

MONITORING YOUR GAUCHER DISEASE TYPE 1

If you have **Gaucher disease type 1 (GD1)**, your healthcare provider will likely want to see you regularly to monitor your health. GD1 is progressive, meaning it may worsen over time. Monitoring your health is recommended to know whether your signs and symptoms are getting better, worse, or staying the same.

INITIAL ASSESSMENT

If you are newly diagnosed with GD1, your healthcare provider may perform tests to initially assess your signs and symptoms and establish baseline measurements to help track disease progression.

Blood Tests: Blood tests show how healthy your blood and organs are and the impact of GD1 on your body



Primary Blood Tests

- Hemoglobin
- Platelet count
- Biochemical markers
 - Chitotriosidase (CHITO)
 - Angiotensin-converting enzyme (ACE)
 - Tartrate-resistant acid phosphatase (TRAP)
 - Glucosylsphingosine (lyso-Gl-1)
- Gaucher disease genetic testing results



Additional Blood Tests as Indicated*

- Aspartate aminotransferase (AST) and/or alanine aminotransferase (ALT)
- Alkaline phosphatase
- Calcium
- Phosphorus
- Prothrombin time (PT)
- Partial thromboplastin time (PTT)
- White blood cell (WBC)
- Bilirubin
- Albumin
- Antibody sample[†]
- Total protein
- Iron
- Iron-binding capacity
- Ferritin
- Vitamin B₁₂
- Serum immunoelectrophoresis

*These tests may not apply to all patients. Assessments will be evaluated selectively based on each patient's age and clinical status.

[†]Antibody sample is done right before starting treatment and possibly 6 months after. It tests for the presence of antibodies against the enzyme used in the treatment.

Spleen and Liver: Spleen and liver imaging tests are needed to evaluate their size and structure



- Spleen volume: Magnetic resonance imaging (MRI) or computed tomography (CT)
- Liver volume: MRI or CT

Bone Scans: Bone scans show how healthy and dense your bones are



- Dual-energy X-ray absorptiometry (DEXA): Lower back (lumbar spine) and below the ball of hip joint (femoral neck)
- X-ray: Entire thigh (femur) bones and spine
- MRI: Entire thigh (femur) bones
- Bone age (for patients 14 years or younger)

Heart and Lungs: Heart tests show the activity and structure of your heart, and lung tests show if GD1 is affecting your lungs



- Electrocardiogram (ECG), chest X-ray, and echocardiogram (ECHO) for patients older than 18 years

Quality of Life: Quality-of-life (QoL) survey helps your healthcare provider understand how you feel about your health and well-being



- Short Form Health Survey (SF-36): Patient-reported functional health and well-being

Talk with your healthcare provider about questions you may have about these tests or your health.

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PERSONALIZED SUPPORT SERVICES

Sanofi CareConnect Personalized Support Services is a nationwide patient support program that provides eligible patients with access to a variety of resources, tailored education, and support related to living with a rare disease.

We have a dedicated team who will work with you to understand your specific needs. We understand that circumstances in your life may change, and we are here to help you every step of the way.

As you move from diagnosis to treatment, your dedicated CareConnect team will be by your side. We will work with you individually and offer educational support that can be tailored to your unique needs.

Visit [CareConnectPSS.com](https://www.careconnectpss.com) or call 1-800-745-4447 and select Option 3 to learn about patient support services.

MONITORING YOUR HEALTH WITH GAUCHER DISEASE TYPE 1 (GD1)

This schedule of assessments helps to monitor your GD1 over time, whether you are receiving treatment or not.

Depending on your individual medical needs, your healthcare provider will decide which tests you should have and how often.

ONGOING MONITORING

Assessment Type	Assessment Frequency	Patients Not On Therapy		Patients On Therapy		
		EVERY 12 MONTHS	EVERY 12-24 MONTHS	Not Achieved Therapeutic Goals	Achieved Therapeutic Goals	At Time of Dose Change or Significant Clinical Complication
Physical 	Comprehensive physical exam	●		●	● (once a year)	
	Blood Tests 	Hemoglobin	●		●	●
Platelet count		●		●	●	●
Biochemical markers • CHITO • ACE • TRAP • Lyso-GL-1		●		●	●	●
Additional blood tests		To be followed based on your age and clinical status if previous tests were abnormal				
Spleen and Liver 	Spleen volume (MRI or CT)		●	●	●	●
	Liver volume (MRI or CT)		●	●	●	●
Bones 	DEXA Lower back (lumbar spine) and below the ball of hip joint (femoral neck)		●	●	●	●
	X-rays Entire thigh (femur) bones and spine		●	●	●	●
	MRI Entire thigh (femur) bones		●	●	●	●
Heart and Lungs 	Recommended every 12 months to 24 months for patients with above-normal pulmonary blood pressures at initial assessment					
Quality of Life 	SF-36 Health Survey	●		●	● (once a year)	●

Please note: This is not an all-inclusive list. The tests in this monitoring schedule are recommended by the expert advisors of the International Collaborative Gaucher Group. It is at the discretion of your healthcare provider to determine the appropriate clinical assessments based on your individual needs.

Sanofi does not provide medical advice, diagnosis, or treatment. The health information contained herein is provided for general educational purposes only. Your healthcare professional is the best source of information regarding your health. Please consult your healthcare professional if you have any questions about your health or treatment.

Learn more about GD1 and types of treatment at [CareConnectPSS.com](https://www.CareConnectPSS.com)