YOUR GUIDE TO THE LATEST CANCER RESEARCH AND TREATMENTS

Highlights from the 2016 Annual Meeting of the American Society of Clinical Oncology

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How To Use This Booklet

Each year, Cancer*Care*® publishes a special edition of the Cancer*Care* Connect Booklet Series that presents research highlights from the Annual Meeting of the American Society of Clinical Oncology. The information contained in these pages is intended for discussion with your doctor. He or she can tell you whether these advances in cancer treatment affect your treatment plan and whether a clinical trial is right for you.

Some of the treatments discussed in this booklet are still in the very early stages of research and may not be available to the general public outside of a clinical trial. The advances in treatment that have come about are because of the many people who have taken part in such studies. If current drugs or other types of cancer treatment no longer benefit you, you may wish to explore joining a clinical trial. The members of your health care team will help you fully understand the possible risks and benefits involved. On page 63 you will find a list of resources, including websites where you can search for a clinical trial. If your particular type of cancer is not discussed in this booklet and you wish to take part in a study, these websites can help.

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The content of this booklet was taken from Cancer*Care's* two-part Connect Education Workshop 2016 ASCO Highlights series, during which the following leading experts presented:

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The Importance of Clinical Trials

Clinical trials are the standard by which we measure the worth of new treatments and the quality of life of patients as they receive those treatments. For this reason, doctors and researchers urge people with cancer to take part in clinical trials.

Your doctor can guide you in making a decision about whether a clinical trial is right for you. Here are a few things that you should know:

- Often, people who take part in clinical trials gain access to and benefit from new treatments.
- Before you participate in a clinical trial, you will be fully informed as to the risks and benefits of the trial.
- Most clinical trials are designed to test a new treatment against a standard treatment to find out whether the new treatment has any added benefit.
- You can stop taking part in a clinical trial at any time for any reason.

When considering participation in a clinical trial, it's important to consult with your primary care physician and your oncologist, and to make sure that all of your questions are answered.

This is a very exciting time in cancer research, and there are clinical trials underway to study newer treatment approaches, such as immunotherapy and targeted treatments. In immunotherapy, the immune system's ability to seek out and destroy cancer cells is enhanced. Targeted treatments are designed to target the specific cell mechanisms that are important for the growth and survival of tumor cells.

Brain Cancer

Researchers reported a number of important findings in brain cancer treatment at the 2016 Annual Meeting of the American Society of Clinical Oncology:

- **A first-of-its-kind immunotherapy trial is underway to test a vaccine that targets IDH mutations in gliomas** (page 10).
- **Surgical techniques are being tested for insular gliomas** (page 11).
- **"Next generation sequencing" tests are being conducted for primary brain tumors** (page 11).
- **Results were reported from a phase II clinical trial involving patients with recurrent glioblastomas** (page 12).
- **A trial showed higher 1-year and 2-year survival rates in elderly patients with glioblastomas when the chemotherapy Temodar® was added to a shortened course of radiation therapy** (page 12).
- **The combining of a PD-1 antibody with a CTLA-4 antibody in treatment of recurrent glioblastomas is being studied** (page 13).
- **A number of promising studies are in progress** (page 13).

Vaccine that targets IDH mutations

The IDH1 gene provides instructions for making an enzyme called isocitrate dehydrogenase. Mutations in this gene are common in gliomas. The NOA-16 trial is testing a peptide vaccine that targets IDH mutations in gliomas. The goal of the trial is to evaluate the

safety and tolerability of the vaccine, and to assess whether it intensifies the immune response in killing tumor cells.

What Patients Need to Know

Doctors are optimistic about the future of immunotherapies for the treatment of brain cancer. NOA-16 is the first immunotherapy clinical trial to address the IDH1 mutation in gliomas.

Surgical techniques for insular gliomas

Gliomas in the insular region of the brain (below the surface) are more difficult to safely remove than gliomas on the brain's surface. There are techniques being studied to guide surgery and more safely remove these types of gliomas, including the use of a fluorescent dye called 5-ALA, pre-operative brain-mapping, and neuronavigation (radiological guidance for neurosurgery).

What Patients Need to Know

These "assistive" techniques are designed to allow the surgeon to safely remove the insular tumor without affecting brain function. Early results are promising, and these techniques may also prove to be of benefit in removing gliomas on the surface of brain.

Sequencing for primary brain tumors

Personalized medicine—the right treatment for the right patient at the right time—was a recurrent theme during the 2016 meeting. One example pertinent to brain cancer: A study was conducted in which the mutations in a large number of primary brain tumors were extensively analyzed, toward the end of determining which mutations are likely to respond to which specific drug. This approach is called "next generation sequencing."

What Patients Need to Know

The results of the study, while informative, are at this point theoretical and will be tested in a clinical setting.

Avastin and recurrent glioblastomas

The San Diego-based pharmaceutical company Tracon reported data from its phase II trial involving patients with glioblastoma whose tumors had recurred after treatment with Avastin® (bevacizumab). In the trial, these patients were treated with the clinical-stage antibody TRC105 in combination with Avastin. TRC105 targets the process of new blood vessel formation.

This trial was co-sponsored by the National Cancer Institute.

What Patients Need to Know

While more testing is needed, the results of the trial were promising. An improvement in overall survival was observed, and the data indicates the combination of TRC105 and Avastin was well-tolerated, with no apparent increase in the frequency or severity of adverse events typically associated with each.

Temozolomide for elderly patients

A randomized phase III trial found that adding the chemotherapy Temodar® (temozolomide) during a short course of radiation therapy significantly improved survival of elderly patients with glioblastoma, if followed with monthly maintenance does of Temodar.

The 1-year and 2-year survival rates were 37.8% and 10.4% with radiation plus Temodar, as compared to 22.2% and 2.8% with radiation therapy alone.

What Patients Need to Know

Older adults account for half of all patients with glioblastomas. This is the first study to test the combination of Temodar and radiation therapy in this population. Although side effects were slightly greater in patients receiving Temodar, the overall quality of life was similar in both patient groups.

