

Questions & Answers

What are the treatment options for CML?

Different types of CML treatments are available. Some are standard (*fully approved and available*) and some are newer ones being tested in clinical trials. A treatment clinical trial is a research study meant to help improve current treatments or obtain information on new treatments. If you are enrolled in one, you could be put on a new treatment.

My physician prescribed a molecular-targeted therapy. What is that?

Molecular-targeted therapies are a recent advance in the treatment of CML and act directly on the abnormal cells to block their ability to reproduce. There are three such drugs - Gleevec (*Imatinib*), Sprycel (*Dasatinib*) and Tasigna (*Nilotinib*). These drugs, taken by mouth, are not the same as chemotherapy. They have little to no effect on healthy tissues, so they have less severe side effects than chemotherapy. Still, they are not entirely without side effects.

How can I know if a treatment is working?

During treatment, doctors rely on various tests to help them monitor how well you are responding. These include tests that are used to initially diagnose the disease.

What is PCR testing?

PCR stands for polymerase chain reaction and testing for it is done at the molecular level to determine very specifically how well treatment has suppressed your CML.

What happens if my treatment doesn't work?

Based on tests results, your doctor will decide whether to continue, change or stop treatment.

WHAT does that mean?

Some medical terms explained

Blast cell: An immature white blood cell that does not function properly.

Blood count: The number of red and white blood cells and platelets in a blood sample.

Bone marrow: The soft tissue inside bones that produces blood cells.

Bone marrow test: A simple procedure involving collecting a small sample of cells or tissue from the bone marrow.

Chromosome: An organized structure of DNA and protein that is found in cells.

FISH Assay(*fluorescence in situ hybridization*): is a cytogenetic technique that allows scientist to localize the presence or absence of specific DNA sequences on chromosomes. In our case it finds the Philadelphia Chromosome.

International Scale: A standardization process to ensure that all laboratories performing PCR testing on CML patients cell samples adhere to the same standardization process.

Leukemia: A broad term generally used to refer to a variety of cancers of the blood and bone marrow.

Log Reduction: It is a mathematical term used to express the reduction of CML cells in your body and how well you may be responding to treatment.

PCR(*Polymerase chain reaction*): A molecular duplicating process that creates millions of copies of a desired portion of DNA through repeated cycling of a reaction using heating/cooling. This process enables scientists to obtain DNA information from small specimens. Watch the CML Society of Canada educational video "What is PCR" on Youtube: <http://www.youtube.com/watch?v=ZwgynKh6J5U>

Philadelphia chromosome: The chromosome abnormality that causes CML caused by pieces from two different chromosomes breaking off and joining together to create this new, abnormal chromosome.

Platelets: The blood cells that promote clotting to help stop bleeding.

Prognosis: A medical term referring to the chance of a patient's recovery and the doctor's prediction of how that patient will progress.

Remission: The disappearance of the signs and symptoms of disease.

White blood cells: Blood cells that help fight infections

FIGHTING BACK



Treatments for Chronic Myelogenous Leukemia

CML is serious, but the good news is that doctors have learned a lot about the disease in recent years and new treatments have made it much more manageable.

The aim of CML treatment is to reduce the growth of abnormal white cells (*leukemia cells*) in the bone marrow in order to bring about a remission and control the symptoms. Treatment plans vary depending on how far the cancer has progressed and your specific characteristics, such as your age and your general health condition. Your doctor will discuss the possible treatment options with you and the benefits and disadvantages of each.

Today, the majority of people with CML who are careful about following treatment can expect to be able to manage their disease for many years, and still enjoy a full and satisfying life.

WHAT CAN TREATMENT DO?

People may experience varying responses to treatment, including:

Complete Hematologic Response (CHR):

All blood counts return to the normal value ranges (*this may happen within the first three months of treatment*).

Partial Cytogenetic Response (Pycr):

1% to 34% of the bone marrow cells are positive for the Philadelphia Chromosome by FISH assay (*see definition in this brochure*) or approximately a 1 log reduction, or 10 on the International Scale.

Complete Cytogenetic Response (CCR):

There are no abnormal Philadelphia Chromosomes detectable by FISH Assay (*see definition in this brochure*). You will also hear this as being referred to as having achieved a two and a half log reduction or 0.1 to 1 on the International Scale.

Major Molecular Response (MMR):

This response is detected through a more sensitive test called Polymerase Chain Reaction or PCR (*see definition in this brochure*). It is equivalent to a 4 or 5 log reduction, or 0.01 to 0.001 on the International Scale.

Complete Molecular Response (CMR):

No BCR ABL transcripts are detectable PCR (*Polymerase Chain Reaction*) testing. It signifies a response of better than a 5 log reduction or 0.0001 on the International Scale.

Routine testing of your blood and bone marrow is important to see how your current therapy is working against leukemia cells in your body over time to control your CML. Regular testing is necessary even if you are achieving good results because CML is a chronic disease that does not go away.

TREATMENT OPTIONS

Biological therapy

Biological therapy works to strengthen your immune system to help fight cancer or control side effects from other treatments like chemotherapy.

Chemotherapy

Chemicals that have a specific toxic effect on the cancer tissue are administered, either orally or by injection into a vein or a muscle. It is called a systemic treatment because the drugs are in the bloodstream so travel through the body, and can destroy cancer cells outside the target area.

Bone marrow/stem cell transplant

A bone marrow transplant is a procedure to replace the leukemia cells in a patient's bone marrow with healthy cells from a donor or from you. When used in combination with other treatments, your chances of remission are maximized. With a stem cell transplant, some patients can be cured.

Molecular-targeted therapies

These are new drugs that block the growth and spread of cancer by interfering with the specific molecules that cause normal cells to become cancer cells.

ANOTHER GOOD THERAPY...

The understanding of friends and family can be a great help, so reach out and discuss your feelings with someone you trust.

RESISTANCE

When the disease fights back

Many people with CML have achieved and maintained their treatment goals by taking the same medication. However, sometimes the disease cells may not respond to the effects of a drug so their growth is not stopped or even slowed down.

This is called resistance. It can happen soon after you start taking the drug, after you have been treated with the same medication for a while, or never – it's not your fault and can't be predicted or prevented. The causes are complicated and are not fully understood.

Resistance can be detected through tests, which is why it is so important to make sure you have the right lab tests done at the right time, as recommended by your doctor.

When resistance happens, you won't be able to meet your treatment milestones with your current treatment, but there are options:

- Taking a different medication
- Bone marrow or stem cell transplantation
- Interferon treatment to enhance your immune system
- New treatments through a clinical trial

Discuss with your doctor all your questions about your treatment, what regular tests you need and when, and the options available to you.

Established in 2006, the CML

(Chronic Myelogenous Leukemia) Society provides support, education and information on CML, current and emerging treatments and research initiatives for people living with CML and their families.

Through these efforts and ongoing advocacy, the mission of the CML Society is to help reduce suffering and improve care and the quality of life of CML patients.

Tel: 1-866-931-5165

www.cmlsociety.org

