

When No News Is Good News: The Importance of Cancer Prevention Research

If you carry genetic risk factors for cancer, is there anything preventative you can do?

May 23, 2023 By [Damon Runyon Cancer Research Foundation](#)

Imagine you have just learned that you are genetically predisposed to developing blood cancer. Everyone acquires mutations in their blood as they age, your doctor explains, but certain mutations carry higher risk than others. When a mutation occurs in a blood stem cell and confers an evolutionary advantage, that mutant blood stem cell will give rise to a whole subpopulation of cells with the same mutation. This is known as clonal hematopoiesis (CH), and again, it is a normal age-related phenomenon. But when paired with a condition known as cytopenia, marked by a low blood cell count, CH almost invariably develops into blood cancer. At this point, the doctor recommends that you make an appointment with Dr. Kelly Bolton.

Damon Runyon Clinical Investigator Kelly L. Bolton, MD, PhD, has a background in genetic epidemiology, or the study of inherited risk. Part of her research involves analyzing disease outcomes in large population datasets to make recommendations for people who carry genetic risk factors. Should someone with a known mutation “wait and see”? Should they engage in active surveillance, via frequent scans or blood draws? And, most pressingly, is there anything preventative they can do?

For some cancers, such prophylactic interventions are available. Patients at increased risk for breast cancer, for example, may opt to have a mastectomy or take antiestrogen therapy; those wary of lung cancer may decide to quit smoking. Unfortunately, there are no options yet for preventing blood cancer. But with support from Damon Runyon, Dr. Bolton is working to change that.

A New Kind of Clinical Trial

In late 2021, Dr. Bolton and her colleagues Memorial Sloan Kettering Cancer Center launched two clinical trials for drugs that target a mutation in the IDH1 or IDH2 gene, both of which have been identified as blood stem cell mutations that cause CH and increase the likelihood of developing cancer. The team is recruiting those at highest risk: healthy individuals who harbor one of these

mutations and have cytopenia. The drugs, known as enasidenib and ivosidenib, are designed to block the mutant IDH protein, which may improve blood cell counts. If successful, they would be the first genetically targeted preventative therapies for cancer.

In fact, this trial is unusual in a number of ways. For one, the goal is to conduct it entirely virtually. The team will ship the drug to each participant and work with their local provider to make sure that they remain healthy as they complete the regimen. Dr. Bolton notes that a preventative trial is “the perfect setting” to employ virtual research methods because the participants are not sick and the drug is safe, but she expects to see future clinical trials follow suit.

“This is probably going to be the model,” she says. “The few studies that have happened in the COVID era that have been fully virtual—they’re great because they increase participation. They lower the barrier to enrolling and they reduce the cost, so people of more diverse economic and geographic backgrounds are able to participate.”

For now, though, recruitment is limited to 15 participants.

“It’s essentially a proof-of-concept trial,” Dr. Bolton explains. “Prevention studies are very, very long-term and very expensive, and they probably have to be done on a federal level. But this kind of work is what you need to get federal funding for those bigger trials.”

In for the Long Haul

Cancer prevention work, as Dr. Bolton knows well, does not always get the same level of funding and attention that other areas of cancer research do. There are several reasons for this. Prevention studies necessarily take a long time, and their success is often difficult to quantify. They rely upon epidemiology, not discovery, and this makes for less flashy headlines.

“The people who figured out that you need to raise taxes on cigarettes to prevent people from smoking—things like that have huge public health consequences,” she says. “But you don’t see people getting the Nobel prize for making something not happen.”

There are also challenges involved in asking healthy individuals to undergo treatment.

“The drug has to be safe, well-tolerated over long periods of time, and effective. There are not a lot of examples of drugs like this, especially because molecularly targeted therapies are so new,” Dr. Bolton explains. “But that’s going to change. The era of medicine that we’re entering is focused on developing molecularly targeted therapies, so we should have more options moving forward.”

In the meantime, Dr. Bolton continues to counsel those who come into her clinic at Washington University in St. Louis, assessing their risk levels and monitoring their health over time, while investigating risk-related questions in her lab. She is grateful that Damon Runyon recognizes the importance of this work.

“We are using population-based studies to try to understand cancer risk, and that teaches us about mechanism and prevention strategies. Our clinical trial is a great example of how we can take this from computer bench to bedside. I think having more people involved in implementation science and epidemiology is really important.”

[This post](#) was originally published by [Damon Runyon Cancer Research Foundation](#). It is republished with permission.

© 2026 Smart + Strong All Rights Reserved.

<http://beta.docker.cancerhealth.com/blog/news-good-news-importance-cancer-prevention-research>