Antibody drugs and recurrent glioblastomas

Many cancer cells carry "checkpoint" proteins that prevent the immune system from attacking tumors. In an important area of research, agents are being studied that can block those proteins, thereby strengthening the body's immune response. A cohort of the CheckMate-143 study will look at the effect of combining a PD-1 antibody with a CTLA-4 antibody in treatment of recurrent glioblastomas.

What Patients Need to Know

Both the PD-1 antibody and CTLA-4 antibody are "checkpoint inhibitors." The study of this type of drug is a very hopeful area of immunotherapy research for recurrent glioblastomas.

A number of promising studies are in progress

Updates were provided on a number of studies, including:

- GDC-0084i is a small molecule inhibitor of PI3K and mTOR signaling pathways, which regulate cell survival and proliferation. A first-in-human, phase I dose escalation study of GDC-00841 was conducted in patients with high-grade glioma.
- A phase I study which tested an investigational chimeric antigen receptor (CAR) therapy made from patients' own T cells engineered to target a tumor-specific protein known as

EGFRvIII, which is found in about 30 percent of glioblastoma patients' tumors.

- Studies involving "precision medicine," defined by the National Institutes of Health (NIH), as "an emerging approach for disease treatment and prevention that takes into account individual variability in genes, environment, and lifestyle for each person."

What Patients Need to Know

Precision medicine was a theme throughout the 2016 ASCO meeting, for many types of cancer. It is hoped that in the future immunotherapy and precision medicine will be used in combination to further progress the treatment of cancer.

Breast Cancer

Researchers reported a number of important findings in breast cancer treatment at the 2016 Annual Meeting of the American Society of Clinical Oncology:

- **In post-menopausal women, extending treatment with an aromatase inhibitor to 10 years improves disease-free survival, but not overall survival.** The extension of treatment was after an initial 5 years of therapy with an aromatase inhibitor/tamoxifen (page 16).
- **Palbociclib, which improves survival for women with hormone receptor-positive breast cancer, was approved.** Patients that received palbociclib and letrozole led to a doubling of survival without the cancer progressing (page 16).
- **The cyclin-dependent kinase 4/6 (CDK-4/6) inhibitor abemaciclib is showing promise for certain patients.** The patients who seemed to benefit had a progression of their hormone receptor-positive breast cancer after endocrine treatment and chemotherapy (page 17).
- **A combination of TDM1 and the antibody pertuzumab may be effective in eliminating tumors in HER2 positive, early-stage, pre-operative breast cancer.** However, combining classic chemotherapy drugs with trastuzumab and pertuzumab resulted in a higher success rate in eliminating tumors (page 18).

Extending treatment with an aromatase inhibitor

MA.17R is a double-blind, placebo-controlled phase III trial which included 1,918 post-menopausal women who had been diagnosed with early-stage breast cancer and who had been treated with an aromatase inhibitor (some had also been treated with tamoxifen) for 5 years, without recurrence of the cancer. The participants were randomized to two groups: one receiving an additional 5 years of an aromatase inhibitor and one receiving a placebo.

The group who received an additional 5 years of an aromatase inhibitor had significantly lower rates of cancer recurrence than the group that received the placebo, but their overall survival was not higher.

What Patients Need to Know

The overall quality of life for patients receiving the additional 5 years of an aromatase inhibitor was not significantly different, but bone-related side effects (including bone pain, bone fractures, and new-onset osteoporosis) occurred more frequently.

Palbociclib for advanced, hormone receptor-positive breast cancer

The small clinical trial PALOMA-1 led to the 2015 approval of palbociclib, in combination with the chemotherapy letrozole, for the treatment of advanced, hormone receptor-positive breast cancer in postmenopausal women who have received no prior endocrine therapy.

Palbociclib is a cyclin-dependent kinase 4/6 (CDK-4/6) inhibitor and was the first drug in its class to be approved in the U.S. The larger PALOMA-2 trial included 666 women with the same

disease profile and confirmed the results of PALOMA-1: that the combination of palbociclib and letrozole led to a doubling of survival without the cancer progressing.

What Patients Need to Know

Patients in the group who received a combination of palbociclib and letrozole had a greater incidence of a reduction in the count of certain white blood cells, and a higher overall incidence of serious adverse events: 19.6% versus 12.6% for those in the "letrozole only" group.

Inhibitor abemaciclib and hormone receptor-positive breast cancer

MONARCH 1 was a phase II study of single-agent (given alone) abemaciclib, a CDK-4/6 inhibitor. The study enrolled 132 women with hormone receptor-positive breast cancer who had previously received at least one (but no more than two) chemotherapies. The results were encouraging, especially as the study participants were a heavily "pre-treated" population and more than half had cancer that had spread to three sites within the body.

Low white blood cell counts and diarrhea were the most often reported side effects.

What Patients Need to Know

While not yet approved by the FDA for any disease, preliminary clinical trials indicate that abemaciclib may offer substantial advantages over existing treatment options. As a result, the drug has been given "breakthrough" status by the FDA. If additional trials also show positive results, a priority review may be granted.

New treatment for HER2 positive pre-operative breast cancer

Breast cancers that have large amounts of a protein called HER2 on the surface of the cells can be treated with trastuzumab, which targets the HER2 protein. TDM1 is a cancer treatment that contains the chemotherapy drug DM1 and trastuzumab.
The phase III clinical trial KRISTINE showed that a combination of TDM1 and the antibody pertuzumab was effective in eliminating tumors in early-stage HER2 positive, pre-operative breast cancer in 44% of the cases. However, combining classic chemotherapy drugs (such as paclitaxel and docetaxel) to trastuzumab and pertuzumab resulted in a higher success rate (56%) in eliminating tumors.

What Patients Need to Know

While the inclusion of classic chemotherapy drugs in the treatment of early-stage HER2 positive, pre-operative breast resulted in higher success rate in eliminating tumors, it is intriguing that a significant percentage of women in the KRISTINE trial had a full elimination of their tumors without classic chemotherapy (and its associated side effects).

