NCI Director Kimryn Rathmell Marks One Year in Cancer Research and Much to Celebrate

There's no shortage of cancer research that is having far-reaching effects, writes Kimryn Rathmell, director of the National Cancer Institute.

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By Kimryn Rathmell, MD, PhD, director of the National Cancer Institute

As 2024 comes to a close, I've been reflecting on what's transpired in cancer and cancer research over the past year. Yesterday, in fact, marked my one-year anniversary as NCI director so it's been a year I'll personally never forget.

I've met so many amazing people in the cancer research community, learned about what they do, how they do it, and why they do it. And I can say unequivocally that I have gained an even greater appreciation for their ingenuity, passion, and dedication to their work.

The dividends of this research were on full display this year, including long overdue advances in the treatment of some of the most stubborn cancers and studies that continued to unravel the complex and unwieldy biology of this collection of diseases under the umbrella of "cancer."

Among the findings I keep returning to are those from a clinical trial testing the immunotherapy drug dostarlimab (Jemperli). The trial enrolled people with locally advanced rectal cancer whose tumors had a malfunction in their DNA repair capabilities called mismatch repair deficiency. According to results presented in June, of 42 patients treated with dostarlimab for 6 months, all 42 had a complete response. A 100% complete response rate! And in most trial participants, those responses lasted for more than 2 years.

That small trial and its remarkable results epitomize the ideal of modern cancer research: patients were enrolled based on a tumor marker that studies have suggested can predict response to immunotherapy and a drug was tested that should have fewer side effects than the standard treatment for people with this cancer.

Of course, this is just one of many studies from this year that, in my opinion, represent bona fide advances in cancer care or that advanced our knowledge of this disease in profound and lasting ways.

Immunotherapy's ever-expanding impact

Not surprisingly, several of the notable cancer treatment advances reported this year involve immunotherapy. In my own specialty area, kidney cancer, for instance, a trial testing the immune checkpoint inhibitor pembrolizumab (Keytruda) was the first ever in which an adjuvant therapy improved overall survival in adults with the disease.

There were also several landmark Food and Drug Administration (FDA) approvals of immunotherapy drugs this year. They included two treatments for small cell lung cancer, which for decades has been impervious to every therapy scientists have thrown at it. The approvals of tarlatamab (Imdelltra) and of durvalumab (Imfinzi), both based on impressive results in clinical trials, show that's finally starting to change.

In 2024, immunotherapy also made very strong inroads against childhood cancers. For some infants and children with acute lymphoblastic leukemia (ALL), for example, blinatumomab (Blincyto)—a type of drug known as a bispecific T-cell engager, or BiTE—was already a standard treatment. But thanks to a new FDA approval and outstanding findings from an NCI-supported clinical trial, many more infants and children are likely to be cured with the help of this drug.

And following the initial results of a small clinical trial of an investigational CAR T-cell therapy, there are now legitimate hopes that an effective treatment is on the horizon for children with extremely aggressive brain cancers called diffuse midline gliomas.

Several children who received the GD2-targeted CAR T-cell therapy had responses that have lasted 2 years or more, a never-before-seen result in this cancer. NCI researchers have already launched a clinical trial testing the same CAR T-cell therapy in children with two other aggressive cancers for which effective treatments are desperately needed, neuroblastoma and osteosarcoma.

This year also marked the arrival of two new cancer cellular therapies. In February, FDA approved the first-ever tumor infiltrating lymphocyte (TIL) therapy, in this case for people with advanced melanoma. And in August, the agency approved the first-ever T-cell receptor (TCR) therapy, for the treatment of advanced synovial sarcoma.

Those approvals had to be gratifying moments for NCI's Steve Rosenberg, M.D., and his colleagues over the decades who conducted so much of the pioneering work on both TIL and TCR therapies.

A potential cachexia treatment, and exciting findings on ecDNAs and tumor atlases

This year may also represent the moment when the tide finally turned in our ability to treat the wasting syndrome called cancer cachexia, which is estimated to be the direct cause of approximately 30% of cancer deaths. Earlier this year, in a small clinical trial, treatment with an experimental drug called ponsegromab literally reversed cachexia-induced weight loss in people

with advanced cancer.

What's instructive about this clinical trial is the direct line that can be drawn between the drug tested in the study and earlier basic science studies that went through the painstaking work of identifying the important molecular players in cachexia. The development of ponsegromab, and several similar drugs, is evidence yet again of the importance of basic science and why NCI is so steadfastly committed to providing robust funding for it.

In keeping with the theme of basic research, I'm particularly excited about findings published this fall from a series of studies on extrachromosomal DNAs, or ecDNAs. The studies were conducted by an international research group funded by Cancer Grand Challenges, an initiative led by NCI and Cancer Research UK.

Until a decade ago, these tiny chunks of DNA that reside in the cell nucleus but are unattached to chromosomes were thought to be meaningless genetic junk. Over the last decade, however, it's become clear that, in tumors, that's not so. These recent studies provide the strongest evidence to date that ecDNAs are intimately involved in two of the biggest challenges we face in cancer: treatment resistance and metastasis. One even demonstrated that it may be possible to specifically kill tumors that are reliant on ecDNAs.

Then there were the 10 studies from research groups in the Human Tumor Atlas Network, a Cancer Moonshot℠ initiative to construct three-dimensional maps of human tumors. The studies were impressive not only in the diversity of their investigations but also because several involved the development of novel technologies for probing cancer's deepest recesses.

Some of the studies provided important new insights into how specific components of the immune system help cancers spread, and others reconstructed some of the key steps involved in the development of colorectal cancer from precancerous growths to full-blown metastatic disease. These are foundational studies that I have little doubt will be a springboard for many important advances in prevention and treatment.

Telehealth can improve cancer care, progress in early detection and prevention

Using advanced technologies to delve into pernicious DNA circles or create detailed maps of tumors are one end of the cancer research spectrum. On the other end are studies intended to refine and enhance patient care and improve quality of life.

And along those lines, the results from several such studies published this year all point to a single conclusion: Telehealth is having its moment in cancer care.

Using everything from tablet computers to old-fashioned phone calls, researchers showed that telehealth could help improve the management of symptoms and treatment side effects in children with cancer and ensure that patients receive needed palliative care.

Other priority areas of research include finding effective and efficient ways to identify more cancers as early as possible and, of course, to prevent cancer in the first place. In 2024, we saw progress in both areas.

For the former, there were promising results from small studies of blood tests for cancer that rely on circulating tumor DNA, including one for pancreatic cancer. And in the latter case, a study out of Scotland found no cases of cervical cancer among women who had been fully vaccinated against HPV as teenagers. Along those same lines, the evidence continued to build this year that a single dose of the HPV vaccine may offer long-lasting protection against HPV-related cancers.

Pushing forward into 2025 and beyond

There's no shortage of cancer research that is having far-reaching effects, and I'll be sharing more of my thoughts on the "greatest hits" of cancer research in 2024 late next week on NCI's social media channels.

In the meantime, I can promise that, at NCI, we are committed to doing everything in our power to support as much excellent cancer research as we can. Because we know that doing so will deliver advances and breakthroughs that will save more lives and help more people affected by cancer.

In my mind, there can be no greater mission.

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