

Chronic granulomatous disease (CGD) Basics



CGD facts

Chronic granulomatous disease (CGD) is a rare disease. There are about 20 children born with CGD each year in the United States. People with CGD have an immune system that doesn't work properly. A healthy immune system usually prevents infections from becoming serious. An immune system with CGD can typically stop viral infections like a cold or flu, but can't stop serious infections caused by certain bacteria and fungi. Since CGD is a chronic condition, it means that a person will have the disease for all his or her life.



What is a granuloma? When the immune system finds harmful things in the body, like bacteria and fungi, it sends immune cells to kill them. If the infection doesn't go away, more immune cells show up to try to kill the bacteria or fungi. In time, the extra immune cells build up and form a hard lump called a granuloma. This is how the disease got its name.

As the granulomas build up, they can cause problems in places like the intestine, and can cause symptoms such as **pain in the stomach, diarrhea, and weight loss**. These symptoms are similar to symptoms of Crohn's disease, which is a type of inflammatory bowel disease (IBD).



The problem with pathogens

Pathogens include harmful bacteria and fungi that can cause serious, life-threatening infections in people with CGD. A healthy immune system usually prevents infections from becoming serious. But people with CGD are at a greater risk of unusual and repeat infections because their immune system can't fight them off.

Bacteria and fungi can be found in places all around us:



woodchips
playgrounds



soil, mulch
gardens,
houseplants



dust
new
construction



mold
barns, sheds,
basements, caves



dander, dirt
carpets, pets



**standing
water**
lakes, ponds

Certain times of the year can cause bacteria and fungi to be more plentiful.

- Spring: Rain showers can create puddles filled with bacteria
- Summer: Dry weather can lead to more dirt and spores in the air
- Fall: It's best to avoid hayrides or being around anyone raking leaves

Please see **Important Safety Information** on pages 5 and 6.

Please see **Full Prescribing Information** at ACTIMMUNE.com.





CGD starts in the genes

CGD isn't something you can catch. It's a genetic condition, which means you're born with it. It's passed down to a child from one or both parents who can be "carriers." A carrier doesn't have the disease, but his or her children can end up with it. Carriers of CGD have 1 normal copy of the gene and 1 copy of the gene that doesn't work right. There are 2 types of CGD: X-linked and autosomal recessive.

X-linked CGD

The most common form of CGD is X-linked. It's passed down from the mother because she carries a faulty X chromosome. This means she is a carrier of CGD. Usually only males get X-linked CGD. A male born to a carrier mother has a 50% chance of having CGD.



can lead to:

- a boy with CGD
- a boy or girl without CGD
- a carrier girl

Autosomal recessive CGD

Both males and females can get autosomal recessive CGD. A child needs 2 copies of a gene that doesn't work, 1 from each parent, to have autosomal recessive CGD. Any child born of carrier parents has a 25% chance of having autosomal recessive CGD, and a 50% chance of being a carrier.



can lead to:

- a boy or girl with CGD
- a boy or girl without CGD
- a carrier boy or girl



CGD signs and symptoms

CGD is usually diagnosed before a child is 5 years old. Some people with milder forms of CGD may not show signs until they are a teen or adult. Common signs and symptoms to look for include:

- Serious and frequent infections that can occur in many places in the body, including the lungs (pneumonia), liver, and bones
- Skin infections that cause boils, blisters, and sores that don't go away
- Diarrhea, weight loss, or abdominal pain due to inflammation in the intestines
- Pain or difficulty eating or going to the bathroom
- Vomiting after eating
- Swollen lymph nodes (These are glands found all over the body, but mostly in the neck, armpits, and groin areas)
- Fever, cough, being tired all of the time, or bone and/or joint pain

CGD can be misdiagnosed as inflammatory bowel disease (IBD) because the symptoms are similar.

Please see Important Safety Information on pages 5 and 6.

Please see Full Prescribing Information at ACTIMMUNE.com.





Testing for CGD

A doctor can diagnose CGD by ordering a lab test called a dihydrorhodamine test, or DHR test. This involves taking a sample of blood and sending it to a lab. The DHR test is preferred over other CGD tests because it's simple and more accurate. The DHR test can also be used to find out if any other family members—such as a brother, sister, or aunt—have CGD or are X-linked carriers of CGD. If any family members have CGD-like symptoms, the test can confirm that person has CGD.



Treating CGD

In addition to making health and lifestyle changes, there are medicines that can help manage CGD. These medicines include antibiotics, antifungals, and ACTIMMUNE® (Interferon gamma-1b). It's important to talk to your doctor about treatment options that work best for you or your loved one.

Antibiotics

Infections from bacteria can appear in many places, including the skin, lungs (pneumonia), bones, and lymph nodes. Antibiotics, such as Bactrim®, are used to help prevent bacterial infections by killing or slowing down the growth of bacteria. Common side effects of antibiotics include diarrhea and upset stomach.

Antifungals

Infections from fungi, like *Aspergillus* species, can be very serious for people with CGD. Fungal infections can appear in many places, including the lungs (pneumonia), liver, and bones. Antifungal medicine, such as Sporanox®, kills the fungus and helps prevent it from spreading and damaging the body.

ACTIMMUNE® (Interferon gamma-1b)

ACTIMMUNE® helps lower the risk of serious infection* in people with CGD. It can do this because it contains a protein called interferon gamma. This protein is almost the same as the one the body's own immune system makes naturally as it fights infections. That's why ACTIMMUNE®, taken along with prescribed antifungal and antibiotic medicines, plays an important role in helping to protect people from serious infections. The most common side effects are flu-like symptoms and include fever, headache, chills, muscle pain, and being tired. These symptoms may lessen as treatment continues.

Bone marrow transplant

Bone marrow transplant, or BMT, is an option for some patients and provides a possible cure for CGD. A BMT replaces an immune system that isn't working properly with one that is healthy. Talk to your doctor for more information about the risks and benefits of a bone marrow transplant.

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

Please see Important Safety Information on pages 5 and 6.

Please see Full Prescribing Information at ACTIMMUNE.com.





Being a carrier of CGD

Hearing that you're a carrier of CGD could come as a complete surprise to you. You may be thinking "How did this happen?" or "What did I do to get this?" Remember, you did nothing wrong. CGD is just part of who you are. It's common for carriers of CGD to have feelings of sadness or guilt, especially in the beginning. This may lead to trouble sleeping and anxiety. If you're having any of these symptoms and they continue over time, it's important to talk to your doctor to get the support you may need. When you're taking care of yourself, you're making sure that you're healthy enough to care for your child with CGD, too.

Carrier symptoms

Female carriers of CGD can have symptoms that may need medical attention. For example, it's common for carriers to have symptoms of a type of lupus. Depending on how serious the symptoms are, your doctor may prescribe medicine and suggest treatments that work best for you.

Symptoms to look for include:



skin rash on
face, hands,
and chest



sensitivity to
the sun



pain and
swelling in
the joints



feeling tired
all the time



weight
loss

Genetic counselor

Besides your doctor, a genetic counselor plays an important role in understanding CGD.

A genetic counselor can:

- Explain what happens when someone inherits CGD
- Trace your family's history with the disease
- Discuss family planning options and testing for CGD

Please see Important Safety Information on pages 5 and 6.

Please see Full Prescribing Information at ACTIMMUNE.com.



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.

Please see Full Prescribing Information at ACTIMMUNE.com