Colorectal Cancer

Researchers reported a number of important findings in colorectal cancer treatment at the 2016 Annual Meeting of the American Society of Clinical Oncology:

- **There are currently 11 anti-cancer agents approved for the treatment of metastatic colorectal cancer that can be used in hundreds of different combinations.** Research continues to provide safer and more effective treatment options (page 20).
- **Interim results were reported from a phase II study of the combination of the immunotherapies nivolumab and ipilimumab in patients with or without microsatellite instability (MSI)** (page 20).
- **Biological differences in tumors depending on their location within the colon may help guide optimal treatment selection for patients with metastatic colorectal cancer.** Studies will likely continue to focus on understanding the biological differences between left-side and right-side tumors (page 21).
- **Results from an early phase study showed the combination of cobimetinib and atezolizumab demonstrated encouraging clinical activity in patients with microsatellite stable colorectal cancer (MSS CRC)** (page 21).
- **The epidermal growth factor receptor (EGFR) is recognized as an important player in the initiation and progression of colorectal cancer, and EGFR therapies are being studied** (page 22).

Anti-cancer agents currently approved for treatment of metastatic colorectal cancer

Current treatments fall into the classifications of chemotherapy, targeted therapy, and immunotherapy. Treatments can consist of a single agent, or a combination of agents.

Chemotherapy remains an important part of treatment for many people with metastatic colorectal cancer, and research continues into how to make it safer and more effective. Several targeted therapies are also used to treat this type of cancer; these drugs work differently from standard chemotherapy, in that they affect specific parts of cancer cells. Immunotherapy uses the body's immune system to fight cancer; it is an exciting area of research for the treatment of metastatic colorectal cancer.

What Patients Need to Know

There is much more to learn about immunotherapy in the treatment of metastatic colorectal cancer. Studies have shown that tumors that have microsatellite instability (MSI) may be well suited to immunotherapy.

Immunotherapies nivolumab and ipilimumab studied in combination

In the phase II CheckMate-142 study, patients with microsatellite instability (MSI) metastatic colorectal cancer received nivolumab alone or with ipilimumab, and patients with microsatellite stable colorectal cancer (MSS CRC) received one of three varying dose combinations of nivolumab with ipilimumab.

What Patients Need to Know

The results of the interim analysis indicate that nivolumab alone and with ipilimumab has promising clinical activity in the MSI

group and was well-tolerated, with the more common adverse events being fatigue and diarrhea.

Location in colon affects biology of tumors

In offering therapy options, it's important to understand the biological differences in tumors originating from the left side of the colon (the descending colon, sigmoid colon, and rectum) and the right side (the cecum and ascending colon). Left-sided tumors are associated with improved survival, even those with the KRAS mutation.

What Patients Need to Know

There will likely be increased focus on the biological differences between left-side and right-side tumors going forward, as doctors seek to more deeply understand the difference in treatment options and outcomes based on the location of the tumor.

Early phase study of combination therapy in MSS CRC

Results were reported from a phase Ib study combining the PD-L1 inhibitor atezolizumab with the MEK-inhibitor cobimetinib in patients with microsatellite stable colorectal cancer (MSS CRC). The results were promising, showing that patients with MSS CRC can respond to the combination treatment.

What Patients Need to Know

The study also showed that the combination of atezolizumab and cobimetinib is well tolerated at the maximum administered doses. The results of the study indicate that continued evaluation of the combination is warranted.

The role of EGFR therapies

Most patients with colorectal cancer demonstrate high levels of the epidermal growth factor receptor (EGFR); correlating with a poor prognosis. EFGR has therefore become a key target of therapeutic strategies designed to treat metastatic colorectal cancer.

What Patients Need to Know

KRAS is a molecule responsible for signaling from EGFR to the cell nucleus. Routine testing for KRAS mutations in all patients with colorectal cancer is recommended and only those harboring wild-type (WT) KRAS should be candidates for EGFR therapies, thus improving outcomes and minimizing unnecessary toxicity.

Leukemia

Researchers reported a number of important findings in leukemia treatment at the 2016 Annual Meeting of the American Society of Clinical Oncology:

- **Targeted therapy venetoclax was found to be effective in the treatment of relapsed/refractory acute myelogenous leukemia (AML).** A phase II study has shown this to be effective, especially for older patients with AML (page 24).
- **A formulation of cytarabine and daunorubicin improved survival in patients with newly diagnosed secondary AML compared to the conventional regimen.** A phase III randomized trial showed improvement in overall survival (page 24).
- **A phase II study investigated the efficacy and safety of the WT1 peptide vaccine in prevention of AML relapse** (page 25).
- **In recent years, progress has been made in developing chimeric antigen receptor (CAR) T-cell therapy to treat relapsed and refractory acute lymphoblastic leukemia (ALL).** More ways to minimize the side effects resulting from this form of therapy are being learned through clinical trials (page 25).
- **A study found that the chemotherapy nilotinib induces treatment-free remission in patients with chronic myeloid leukemia (CML)** (page 26).
- **Promising results were reported from a study of the targeted therapy SL-401 in treatment of blastic plasmacytoid dendritic cell neoplasm (BPDCN)** (page 26).

Targeted therapy venetoclax studied for AML

A phase II study has shown the BCL-2 inhibitor venetoclax to be clinically active in patients with high-risk relapsed/refractory AML or those unable to tolerate intensive chemotherapy. BCL-2 is a gene that can prevent the death of cancer cells; it is overactive in some forms of leukemia.

The study results support evaluating venetoclax, in combination with other agents, in treating patients with AML.

What Patients Need to Know

The results were promising, especially for older patients with AML. Venetoclax was also found to be well tolerated by the study participants, all of whom had undergone previous treatment.

Formulation of cytarabine and daunorubicin improves AML survival

In older adults with newly diagnosed secondary acute myeloid leukemia (AML), a phase III randomized trial showed that CPX-351, a liposomal formulation of cytarabine and daunorubicin, significantly improved overall survival compared to a conventional "7 + 3" regimen of cytarabine and daunorubicin.

