

RECOGNIZING AUTOSOMAL RECESSIVE CHRONIC GRANULOMATOUS DISEASE (CGD)

A portrait of a woman with long, straight brown hair, smiling. She is wearing a blue denim jacket over a dark green shirt.

CAITLIN

INDICATIONS AND USAGE

ACTIMMUNE® (Interferon gamma-1b) is indicated:

- For reducing the frequency and severity of serious infections associated with Chronic Granulomatous Disease
- For delaying time to disease progression in patients with severe, malignant osteopetrosis

A portrait of a woman with long, wavy reddish-brown hair, smiling. She is wearing a grey zip-up top.

TATUM

SELECT IMPORTANT SAFETY INFORMATION

CONTRAINDICATIONS

- In patients who develop or have known hypersensitivity to interferon-gamma, *E coli*-derived products, or any component of the product

Please see Important Safety Information on page 8 and [click here](#) for ACTIMMUNE® Full Prescribing Information.

ACTIMMUNE®
(Interferon gamma-1b)

ACTIMMUNE® is approved by the FDA specifically
for patients with CGD. Learn more at [ACTIMMUNEhcp.com](https://www.ACTIMMUNEhcp.com).

35% of CGD cases are inherited in an autosomal recessive manner¹

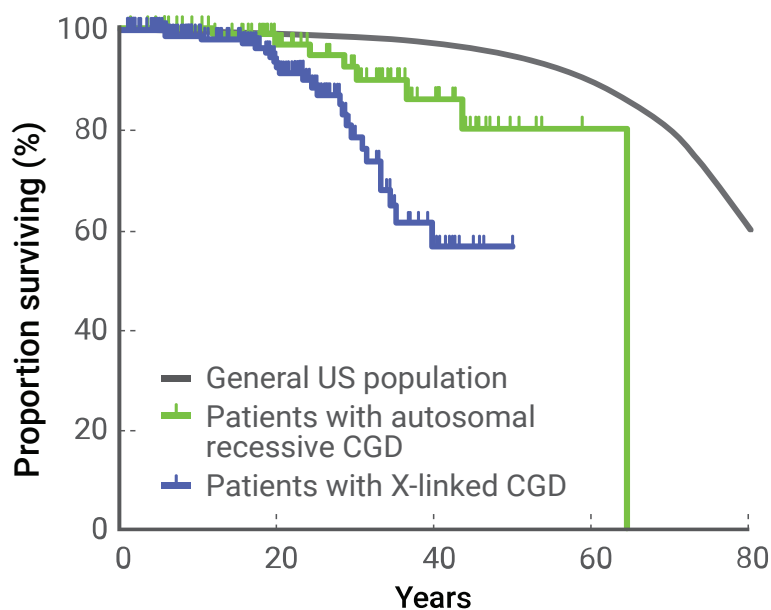
Diagnosis of autosomal recessive chronic granulomatous disease (CGD) may be delayed because of milder disease manifestations compared with those of X-linked CGD²

- 1 Autosomal recessive CGD affects both males and females^{2,3}
- 2 Patients may present later in life compared with patients with X-linked CGD³
- 3 Autosomal recessive CGD includes 4 different forms of genetic defects, and this spectrum explains a considerable heterogeneity of disease severity^{1,3,4}

People living with CGD can experience frequent, repeat, life-threatening infections, regardless of inheritance pattern and **survival is well below that of the general population**²⁻⁴

