for people with multiple myeloma (a blood cancer) after they had a stem cell transplant.

The official title of this study is:
CALGB 100104: A phase III randomized double-blind study of maintenance therapy with lenalidomide or placebo following autologous stem-cell transplant for multiple myeloma

Why the study was done

Multiple myeloma is a cancer of blood cells called "plasma" cells that fight infection as part of the immune system. Plasma cells are in a person's bone marrow. When they turn cancerous, they crowd out normal bone marrow, causing low blood counts and raising the risk of bleeding and infection. Plasma cancer cells can also cause holes in the bones, called lytic lesions, which can lead to serious problems that lower a person's quality of life. This disease was hard to treat until new drugs were found that work better and help people live longer.

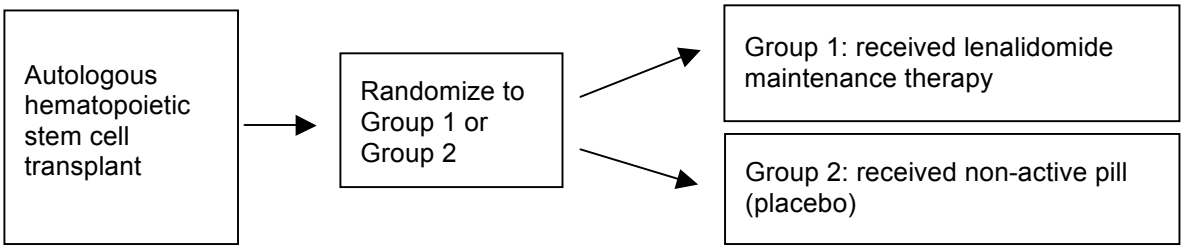
This study was done to find out if lenalidomide (Revlimid®), a drug used to treat multiple myeloma, can help maintain a person's response to treatment (called "maintenance therapy"), prevent cancer from coming back (recurrence) and help people with multiple myeloma live longer.

All patients got an autologous hematopoietic stem cell transplant (called "transplant"), which is a treatment that collects the patient's own blood stem cells and gives them back to replace stem cells that are ruined during high dose chemotherapy (other drug treatment). Blood stem cells create all blood cell types.

About 100 days after the transplant, patients were put into two groups by chance (randomized) to reduce differences between the groups. This was done because no one knew if one group was better than another.

Half the patients received lenalidomide. The other half of patients received a placebo (a non-active pill). All patients remained on the study unless the multiple myeloma got worse. Their doctor then treated patients who had a recurrence of multiple myeloma.

Here is a picture that explains how patients were placed into one of two groups.



When did the study start and end? The study started in April 2005. All patients were enrolled by July 2009.

How many patients joined? 568 patients agreed to be in this study, and 460 were put into Group 1 or Group 2.

Study results

Studies like this one are closely watched by a Data and Safety Monitoring Board (DSMB), which is made up of people who are not directly involved in the study. The DSMB makes sure the study is managed well, and that benefits and risks are acceptable for patients who joined the study. The DSMB looked at study results in December 2009, and agreed the results were positive enough to state which patients were in Group 1 and which were in Group 2 (this is called "unblinding the study"). 86 patients then decided to move or "cross over" from Group 2 to Group 1 after the study was unblinded. The DSMB reviewed the study again in October 2011, and the results remained positive. It is not likely that the results could have happened by chance.

Important findings:

- 63-80 out of 100 people had better responses from Group 1 (lenalidomide therapy) compared to 42-56 out of 100 in Group 2 (placebo).
- 85 out of 100 people survived in Group 1 compared to 77 out of 100 in Group 2.
- There were more side effects in Group 1, including lower blood counts.
- More new types of cancers, in addition to multiple myeloma, were found in Group 1. These are called "new primary" cancers.

We are working to better understand why these new cancers happened.

What the results mean

This means:

- Lenalidomide maintenance therapy lengthens the time before multiple myeloma returns.
- Lenalidomide maintenance therapy also helps people live longer (improves overall survival).
- Lenalidomide maintenance therapy is linked to more new primary cancers and worse side effects.

These results are for multiple myeloma patients who have had a stem cell transplant. It offers them the chance to lengthen their response to therapy.

You can talk with your doctor for more information.

Scientific publications about this study

Details about the study can be found in these articles:

- Lenalidomide after stem-sell transplantation for multiple myeloma (CALGB 100104)
  McCarthy PL, Owzar K, Anderson KC, Hofmeister CC, Hurd DD, Hassoun H, Richardson P, Giralt S, Stadtmauer E, Weisdorf DJ, Vij R, et al.
  New England Journal of Medicine 366 (19):1770-81, 2012

- Phase III intergroup study of lenalidomide versus placebo maintenance therapy following single autologous hematopoietic stem cell transplant (AHSCT) for multiple myeloma (MM): CALGB 100104
  McCarthy PL, Owzar K, Anderson KC, Hofmeister CC, Hurd DD, Hassoun H, Giralt S, Stadtmauer E, Richardson P, Weisdorf DJ, et al.
  Blood. 116 (21):37, 2010

- Phase III intergroup study of lenalidomide (CC-5013) versus placebo maintenance therapy following single autologous stem cell transplant for multiple myeloma (CALGB 100104): Initial report of patient accrual and adverse events
  McCarthy PL, Linker C, Hofmeister CC, Owzar K, Stadtmauer E, Anderson KC, Richardson P, Hurd DD, Weisdorf DJ
  Journal of Clinical Oncology, ASCO Meeting Proceedings. 28 (15s):8017, 2010

This sheet reviews what is known about this research study as of June 2012. New Information may be available.

This study was sponsored by Cancer and Leukemia Group B, one of the groups that merged to form the Alliance for Clinical Trials in Oncology (Alliance) – a national cooperative group that runs large-scale cancer clinical trials. The groups are supported by the National Cancer Institute (NCI) and bring together scientists to develop better treatments for cancer. More information is at http://www.allianceforclinicaltrialsinoncology.org.

Research studies (or clinical trials) are done to learn what works better for people in order to find, treat, or prevent cancers. Thank you for your interest in learning more about cancer research advances.