In addition to overall survival, CPX-351 significantly improved event-free survival without an increase in the frequency or severity of side effects.

What Patients Need to Know

The conclusion of the study was that CPX-351 should become the standard of care for older patients with secondary AML.

WT1 peptide vaccine studied in prevention of AML relapse

Abnormal expression of the WT1 gene occurs in certain subtypes of leukemia, including AML.

The aim of this phase II study was to investigate whether the WT1 peptide vaccine caused a safe immune response that inhibited AML relapse (kept the patients in remission).

The results suggest that the WT1 vaccine is well tolerated, stimulates a beneficial immune response, and is associated with prolonged survival.

What Patients Need to Know

A pivotal international multicenter randomized study is planned for 2016.

Engineering T-cells to treat relapsed and refractory acute lymphoblastic leukemia

Although 60 percent to 80 percent of adults with acute lymphoblastic leukemia (ALL) achieve remission following chemotherapy, about half of them relapse after the initial treatment.

The use of chimeric antigen receptor (CAR) T-cell therapy to treat relapsed and refractory ALL involves engineering the patient's own T-cells with a gene that expresses the CAR; at the end point of the process, the patient receives an infusion of the modified T-cells.

CAR T-cell therapy is usually given in a hospital setting.

What Patients Need to Know

More is being learned about this therapy, including using it in ways that minimize side effects, and its potential value as an earlier treatment approach.

Nilotinib can induce treatment-free remission in patients with chronic myeloid leukemia

According to a single-arm phase II study, frontline treatment with the chemotherapy nilotinib for at least three years led to treatment-free remission in a majority of patients with CML in chronic phase. More than half of the patients remained in tumor-free remission for 48 weeks; further, almost every patient who required additional treatment once again achieved major molecular response.

What Patients Need to Know

While further study is needed, these results suggest that patients with CML may not need to be treated indefinitely.

Targeted therapy studied in treatment of BPDCN

Blastic plasmacytoid dendritic cell neoplasm (BPCDN) is a very rare type of leukemia. SL-401 is a targeted therapy directed to the interleukin-3 receptor (CD123), a target overexpressed in BPDCN and other hematologic cancers. Results from the ongoing phase II trial were very promising; 89% of the participants had a positive clinical response.

What Patients Need to Know

Results such as reported from this study provide encouragement that targeted therapies will continue to be developed for treatment of many types of leukemia.

Lung Cancer

Researchers reported a number of important findings in lung cancer treatment at the 2016 Annual Meeting of the American Society of Clinical Oncology:

- **A phase I study showed promise for the combination of two immunotherapy drugs as a first line treatment for non-small cell lung cancer (NSCLC)** (page 28).
- **The standard of care may be changing for the subset of NSCLC patients with the anaplastic lymphoma kinase (ALK) gene** (page 28).
- **Chemotherapy is still an effective treatment option for lung cancer, and combination treatments (compared to single agent) continue to be studied** (page 29).

- **There is now the possibility of testing a tumor's DNA via a blood test (a "liquid biopsy") rather than via a physical biopsy; this represents an advance in care, as liquid biopsies are less invasive and allow for quicker results** (page 30).
- **A study showed that the chemotherapy crizotinib is effective in treating NSCLC patients with a mutation of the MET gene, which encodes tyrosine-kinase activity** (page 30).

Immunotherapy combination treatment studied for NSCLC

The phase I CheckMate 012 study was designed to evaluate the combination of the immunotherapies nivolumab and ipilimumab as a first line treatment for non-small cell lung cancer (NSCLC). The trial enrolled patients with stage IIIB/IV NSCLC who had not received prior chemotherapy. The effectiveness and tolerability of various dosages were studied, and the results showed promise.

What Patients Need to Know

The combination of nivolumab and ipilimumab is currently approved for the treatment of metastatic melanoma and has demonstrated clinical benefits across multiple tumor types.

Second-generation ALK-inhibitors being studied as a first-line treatment option

The current first line treatment standard of care for ALK-positive NSCLC is crizotinib, a first-generation ALK inhibitor that was granted an accelerated FDA approval in 2011. The randomized open-label phase III J-ALEX study, consisting of just over 200 patients, compared crizotinib to the more active second-generation ALK-inhibitor alectinib, with progression-free

survival (PFS) being the primary endpoint. The results showed a significant statistical and clinical improvement in PFS when the cancer was treated by alectinib.

What Patients Need to Know

Alectinib and ceritinib (another second-generation ALK-inhibitor) are currently FDA-approved for patients who have developed progression after being treated with crizotinib (or who are not able to tolerate it). There are other second-generation ALK-inhibitors in development; the most promising of which is brigatinib.

Combination chemotherapy as second or third line treatment shows promising results

ULTIMATE, a phase III randomized open-label study, evaluated the combination of the chemotherapies bevacizumab and paclitaxel vs. the chemotherapy docetaxel in 166 patients as a second or third line treatment of advanced non-squamous NSCLC. The study met its primary endpoint, showing significant improvement of progression-free survival with a manageable safety profile.

What Patients Need to Know

The study concluded that the combination of bevacizumab and paclitaxel should be considered as a treatment option when the first-line treatment of platinum-based chemotherapy stops working or had to be stopped because of intolerable side effects.

Liquid biopsies represent an advance in care

Circulating tumor cells (CTCs) are malignant cells that have broken off from a tumor and have entered the bloodstream. A liquid biopsy allows for the analysis of CTCs through a simple blood test, rather than a more-invasive physical biopsy. There is increasing interest in studying CTCs as a prognostic or predictive biomarker in several types of cancer, including lung cancer.

What Patients Need to Know

The analysis of CTCs has been incorporated into many phase III clinical trials. Understanding the number and characteristics of CTCs may lead to decisions about therapy, and also provide information about the basic mechanisms of tumor biology.

