What is CGD?

Chronic granulomatous disease (CGD) is a rare disease found in 1 out of every 200,000 people in the United States. People with CGD have an immune system that isn’t working right. A healthy immune system can fight off infections from bacteria and fungi, also called pathogens. White blood cells of the immune system go to the spot where the infection is in the body and kill it. An immune system with CGD can’t kill infections from bacteria and fungi, and these infections become life-threatening. When white blood cells rush to the site of the infection and then cannot kill it, they build up. These buildups of white blood cells form lumps called granulomas. This is how the disease got its name.

Getting CGD

CGD is not contagious, which means it’s not something you catch like a cold or flu. CGD is a disease a child is born with. It is passed down in the genes from the parents. There are 2 major ways a child can get CGD. The most common way is called X-linked because it is caused by a faulty gene on the X chromosome. Usually only males get X-linked CGD. Males have 1 X and 1 Y chromosome, so if they get the X chromosome that doesn’t work, they will have CGD. Females have 2 X chromosomes, so they don’t usually get X-linked CGD, except in rare cases when an X chromosome becomes inactive. Otherwise they can only be carriers of X-linked CGD and can pass it down to their children. The other way a child can get CGD is called autosomal recessive. This means that the child with CGD has 2 faulty genes, 1 from each parent. Both males and females can get autosomal recessive CGD.

Spotting CGD

CGD is usually diagnosed before a child is 5 years old. However, people with milder forms of CGD may not have symptoms until they are in their teens or even adulthood. Some common signs and symptoms of CGD to watch out for are:

- Infections caused by certain types of bacteria or fungi that patients cannot fight off on their own and that frequently affect many areas of the body, including the lungs, lymph nodes, liver, bones, or skin
- Infections that are serious, show up suddenly, and keep coming back
- Abdominal (stomach area) pain, diarrhea, and weight loss
- Vomiting after eating
- Pain or difficulty urinating (peeing) or swallowing
- Slow growth in childhood (for example, children being small for their age)
- Granulomas, which usually appear in the bladder and intestines

Please see Important Safety Information on pages 3 and 4.
Making healthy lifestyle choices

Infections caused by bacteria and fungi can live in places all around us. They live in places like gardens (in mulch), playgrounds (in woodchips), and in lakes and ponds. People with CGD can still have fun outdoors, but they should stay away from these places. Here are some other tips to avoid infections:

• Don’t rake leaves or be around someone raking leaves
• Stay out of barns, caves, and other musty places
• Don’t go on hay rides
• Wash all cuts and scrapes with soap and water
• Make sure to talk to your doctor about any cut or fever
• Don’t smoke or inhale smoke
• Vaccinate all pets

Treating CGD

You have options to help lower your risk of getting a serious infection because of CGD. Recommended treatment for CGD includes antibiotics, antifungals, and ACTIMMUNE® (Interferon gamma-1b). It’s important to talk to your doctor about your options.

Antibiotics
Infections from bacteria can show up in many places in the body, including the lungs, bones, and blood. Antibiotics are used to help prevent bacterial infections by destroying or slowing down the growth of bacteria.

Antifungals
Infections from fungi, like Aspergillus species, can happen in many places in the body. A fungal infection in the lungs can cause pneumonia, which is very dangerous. Antifungal medicine is toxic to fungi and helps destroy it and prevent it from spreading and damaging the body.

ACTIMMUNE® (Interferon gamma-1b)
ACTIMMUNE® contains a protein called interferon gamma that’s almost the same as the one the body makes naturally as it fights infections. ACTIMMUNE®, taken along with prescribed antifungal and antibiotic medicines, plays an important role in lowering the risk of serious infections.*

Bone marrow transplantation
Bone marrow transplant, or BMT, is an option for some people and is a possible cure for CGD. BMT involves taking stem cells from a healthy person and transplanting them into the person with CGD. Talk to your doctor for more information about the risks and benefits of BMT.

*An infection is serious if you have to go to the hospital and get intravenous (IV) antibiotics.

Select Safety Information

Don’t use ACTIMMUNE® if you are allergic to interferon-gamma, E coli-derived products, or any ingredients contained in the product.

Please see Important Safety Information on pages 3 and 4.
Important Safety Information

What is ACTIMMUNE® (Interferon gamma-1b) used for?

ACTIMMUNE® is part of a drug regimen used to treat Chronic Granulomatous Disease, or CGD. CGD is a genetic disorder, usually diagnosed in childhood, that affects some cells of the immune system and the body's ability to fight infections effectively. CGD is often treated (though not cured) with antibiotics, antifungals, and ACTIMMUNE.

ACTIMMUNE is also used to slow the worsening of severe, malignant osteopetrosis (SMO). SMO is a genetic disorder that affects normal bone formation and is usually diagnosed in the first few months after birth.

When should I not take ACTIMMUNE?

Don’t use ACTIMMUNE if you are allergic to interferon-gamma, *E coli*-derived products, or any ingredients contained in the product.

What warnings should I know about ACTIMMUNE?

At high doses, ACTIMMUNE can cause (flu-like) symptoms, which may worsen some pre-existing heart conditions. ACTIMMUNE may cause decreased mental status, walking disturbances, and dizziness, particularly at very high doses. These symptoms are usually reversible within a few days upon dose reduction or discontinuation of therapy.

Bone marrow function may be suppressed with ACTIMMUNE, and decreased production of cells important to the body may occur. This effect, which can be severe, is usually reversible when the drug is discontinued or the dose is reduced.

Taking ACTIMMUNE may cause reversible changes to your liver function, particularly in patients less than 1 year old. Your doctor should monitor your liver function every 3 months, and monthly in children under 1 year.

In rare cases, ACTIMMUNE can cause severe allergic reactions and/or rash. If you experience a serious reaction to ACTIMMUNE, discontinue it immediately and contact your doctor or seek medical help.

What should I tell my healthcare provider?

Be sure to tell your doctor about all the medications you are taking.

Tell your doctor if you:

• are pregnant or plan to become pregnant or plan to nurse
• have a cardiac condition such as irregular heartbeat, heart failure, or decreased blood flow to your heart
• have a history of seizures or other neurologic disorders
• have, or have had, reduced bone marrow function. Your doctor will monitor these cells with blood tests at the beginning of therapy and at 3-month intervals on ACTIMMUNE therapy

What are the side effects of ACTIMMUNE?

The most common side effects with ACTIMMUNE are “flu-like” symptoms such as fever, headache, chills, muscle pain, or fatigue, which may decrease in severity as treatment continues. Bedtime administration of ACTIMMUNE may help reduce some of these symptoms. Acetaminophen may be helpful in preventing fever and headache.
What other medications might interact with ACTIMMUNE?

Some drugs may interact with ACTIMMUNE to potentially increase the risk of damage to your heart or nervous system, such as certain chemotherapy drugs. Tell your doctor about all other medications you are taking.

Avoid taking ACTIMMUNE at the same time as a vaccination.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088. You may also contact the Horizon Pharma Medical Information Department toll-free at 1-866-479-6742 or medicalinformation@horizonpharma.com.

The risk information provided here is not comprehensive. To learn more, talk about ACTIMMUNE with your healthcare provider or pharmacist. The FDA-approved product labeling can be found at http://www.ACTIMMUNE.com or 1-866-479-6742.

For more information about CGD and ACTIMMUNE®, visit ACTIMMUNE.com.