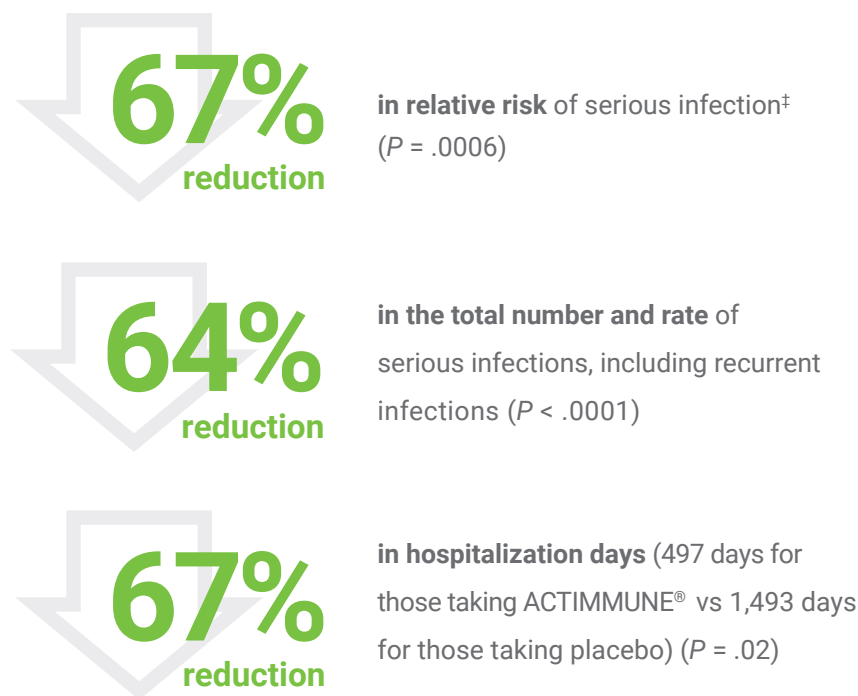
High risk of mortality^{4,5}

Kaplan-Meier survival curves for patients with X-linked and autosomal recessive CGD.⁴



Reduce the risk of serious infections for patients with autosomal recessive CGD⁶

In a clinical trial of patients with various types of CGD taking ACTIMMUNE® (Interferon gamma-1b) vs placebo, ACTIMMUNE® demonstrated a^{*,†,6,7}:



ACTIMMUNE® is the only FDA-approved biologic therapy indicated for reducing the frequency and severity of serious infections associated with CGD

ACTIMMUNE® (n = 63)
Placebo (n = 65)

*In subgroup analyses, ACTIMMUNE® was beneficial regardless of age or type of CGD inheritance.

†More than 85% of patients were receiving prophylactic antibiotics in addition to either ACTIMMUNE® or placebo.

‡Serious infection is defined as a clinical event requiring hospitalization and intravenous antibiotics.

SELECT IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS

• **ACTIMMUNE should be used with caution in patients with:**

- Pre-existing cardiac conditions, including ischemia, congestive heart failure, or arrhythmia
- Seizure disorders or compromised central nervous system function; reduce dose or discontinue

- Myelosuppression, or receiving other potentially myelosuppressive agents; consider dose reduction or discontinuation of therapy
- Severe renal insufficiency
- Age <1 year

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Delayed diagnosis due to patient's sex and age at first serious infection

Tatum

Age at diagnosis: 14 years

CGD inheritance pattern: autosomal recessive

Therapy: ACTIMMUNE® (Interferon gamma-1b),
trimethoprim-sulfamethoxazole, posaconazole

This case study is the experience of one individual and results may vary.

“The doctor said,
‘Mold can cause
pneumonitis in people
with a particular genetic
disorder, **but she’s too
old to have that.**”

JUSTIN,
father of Tatum

SELECT IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS (CONT'D)

- **Monitoring:**

- Patients begun on ACTIMMUNE before age 1 year should receive monthly assessments of liver function. If severe hepatic enzyme elevations develop, ACTIMMUNE dosage should be modified

- Monitor renal function regularly when administering ACTIMMUNE in patients with severe renal insufficiency; accumulation of interferon gamma-1b may occur with repeated administration. Renal toxicity has been reported in patients receiving ACTIMMUNE

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Tatum's case history

- **2001 Born**
- **2007 Salivary gland infection and abscess**
 - Infection refractory to treatment
 - Salivary gland surgically removed
- **2012 Severe sinus infection**
 - Refractory to 2 courses of antibiotics
 - Resolved with steroids
- **2016 Mucocutaneous pneumonitis caused by *Aspergillus* species**
 - 39-day hospitalization
 - 19 days in the intensive care unit (ICU)
 - 12 days on ECMO
 - 22 pounds lost

Diagnosed with autosomal recessive CGD 3 weeks after initial presentation, during week 2 of a 5-week hospitalization. During hospitalization, started on ACTIMMUNE® (Interferon gamma-1b), antibiotic, and antifungal

 - 3 months on blood thinners due to ECMO
 - Required physical, occupational, and speech therapy
- **2017 Infected ingrown toenails**
 - Posaconazole dose temporarily increased
 - Partially alleviated with steroid cream
 - Portion of toenails removed preventively
- **2020 Taking medications as prescribed**
 - With continued adherence, Tatum has not experienced a serious infection since 2016

SELECT IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS (CONT'D)

• **Pregnancy, Lactation, and Fertility:**

- ACTIMMUNE should be used during pregnancy only if the potential benefit outweighs the potential risk to the fetus

- Use of ACTIMMUNE by lactating mothers is not recommended. ACTIMMUNE or nursing should be discontinued dependent on the importance of the drug to the mother
- Long-term effects of ACTIMMUNE on fertility are not known

Currently adherent to her medications and living a full life with CGD

Caitlin

Age at diagnosis: 7 years

CGD inheritance pattern: autosomal recessive

Therapy: ACTIMMUNE® (Interferon gamma-1b), trimethoprim-sulfamethoxazole, ciprofloxacin, and itraconazole

This case study is the experience of one individual and results may vary.