ALK-inhibitor crizotinib studied in NSCLC patients with mutations of the MET gene

NSCLC patients with a mutation of the MET gene (MET exon 14-altered NSCLC) were enrolled in an expansion cohort of the ongoing phase I PROFILE 1001 study and received the ALK-inhibitor crizotinib twice-daily at a starting dose of 250 mg. The results were promising; showing that crizotinib shrunk the cancer and was generally well-tolerated.

What Patients Need to Know

Crizotinib is currently approved for the treatment of ALK-positive NSCLC. Further study of crizotinib in patients with MET exon 14-altered NSCLC is warranted.

Lymphoma

Researchers reported a number of important findings in lymphoma treatment at the 2016 Annual Meeting of the American Society of Clinical Oncology:

- **Immune checkpoint inhibitors are currently being studied in clinical trials for the treatment of many forms of lymphoma** (page 32).
- **In a small study, the antibody mogamulizumab showed promising results in the treatment of Adult T-cell leukemia/lymphoma (ATLL)** (page 32).
- **Maintenance rituximab failed to improve survival after initial treatment with rituximab and bendamustine for patients with mantle cell lymphoma.** A small study showed that rituximab, given as a maintenance therapy, did not provide any additional benefit (page 33).

Immune checkpoint inhibitors being studied

Tumor cells often have immune checkpoint molecules that act as a shield, allowing the cancer to evade an attack by the immune system. This can be countered by immune checkpoint inhibitors—drugs designed to remove the shield and allow the immune system to attack the cancer cells.

Immune checkpoint inhibitors are currently being studied in clinical trials, and it is thought that they may have a role in treating some types of lymphoma.

What Patients Need to Know

The immune checkpoint inhibitor nivolumab was approved by the FDA in in May 2016 for treatment of patients with classical Hodgkin lymphoma that has relapsed or progressed after autologous hematopoietic stem cell transplantation (HSCT) and post-transplantation treatment with brentuximab vedotin.

Monoclonal antibody studied for treatment of ATLL

ATLL is a rare type of lymphoma that does not respond well to standard treatments. In a small, randomized study, the anti-CCR4 monoclonal antibody mogamulizumab demonstrated a clear benefit in the treatment of ATLL, compared to chemotherapy.

What Patients Need to Know

Antibodies are small proteins that circulate in the bloodstream; as part of the body's immune system, they bind to proteins and other chemicals in the body which they recognize to be "foreign." Mogamulizumab binds to (and inhibits) CCR4, a protein-receptor which is sometimes overexpressed in ATLL.

Maintenance therapy studied for MCL

A small, randomized study was conducted to assess whether there was benefit for patients with mantle cell lymphoma (MCL) to undergo prolonged maintenance therapy with the antibody rituximab after initial treatment with rituximab and the chemotherapy bendamustine.

Rituximab was given as maintenance therapy over a 2-year period. The results indicated that this approach appeared to have no additional benefit compared with observation alone.

What Patients Need to Know

While the results of this study were non-definitive, patients with MCL or other B-Cell lymphomas may want to talk to their doctor about whether maintenance therapy with rituximab should be considered.

Melanoma

Researchers reported a number of important findings in melanoma treatment at the 2016 Annual Meeting of the American Society of Clinical Oncology:

- **There is an ongoing phase III study comparing dabrafenib and trametinib combination therapy to dabrafenib administered with a placebo, in treatment of cutaneous melanoma** (page 34).
- **The combination treatment with nivolumab and ipilimumab improved progression-free survival for previously untreated metastatic melanoma.** Results from a phase III trial showed an improvement in the combination treatment compared with nivolumab alone and ipilimumab alone (page 35).
- **The anti-PD-1 antibody pembrolizumab provides long-term survival benefit in advanced melanoma.** Some patients in this study had undergone prior treatment, and some had not (page 36).
- **In patients with advanced melanoma, there's an ongoing study to evaluate the safety and efficacy of different dosing schedules of pembrolizumab, compared to ipilimumab** (page 36).

Trial underway to compare combination therapy to monotherapy for cutaneous melanoma

A two-arm, double-blinded, randomized phase III study is being conducted which compares the targeted therapies dabrafenib and trametinib (combination therapy) to dabrafenib administered with a placebo (monotherapy).

Participants have cutaneous melanoma that is either Stage IIIC (unresectable) or Stage IV, with a BRAF V600E/K mutation.

What Patients Need to Know

Participants will be followed for overall survival. The primary endpoint is progression-free survival for participants receiving the combination therapy compared with those receiving dabrafenib monotherapy.

Combination treatment with nivolumab and ipilimumab improved progression-free survival

In patients with previously untreated metastatic melanoma, the findings from the phase III CheckMate-067 trial showed the 18-month progression-free survival rate for the combination treatment nivolumab (targeted therapy) and ipilimumab (immunotherapy) was 46%, compared to 39% for nivolumab alone, and 14% for ipilimumab alone.

What Patients Need to Know

The progression-free survival benefit with the combination treatment compared to the single-agent nivolumab and the monotherapy ipilimumab was upheld across all patient subgroups.

Pembrolizumab provides long-term survival benefit in advanced melanoma

Data was presented on the 3-year overall survival rates for patients enrolled in the phase 1b KEYNOTE-001 study. Some patients had undergone prior treatment, and some had not. Various dosing schedules of the anti-PD-1 antibody pembrolizumab were tested as part of this study.

What Patients Need to Know

The results indicate that pembrolizumab provides long-term survival benefit in patients with advanced melanoma, with 40% of patients alive at 3 years, supporting the use of pembrolizumab in patients with advanced melanoma, regardless of prior treatment.

Phase III study compares pembrolizumab with ipilimumab

The Keynote-006 study is a multicenter, randomized, three-arm phase III study designed to evaluate the safety and efficacy of two dosing schedules of the anti-PD-1 antibody pembrolizumab compared to the immunotherapy ipilimumab. The study participants have unresectable or metastatic melanoma.

What Patients Need to Know

The results show that pembrolizumab provides benefit over ipilimumab in patients with advanced melanoma, regardless of tumor PD-L1 expression or whether patients received prior therapy.

