

Chronic Myelomonocytic Leukemia

A GIVING SMARTER GUIDE TO ACCELERATING RESEARCH PROGRESS

An Executive Summary

**LACK OF BASIC DISEASE BIOLOGY UNDERSTANDING.
FEW TOOLS TO DIAGNOSE PATIENTS.
POOR TREATMENT OPTIONS.
NO CURE.**

Patients and families affected by chronic myelomonocytic leukemia (CMML) face many challenges and critical unmet needs, issues mirrored by the current state of the disease's research. In September 2015, the Milken Institute Philanthropy Advisory Service convened leading academic, clinical, industry, and foundation stakeholders to discuss the state of CMML science and the key challenges impeding research progress. In a new Giving Smarter Guide, the Philanthropy Advisory Service presents the key unmet needs for the disease, and recommendations of how strategic philanthropic investments can change the trajectory of research and ultimately benefit CMML patients.

WHOM AND HOW TO TREAT

Each year in the United States, leukemia claims the lives of more than 24,000 people and affects an estimated

LEUKEMIA CLASSIFICATIONS

- > Acute myeloid leukemia (~12 TYPES)
- > Acute lymphoblastic leukemia (2 TYPES)
- > Acute promyelocytic leukemia (2 TYPES)
- > Acute monocytic leukemia (2 TYPES)
- > Acute erythroid leukemia (2 TYPES)
- > Acute megakaryoblastic leukemia
- > Acute myelomonocytic leukemia (2 TYPES)
- > Chronic myeloid leukemia
- > Chronic myeloproliferative disorders (5 TYPES)
- > Myelodysplastic syndromes (6 TYPES)
- > Mixed myelodysplastic/myeloproliferative syndromes (MDS/MPN, 3 TYPES)

330,000 more. On a societal scale, the economic burden of leukemia is immense, estimated at \$5.44 billion annually, and projected to increase by 25 percent over the next 10 years. While the incidence and burden of the disease are daunting, sustained public and private investments in leukemia research have refined what was known as a “disease of the blood” into approximately

CMML PATIENTS NEED BETTER DRUGS. Current drugs don't work, and if they do have a transient effect, we have no idea why.

PATIENT DIAGNOSIS A KEY CHALLENGE, as it is very difficult to diagnose a CMML patient among closely related or similar diseases.

38 different types of leukemia. With targeted research into specific leukemias, medical breakthroughs have led to an impressive quadrupling of the overall five-year survival rate for leukemia patients, from 14 to 60.3 percent.

While specific classes of leukemia patients have experienced dramatic success in the last several years, those affected by CMML have not been as fortunate. As a chronic and aggressive disease, patients diagnosed with CMML have a median survival of 15 to 20 months. Furthermore, a small percentage of CMML patients are cancer survivors, who developed the disease as a complication of chemotherapy for their previous cancer.

However, recent advancements in CMML diagnosis have led to its re-classification as a myelodysplastic/myeloproliferative syndrome (MDS/MPN). If the historical arc of leukemia research is applied to CMML, then improved diagnosis coupled with dedicated

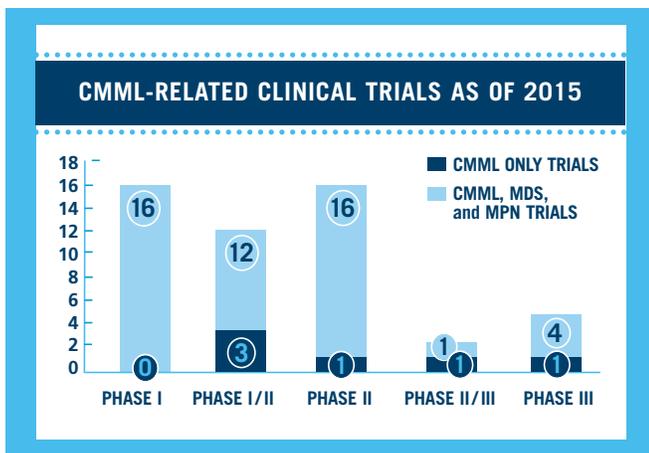
research will greatly benefit CMML patients. Strategic investments in diagnostic and prognostic tools, infrastructure, and discovery science can play a catalytic role in developing novel, targeted treatments options for CMML, and the related MDS/MPN syndromes.

The few drugs approved for CMML patients are not optimal, and their limited and transient effects on the disease are poorly understood. In this Giving Smarter Guide, we outline the key barriers to CMML research progress that impede the new and needed treatments for CMML patients. These barriers include:

- Limited experimental treatment options
- Absence of a patient registry and clinical trial infrastructure
- Overall lack of understanding of the basic and translational biology

Readers will be able to use this guide to pinpoint research solutions aligned with their interests. This guide will help to answer the following questions:

- Why should I invest in CMML research?
- What key things should I know about this disease?
- What is the current standard of care?
- What is the current state of CMML research efforts?
- What are the barriers preventing development of new therapeutics?
- How can philanthropy expand infrastructure to support CMML research and advance new therapies?



FOR THE FULL GIVING SMARTER GUIDE, VISIT [PAS.FASTERCURES.ORG/REPORTS](https://pas.fastercures.org/reports)

The Philanthropy Advisory Service, a program of the Milken Institute, counsels philanthropists, family offices, wealth advisors, and foundations seeking to make transformative investments in medical research. We provide comprehensive, digestible information that helps philanthropists evaluate research efforts and funding opportunities in various disease areas. Our analysis is shaped by scientific advisory boards, in-depth due diligence, and an objective framework for evaluation.