“I know I need to be careful, but **I don't live in a bubble. I live a fun, fulfilling life** and don't let CGD hold me back.”

CAITLIN,
lives with autosomal
recessive CGD

SELECT IMPORTANT SAFETY INFORMATION

ADVERSE REACTIONS

- The most common adverse experiences occurring with ACTIMMUNE therapy are “flu-like” symptoms such as fever, headache, chills, myalgia, or fatigue,

which may decrease in severity as treatment continues, and may be minimized by bedtime administration of ACTIMMUNE. Acetaminophen may be used to prevent or partially alleviate the fever and headache

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Caitlin's case history

1985 Born

1992 Severe pain in stomach led to exploratory surgery

- Granuloma found on intestines during surgery for a ruptured appendix
- Referred to hematologist

Elbow scrape from bicycle fall failed to heal

- Biopsy performed and granuloma was found

Diagnosed with CGD by genetic testing within a month and **started on ACTIMMUNE® (Interferon gamma-1b)** and trimethoprim-sulfamethoxazole

- Family tested for CGD. Mother and father confirmed to be carriers; sister does not have CGD and is not a carrier

1998 Aspergillosis infection in lungs

- 2 months in hospital

1999 Itraconazole added to treatment regimen

2013 Developed esophagitis

- Hospitalized for 1.5 weeks

2014 Serious infection due to blocked bowel from earlier surgery

- Hospitalized for 1 week

2020 Taking medications as prescribed

- With continued adherence, Caitlin has not experienced a serious infection since 2014

SELECT IMPORTANT SAFETY INFORMATION

ADVERSE REACTIONS (CONT'D)

- Isolated cases of acute serious hypersensitivity reactions have been observed in patients receiving ACTIMMUNE
- Reversible neutropenia, thrombocytopenia, and elevations of AST and/or ALT have been observed during ACTIMMUNE therapy

- At doses 10 times greater than the weekly recommended dose, ACTIMMUNE may exacerbate pre-existing cardiac conditions, or may cause reversible neurological effects such as decreased mental status, gait disturbance, and dizziness

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DRUG INTERACTIONS

- Concomitant use of drugs with neurotoxic, hematotoxic, or cardiotoxic effects may increase the toxicity of interferons
- Avoid simultaneous administration of ACTIMMUNE with other heterologous serum protein or immunological preparations (eg, vaccines)

ADVERSE REACTIONS

- The most common adverse experiences occurring with ACTIMMUNE therapy are “flu-like” symptoms such as fever, headache, chills, myalgia, or fatigue, which may decrease in severity as treatment continues, and may be minimized by bedtime administration of ACTIMMUNE. Acetaminophen may be used to prevent or partially alleviate the fever and headache
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References: 1. Noack D, Rae J, Cross AR, et al. Autosomal recessive chronic granulomatous disease caused by defects in NCF-1, the gene encoding the phagocyte p47-phox: mutations not arising in the NCF-1 pseudogenes. *Blood*. 2001;97(1):305-311. 2. Holland SM. Chronic granulomatous disease. *Hematol Oncol Clin North Am*. 2013;27(1):89-99. 3. Leiding JW, Holland SM. Chronic granulomatous disease. In: Pagon RA, ed. *GeneReviews*. Seattle, WA: University of Washington, Seattle; 1993-2016. 4. Kuhns DB, Alvord WG, Heller T, et al. Residual NADPH oxidase and survival in chronic granulomatous disease. *N Engl J Med*. 2010;363(27):2600-2610. 5. Chronic granulomatous disorder: A guide for medical professionals. CGD Society website. www.cgd-society.org/static/media/up/CGDS_Medical_Guide.pdf. Accessed September 16, 2019. 6. The International Chronic Granulomatous Disease Cooperative Study Group. A controlled trial of interferon gamma to prevent infection in chronic granulomatous disease. *N Engl J Med*. 1991;342(8):509-516. 7. ACTIMMUNE (Interferon gamma-1b) [prescribing information] Horizon.



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