Myelofibrosis

Researchers reported a number of important findings in myelofibrosis treatment at the 2016 Annual Meeting of the American Society of Clinical Oncology:

- **A retrospective analysis showed that low platelet count is associated with poor clinical outcomes in patients with myelofibrosis.** Low platelet count proves to be an important surrogate marker of survival in myelofibrosis (page 39).
- **The chemotherapy ruxolitinib demonstrated significant improvements in long-term outcomes for patients diagnosed with myelofibrosis** (page 39).
- **A phase III trial is planned to study ruxolitinib in the treatment of early-stage myelofibrosis** (page 40).
- **A report was given on the long-term safety and efficacy results of the targeted therapy pacritinib in patients with myelofibrosis** (page 40).

Low platelet count associated with poor clinical outcomes in myelofibrosis

Thrombocytopenia (platelets < 100x109/L) is a negative prognostic factor for overall survival in patients with myelofibrosis. An evaluation was conducted of the clinical characteristics and outcome of patients with very low platelets (below 50x109/L) who were treated at MD Anderson Cancer Center between 1984 and 2013.

What Patients Need to Know

The evaluation confirmed the significance of low platelet count as an important surrogate marker of survival in myelofibrosis and concluded that these patients require novel (new) therapy approaches.

Ruxolitinib improves long-term outcomes in myelofibrosis

Long-term results from a study referred to as the COMFORT 1 trial demonstrated that the chemotherapy ruxolitinib provides significant improvements in long-term outcomes among patients with myelofibrosis.

The positive clinical responses were durable, and continued to be seen 5 years following initiation of therapy.

What Patients Need to Know

There is continued evaluation of the optimal use of ruxolitinib in patients with myelofibrosis.

Ruxolitinib to be studied in treatment of early-stage, high mutation myelofibrosis

ReTHINK is a randomized, double-blind, placebo-controlled, multicenter phase III study of the chemotherapy ruxolitinib in early stage myelofibrosis patients who have high molecular risk mutations.

Recent evidence has suggested that patients with high molecular risk mutations experience a more aggressive form of the disease, and that this subset of patients might be candidates for earlier intervention.

What Patients Need to Know

Currently, most patients without overt symptoms are managed through a "watch and wait" strategy, with treatment occurring only when signs of the disease are present.

Updated results from PERSIST-1 trial reported

In treatment of myelofibrosis, the phase III PERSIST-1 trial evaluated the long-term safety and efficacy results of the targeted therapy pacritinib versus the best available therapy.

It was previously reported that the PERSIST-1 trial met its primary endpoint, showing a statistically significant reduction in spleen volume in treatment with pacritinib when compared to patients receiving JAK2 inhibitors.

What Patients Need to Know

Patients in the best available therapy arm of the trial that "crossed over" to receive pacritinib treatment had a similar rate of gastrointestinal events as patients initially randomized to the best available therapy or to pacritinib.

Oral, Neck, and Head Cancers

Researchers reported a number of important findings in oral, neck, and head cancer treatment at the 2016 Annual Meeting of the American Society of Clinical Oncology:

- **"Pseudoprogression" (tumor growth from the effect of treatment) can occur with immunotherapy** (page 41).
- **Hormone deprivation therapy is being investigated for salivary gland tumors** (page 42).
- **A phase II trial is studying a treatment for metastatic adenoid cystic carcinoma** (page 42).
- **A third drug for medullary thyroid cancer was the subject of a study in China** (page 43).

Pseudoprogression can occur with immunotherapy

"Pseudoprogression" (PP) is tumor growth from the effect of the treatment rather than true disease progression. It can sometimes be hard for doctors to distinguish between the two. PP has been described with immune checkpoint inhibitors.

What Patients Need to Know

Immunotherapy is the most cutting-edge area of research for the treatment of head and neck cancers. Although psuedoprogression can occur, the effects of immunotherapy are thought to be long-lasting, and patients may want to talk to their oncologist about the possibility of enrolling in a clinical trial.

Hormone deprivation therapy and salivary gland carcinomas

Due to their rarity and their resistance to therapy, no standard of care currently exists for salivary gland carcinomas. There has been an increase in the number of studies in recent years, with some promising treatment approaches on the horizon, including hormone deprivation therapy.

What Patients Need to Know

Androgen deprivation therapy (ADT), a form of hormone deprivation therapy, is a standard of care for prostate cancer. As such, it is well understood—doctors know how to give it, what results to expect, and what the side effects are. Researchers are encouraged that ADT may prove beneficial for salivary gland carcinomas.

Regorafenib and metastatic adenoid cystic carcinoma

Adenoid cystic carcinoma (ACC) is a slow-growing cancer that is usually treated by "watchful waiting." ACC can stay in the body for many years with no ill effect. However, it sometimes metastasizes; in those cases, there is currently no effective treatment.

What Patients Need to Know

Receptor tyrosine kinases (RTKs) are thought to be activated in ACC. Regorafenib is a tyrosine kinase inhibitor (TKI) that targets RTKs in metastatic ACC.

A phase II trial showed that regorafenib may result in disease control for a subset of ACC patients. Final study information is pending.

