

## How will my condition be monitored?

Regular follow-up examinations of all GIST patients are very important. Guidelines for monitoring follow the same principles developed for adults. This monitoring is done via physical examination and medical imaging techniques such as CT and PET scanning. Typically, follow-up CT scans are performed every three months. Blood tests are also needed to monitor the health status of patients receiving Gleevec or other drugs, although they do not directly indicate tumor response.

As pediatric GIST disproportionately affects girls, it is important to talk to your physician about the appropriate balance between frequent monitoring and the need to prevent long term side effects such as infertility. The difference between the monitoring efficacy of various imaging techniques (CT scan, PET scan and MRI) and the diagnostic reliability of each procedure should also be discussed.

## What are some of the differences in Adult and Pediatric GIST?

### Pediatric

- Affects females much more often than males
- Occurs mainly in the stomach as multiple nodules
- May have a less rapid natural course than adult GIST
- Treatment response to Gleevec is less well defined
- Is not understood as well as adult GIST
- Rarely has the typical *KIT/PDGFR*A gene mutations that are common in adults

### Adult

- Affects men and women almost equally
- Is better understood
- Has a more aggressive natural course
- Treatment with Gleevec is generally more effective

## Looking Toward the Future

GIST and Gleevec are considered by many to be a model for molecularly targeted cancer therapy. With high patient interest in clinical trials, and high interest in molecular targeted therapy by many drug companies, progress continues to be made in treating adult GIST. Pediatric GIST has significant differences from the adult disease and much more research is needed. Even though Pediatric GIST is very rare, there is considerable interest in wanting to understand it.

### Life Raft Group Resistance Research

The Life Raft Group has created a strategic resistance research plan that will enable us to direct grant funds to those research priorities with the greatest prospect of giving us new information and treatments as quickly as possible. We have created a supportive grant infrastructure that holds each researcher accountable for specific results, redirects resources when a project is exhausted and supplements new research when need arises. We have also created two separate tissue banks (one for pediatric GIST and one for adult GIST) to provide researchers with the tools that they need to perform their work.



*Pediatric GIST patients and their siblings came together in NYC. With the theme of "HOPE," they created works of art from surgical pants. From these they created a display, "Hanging in there," for an event in Nov. 2005.*

## The Life Raft Group

The LRG is an international patient support organization for people with GIST and their family members. The mission of the Life Raft Group is to ensure the survival of GIST patients while maintaining the quality of their lives. To accomplish this mission the Life Raft Group devotes its efforts to: Information & Support, Treatment Surveillance, Research, Patient Consultation & Assistance, and Advocacy.

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## LIFE RAFT GROUP



# Pediatric

# Gastro

# Intestinal

# Stromal

# Tumors

**Ensuring that no child has to face GIST alone**

*This pamphlet will tell you the basics about GIST and its treatment. It is intended to answer some of your questions about the disease, but is not a substitute for your physician's guidance and care.*

# What you need to know right away...

## What is Pediatric GIST?

GIST is the short name for Gastrointestinal Stromal Tumor, a type of sarcoma. Sarcomas are cancers of connective tissues, muscles, or bones. GIST is uncommon and your family doctor may never have seen a patient with GIST before. Pediatric GIST is even rarer. GIST was only recognized as a distinct disease a few years ago. Until then, GISTs were often incorrectly classified.

There are important differences between Pediatric GISTs and GISTs in adults. At an historic International Pediatric GIST meeting hosted by the Life Raft Group on November 12, 2004 in Montréal, Canada, over 20 experts agreed to a broad working definition: Pediatric GIST would include any patient with GIST whose disease appeared prior to the age of 18 (some patients present many years later during adulthood with recurrent disease). While the tumors of most adults demonstrate a mutation in the c-KIT oncogene and respond to Gleevec, the tumors of most pediatric patients do not have the mutation (this is called “wild-type KIT”) and responses to Gleevec are not as frequent. (An oncogene is a gene that causes normal cells to change into cancer cells.)

## How is GIST diagnosed?

Usually, a doctor first suspects GIST on the basis of a patient's symptoms, such as anemia or abdominal pain. GISTs may also be discovered during emergency surgery for perforations that cause infections or internal bleeding. Another possibility is that the GISTs may ulcerate and cause blood in the stool, vomiting of blood or anemia. GIST is not diagnosed by a blood test.

## Managing Treatment

In addition to your general practitioner or pediatrician, you may be treated by specialists

such as surgeons and medical oncologists. Radiologists may help with diagnosis and determination of whether the treatment is working. Your doctor may refer you to a specialized cancer center for further consultation. Many patients find that a strong personal support network of family and friends helps them to manage their condition.



Memorial Sloan-Kettering Cancer Center  
Multi-Disciplinary Team

## What is the prognosis for Pediatric GIST?



Pediatric GIST is a life-threatening disease. However, there are many long-term survivors of pediatric GIST. Pediatric GIST often behaves in a more indolent (slower growing) manner than adult GIST.

## Recurrence and Metastasis

Like most cancers, GIST can *metastasize* – it can spread from the original (primary) site to other locations in the body, especially the liver and the peritoneum (the membrane that lines the abdomen). These secondary tumors are called GIST *metastases* (“mets”). (Note that when GIST spreads to the liver, these secondary tumors are GIST tumors, *not*

liver cancers.) These cancers do not always metastasize. In adults, the risk of metastasis of a primary GIST can be estimated by a pathologist; however, it is unclear how accurately the methods used to estimate risk in adult GIST work to estimate the risk in Pediatric GIST. Recurrence and metastasis are common in GIST and continuing medical observation is essential.

## Will I need surgery?

Surgery is often required for removal of the primary GIST tumor. The goal is to remove the tumor and to achieve clear margins. The nature of the surgery will depend on the size and location of the GIST tumor.



As pediatric GIST is usually more slow growing, but does seem to recur, you may wish to talk to the surgeon about the pros and cons of immediate versus delayed surgery.

## Can Pediatric GIST be treated with drugs?

The most important improvement in adult GIST treatment has been the development of highly effective new drugs. Gleevec is the first drug approved (in 2002) specifically for GIST treatment. Although the data is still limited, Gleevec may be less effective for children, since pediatric GISTs do not usually have the mutations that Gleevec targets at the molecular level.

New drugs are being tried in a few Pediatric GIST patients. It is too early to know how well these drugs will work or what the side effects in children will be.

Specialized tests allow pathologists to identify GIST cells in a sample of tumor tissue. (A pathologist is a doctor who diagnoses diseases by laboratory tests, such as the examination of cells under the microscope.) Pathology is always critical for making the diagnosis of GIST. Almost all GISTs are positive in the “KIT” test (an immunohistochemistry test, also called “brown staining,” for identifying KIT protein over-expression). This test determines whether the cancer cells produce a protein called KIT.

## Why did I get GIST?

We don't understand why most people get GIST. GIST strikes young and old, rich and poor. Pediatric GIST occurs disproportionately amongst girls. Sometimes GIST runs in families but most cases do not. There are no known environmental, occupational, or lifestyle causes of GIST. In adults, GIST results when mutations occur in certain genes and in certain cells, but we do not know why these mutations happen. The mutations that commonly occur in adult GISTs do not usually occur in Pediatric GIST. Any mutation(s) that might cause Pediatric GIST are unknown.

## Is GIST the same thing as “stomach cancer”?

No. GIST tumors can start in any part of the digestive tract including the esophagus, stomach, colon, and rectum. GIST is very different from the more common breast, lung, stomach, and colon cancers (carcinomas). In children, GIST tumors usually start in the stomach. Often, more than one stomach tumor will be found either initially or at a later date.

