



## Welcome to Gaucher Connection eNews

### Hello!

You already know treatment can help to manage your Gaucher disease. But what does that really mean, and how do you measure how much it can help? To break it down, your doctor will set therapeutic goals for your treatment. Setting specific goals for <sup>Pr</sup>Cerezyme® therapy creates a structure for measuring how well your treatment is working. In this issue we provide an overview of how treatment goals are used.

### Standard Treatment Goals

Gaucher disease experts have developed standardized goals for treatment based on studying many patients. These include:

- Specific numerical targets for things like blood cell counts and liver size
- Less measurable factors like reducing pain and fatigue
- Timeframes for how long results may take, from a few months to a few years

### Individualized Goals

However, every person with Gaucher responds differently to treatment. Your doctor will use the standard goals as a starting point, and then set individual goals specifically for you based on:

- If you're just starting (or re-starting) treatment and have existing symptoms that need to be addressed
- If you're already on treatment and just need to maintain the good health you've achieved
- The nature and severity of your symptoms
- Any other health issues you may have

Your doctor will use a variety of tests to determine whether your goals are being met. If not, they may decide to adjust the dosage or timing of your infusions, or test more often.

### What to Aim For

Knowing what to expect from treatment, such as what improvements and numbers to watch for, can help you better manage your health. Here's a high-level summary of therapeutic goals for Gaucher<sup>1</sup>, but be sure to talk to your health care team about exactly what targets, numbers, and timeframes you're aiming for.

<b>Bones</b>	<ul style="list-style-type: none"> <li>• Reduce or eliminate bone pain</li> <li>• Prevent bone crisis</li> </ul>
<b>Blood</b>	<ul style="list-style-type: none"> <li>• Increase hemoglobin (in red blood cells) to reduce anemia-related fatigue</li> <li>• Increase blood platelets to minimize bleeding and bruising problems</li> </ul>
<b>Organs</b>	Reduce and maintain: <ul style="list-style-type: none"> <li>• Liver to 1-1.5 times normal size</li> <li>• Spleen to 2-8 times normal size</li> </ul>

<sup>1</sup>Anderson, HC et al. (2005). Individualization of long-term enzyme replacement therapy for Gaucher disease. *Genetics in Medicine*, Vol. 7 - No. 2.

Setting specific goals helps you get the most out of treatment—which means getting more out of life.

### Take Action

Because Gaucher disease is usually progressive, managing the disease and meeting your treatment goals will always involve regular monitoring tests, to help track changes in your body so that new symptoms can be caught early and treated before they cause lasting damage. At your next visit with your doctor, ask to review your treatment goals and current status, and discuss any details you don't fully understand.

For more details on the treatment and monitoring of Gaucher disease, please visit the Gaucher Connection website. [Go >>](#)

### Spotlight: <sup>Pr</sup>Cerezyme® Supply



Genzyme Canada has provided an update on the re-supply of <sup>Pr</sup>Cerezyme®, which includes recommendations for restarting <sup>Pr</sup>Cerezyme®.

Click here to access the latest update on the <sup>Pr</sup>Cerezyme® re-supply. [Go >>](#)

### Patient spotlight: Michael

- 40-year-old architect
- Making progress with his Gaucher
- Due to the Cerezyme supply shortage was concerned when he was unable to receive treatment
- Feels he will be able to better manage some of his Gaucher symptoms, once he re-starts <sup>Pr</sup>Cerezyme®



Read Michael's story about his therapeutic goals. [Go >>](#)