

Welcome to Gaucher Connection eNews

Hello

Thank you for signing up for Gaucher Connection eNews, the monthly newsletter designed to help keep you up-to-date and on track with Gaucher disease treatment and management. In this issue we look at setting treatment goals and review the seven tests that can help you reach them.

Setting Treatment Goals

To get the most from your treatment, it is important to stick with your schedule for receiving infusions. You should also talk to your doctor about setting treatment goals and monitoring your progress. Comprehensive monitoring tests, done at least once a year, provide a way to check the status of your disease and see if you are on track to meet your goals. Even if you are not on treatment and are feeling well, a yearly assessment is a good way to monitor your health.

While you and your doctor will set individual treatment goals based on your situation, treatment guidelines and your doctor's experience with many patients, the table below outlines some general treatment goals and the monitoring tests that can help you and your healthcare team determine the effectiveness of your plan.

General Treatment Goals & Monitoring Tests

1. General Treatment Goal: Bone

- Lessen or eliminate bone pain
- Prevent bone crises (severe pain, usually with a fever, caused by sudden lack of normal blood flow and oxygen to affected bone)

Monitoring Tests:

- **X-ray**
An x-ray detects fractures and late bone problems. However, it is not the best way to assess changes in the bone marrow, the strength of bones, or early signs of bone disease.
- **Magnetic resonance imaging (MRI) of the bones**
MRI uses magnets and radio waves to make pictures of parts of your body. An MRI is the most helpful test to assess to what extent Gaucher cells have built up in the marrow and caused changes in the bone. This makes MRI a powerful and sensitive tool for ongoing monitoring of bone. MRI does not involve use of x-rays.
- **Dual-energy x-ray absorptiometry (DEXA)**
A build-up of Gaucher cells in bones can make the bones thinner, weaker than normal, and prone to fracture. A DEXA scan measures bone mineral density (BMD), which tells your bone strength. It is the "gold standard" for measuring BMD.

2. General Treatment Goal: Blood

- Increase hemoglobin levels to:
 - 11 grams per deciliter or higher for women and children
 - 12 grams per deciliter or higher for men
- Eliminate the need for blood transfusion
- Reduce fatigue related to anemia
- Maintain platelet counts to reduce or prevent bleeding problems

Monitoring Tests:

- **Hemoglobin test**
This blood test measures the total amount of hemoglobin in your blood. Hemoglobin, a part of red blood cells, carries oxygen. A low hemoglobin level is a sign of anemia.
- **Platelet count**
This blood test measures the number of platelets in the blood. Platelets are needed for clotting blood. A low platelet count may cause bruising and bleeding.
- **Biochemical evaluations**
These are special blood tests that can pick up subtle changes in the disease and are useful for some patients. They can help check your progress toward achieving the goals of your treatment plan. Among the markers your healthcare providers may check are:
 - Chitotriosidase
 - Angiotensin-converting enzyme (ACE)
 - Tartrate-resistant acid phosphatase (TRAP)

3. General Treatment Goal: Liver and Spleen

- Reduce and maintain liver volume to 1 to 1½ times its normal size
- Reduce and maintain spleen volume to 2 to 8 times its normal size
- Lessen symptoms, such as abdominal pain and feeling full and bloated

Monitoring Test:

- **MRI or computed tomography (CT) of liver and spleen**
These painless tests produce pictures of the inside of the abdomen. MRI can show the size and structure of the spleen and liver, while CT is a type of x-ray that shows a cross-sectional picture of your liver and spleen. The 3-D picture produced by a CT scan allows measurement of these organs.

Take Action

If you have further questions about how these tests work or what they do, be sure to ask your health care providers.

Learn more about the seven tests essential for monitoring Gaucher at the Gaucher Connection website. [Go >>](#)

Did You Know?

It's important to have comprehensive monitoring tests done at least once a year.

Patient spotlight: Cody

- 9-year-old boy
- Benefits from earlier generations
- Part of a supportive community



Read Cody's story about [how he's grown up in the Gaucher Community](#). [Go >>](#)

Patient Stories are examples based on aggregate medical data. The characters represented are not actual Gaucher patients. Their stories are inspired by those of real Gaucher patients.

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