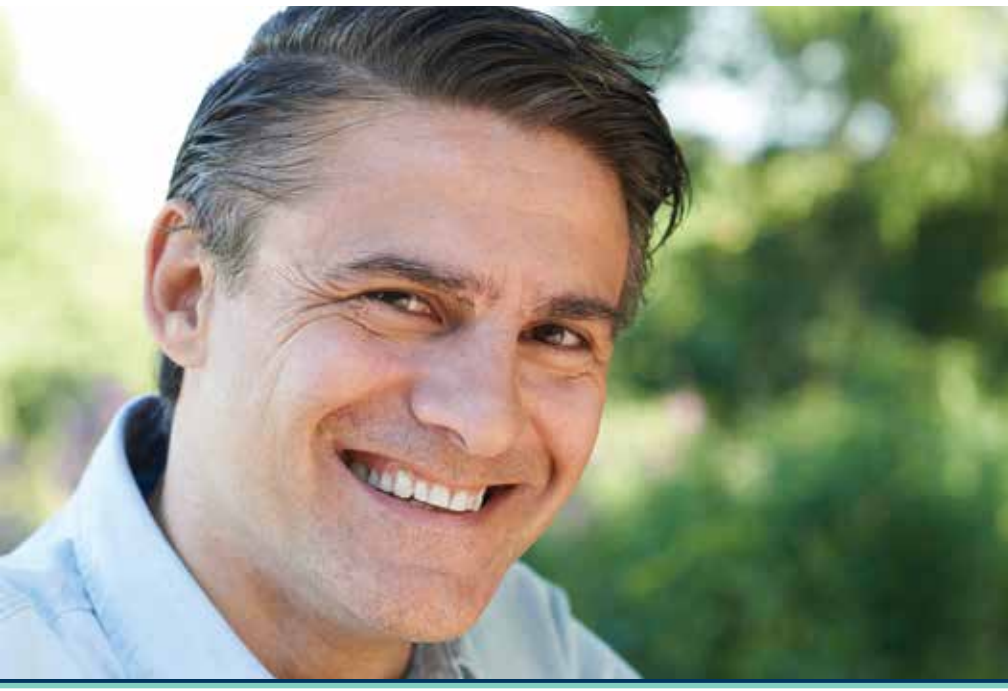
Anlotinib and medullary thyroid cancer

Medullary thyroid cancer begins in thyroid cells called C cells that produce the hormone calcitonin. There are currently two kinase inhibitors approved by the FDA for medullary thyroid cancer—vandetanib and cabozantinib—which target the vascular endothelial growth factor receptor (VEGFR).

A third drug called anlotinib, also a kinase inhibitor, has been evaluated in a phase II study conducted in China. Anlotinib is taken in pill form.

What Patients Need to Know

The study showed a good response rate compared to historical controls, and while most study participants experienced some side effects, the toxicity profile was manageable. At this point, it is unknown if or when anlotinib will be available outside of China.



Ovarian Cancer

Researchers reported a number of important findings in ovarian cancer treatment at the 2016 Annual Meeting of the American Society of Clinical Oncology:

- **Ovarian cancer is not just one disease.** There is a growing focus on personalized precision medicine—treatment approaches should vary based on the sub-type of the cancer (page 44).
- **Early studies show that immunotherapy may be useful in the treatment of ovarian cancer** (page 45).
- **Ongoing studies are investigating ways to treat platinum resistant ovarian cancer.** One drug, fosbretabulin (CA4P), has been approved for FDA "fast track" approval status (page 46).
- **Eleven genetic mutations that increase the risk of ovarian cancer have been newly-identified** (page 47).
- **PARP inhibitors may be effective as a second-line maintenance strategy in ovarian cancer** (page 48).

The growing focus on personalized precision medicine

It is becoming increasingly understood that ovarian cancer is not just one disease, and that treatment approaches should vary depending on the sub-type of the cancer. More than ever, the focus is on personalized precision medicine: finding the right drug for the right patient at the right time.

As a result, changing the course of treatment if necessary is becoming more common. Monitoring a woman's response to therapy is simpler than it was in the past—a blood test or a sampling of cells from the mouth can determine if the cancer is or is not responding to treatment. If it's not responding, a change in the treatment approach can take place.

What Patients Need to Know

From the focus on personalized precision medicine and the ongoing monitoring of how ovarian cancer is responding to treatment, doctors are learning more about what makes certain women "exceptional responders" to certain types of treatment; this can help determine the best therapy for other women who have a similar type of ovarian cancer and share a similar patient profile.

Immunotherapy and ovarian cancer

Immunotherapy is treatment that uses parts of the immune system to fight illnesses such as cancer. Immunotherapies being tested for ovarian cancer fall into six categories: monoclonal antibodies, checkpoint inhibitors and immune modulators, therapeutic vaccines, adoptive T-cell transfer, oncolytic viruses, and adjuvant immunotherapies.

What Patients Need to Know

The testing of immunotherapy as a treatment for ovarian cancer is in its early stages, but immunotherapy has been successfully used to treat other forms of cancer, and early studies show that they may prove useful in the treatment of ovarian cancer.

Fighting platinum-resistant ovarian cancer

When tumors stop responding to platinum-based therapy such as cisplatin and carboplatin, the ovarian cancer is said to be "platinum resistant." Ongoing studies are investigating ways to treat platinum resistant ovarian cancer.

Fosbretabulin (CA4P), a vascular disrupting agent, has been approved for "fast track" status in platinum resistant ovarian cancer. Data from a randomized controlled trial showed the addition of CA4P to bevacizumab improved response rates and progression-free survival in women with recurrent ovarian cancer, particularly among those with platinum-resistant disease. (Bevacizumab works by preventing the growth of new blood vessels that feed tumors.)

In an early-phase trial, napabucasin (BBI-608, BB608), a "stemness" inhibitor, showed antitumor activity in heavily pre-treated patients with platinum-resistant ovarian cancer. Napabucasin targets the STAT3 pathway, one of the processes that the cancer cells use to resist treatment. It was found to be well-tolerated when combined with the chemotherapy drug paclitaxel.

What Patients Need to Know

It is not unusual for ovarian cancer to become platinum resistant, and finding ways to treat platinum resistant ovarian cancer is the focus of much investigation and study. Research is centered both on finding new treatment agents and on finding ways to once more make the cancer sensitive to platinum-based therapy.

Additional genetic mutations associated with ovarian cancer identified

The mutations commonly associated with ovarian cancer are related to BRCA1/2 or Lynch syndrome, but a study of nearly 100,000 women identified 11 other genetic mutations that increase the risk of ovarian cancer.

The study found that 14% of women had at least one significant genetic mutation, and nearly one-third of the mutations were found in these other 11 genes.

A significant association with ovarian cancer history in the study participants was found for the following 11 genes: ATM, BRCA1, BRCA2, BRIP1, MLH1, MSH2, MSH6, NBN, STK11, RAD51C, and RAD51D.

What Patients Need to Know

If confirmed in other studies, these newly identified genes will be important to consider in genetic counseling and in evaluating patients with ovarian cancer.

PARP inhibitors and ovarian cancer

PARP is a type of enzyme that helps repair DNA. In cancer treatment, PARP inhibitors are used to prevent cancer cells from repairing their damaged DNA; this prevention can cause the cancer cells to die.

A randomized phase II double-blind, placebo-controlled trial looked at the PARP inhibitor olaparib as a second-line maintenance strategy in ovarian cancer. The study demonstrated an overall survival advantage associated with the use of olaparib in this setting, particularly in the patient population that had a BRCA mutation.

What Patients Need to Know

PARP inhibitors are a type of targeted therapy. Targeted therapies are designed to target the specific cell mechanisms that are important for the growth and survival of cancer cells. In addition to PARP inhibitors, other targeted therapies being studied include pazopanib, vintafolide, and niriparib.

Pancreatic Cancer

Researchers reported a number of important findings in pancreatic cancer treatment at the 2016 Annual Meeting of the American Society of Clinical Oncology:

- **Adding the oral drug capecitabine to the chemotherapy gemcitabine prolongs post-operative survival without increased toxicity.** This result was shown in a randomized European phase III trial, with 732 participants (page 49).
- **A study showed that adding a FDG PET/CT scan to the standard diagnostic workup in patients with suspected pancreatic cancer could be beneficial** (page 50).
- **Targeted PARP inhibitor rucaparib showed promise in previously treated patients who have the BRCA mutation.** Patients received the targeted PARP inhibitor rucaparib in a phase II clinical study (page 50).
- **Studies are continuing on how to break down stroma, the connective tissue that prevents anticancer drugs from reaching and destroying cancer cells.** These studies show potential in extending patient survival (page 51).

Adding capecitabine to gemcitabine in adjuvant therapy

Treatment with the chemotherapy gemcitabine is the current standard of adjuvant (post-operative) therapy after surgical removal of pancreatic cancer. In the ESPAC-4 trial, 732 patients whose-surgery had taken place in the last 12 weeks were randomly assigned to receive either gemcitabine alone or gemcitabine with capecitabine; the treatment lasted for 24 weeks.

The median overall survival was 28 months with the combination regimen vs. 25.5 months with gemcitabine alone. The estimated 5-year survival rates were 28.8% (combination regimen) vs. 16.3% (gemcitabine alone).

What Patients Need to Know

There were no significant differences in the types and severity of side effects between the two groups. Severe diarrhea and fatigue were slightly more common with the combination regimen. Quality of life was comparable between the two groups.

The role of FDG PET/CT scans in diagnosis and staging

The standard tool for diagnosing pancreatic cancer is a multi-detector CT (MDCT). A study from the United Kingdom showed that adding a PET/CT scan with the radioactive tracer fluorodeoxyglucose (FDG PET/CT scan) provided significant incremental benefit in the diagnosis and staging of pancreatic cancer.

What Patients Need to Know

The diagnosis and staging of pancreatic cancer can be challenging. Adding an FDG PET/CT scan to the process may not be right for every patient, but it is an option that should be considered.

PARP inhibitor shows promise in patients with BRCA mutations

In a phase II study, patients with locally advanced or metastatic pancreatic cancer who had previously been treated with chemotherapy received the targeted PARP inhibitor rucaparib. The results showed that rucaparib was a promising treatment option for patients who have the BRCA1/BRCA2 gene mutation.

All patients who responded positively received only one prior course of chemotherapy therapy, suggesting that rucaparib may be an option earlier in the course of treatment.

What Patients Need to Know

About 9 percent of patients with pancreatic cancer have BRCA1/BRCA2 gene mutations. The results further demonstrate the clinical significance of the BRCA cancer genes, not just for breast and ovarian cancers.

Drugs that target the stroma being studied

Cancer cells can hide behind cellular stroma, made up of hyaluronan (a polysaccharide) and several types of collagen. Studies have shown that drugs designed to strip away the stroma could pave the way for other drugs to reach the cancerous cells within the tumor. Targeting the stroma could potentially extend patient survival even among those with metastatic cancer.

What Patients Need to Know

FG-3019 is an investigational anti-fibrotic antibody that targets the stroma. In patients with locally advanced pancreatic cancer, a phase II randomized study of FG-3019 used in combination with the chemotherapies gemcitabine and nab-paclitaxel showed encouraging results compared to those chemotherapy agents alone.

Prostate Cancer

Researchers reported a number of important findings in prostate cancer treatment at the 2016 Annual Meeting of the American Society of Clinical Oncology:

- **The chemotherapy cabazitaxel failed to provide an overall survival or progression-free survival advantage over the chemotherapy docetaxel.** Patients studied had metastatic castration-resistant prostate cancer (mCRPC) (page 52).
- **A 20-mg/m2dose of cabazitaxel was as effective as the 25-mg/m2 dose.** It was also shown to be less toxic (page 54).
- **Liquid biopsies are being incorporated into many phase III trials.** This is as a means to better understand malignant cells that have broken off from tumors (page 54).
- **There is ongoing study on the use of radioactive tracers in the diagnosis, staging, and treatment of prostate cancer** (page 55).

Cabazitaxel did not demonstrate superiority to docetaxel

Docetaxel is the current standard of care for men with metastatic castration-resistant prostate cancer (mCRPC). The purpose of the three-arm phase III FIRSTANA study was to determine if cabazitaxel had results equal to docetaxel for men in this group.

The 1,168 person study showed that cabazitaxel as a first line treatment did not demonstrate superiority to docetaxel for either overall survival or progression-free survival in patients

with mCRPC. While there was less reported neuropathy with cabazitaxel than with docetaxel, the data showed no statistically significant side-effect differences.

What Patients Need to Know

Docetaxel remains the front-line chemotherapy of choice for men with mCRPC, and cabazitaxel remains the second-line chemotherapy of choice (after treatment with docetaxel).

New standard dose for cabazitaxel

The phase III multi-national trial PROSELICA included 1,200 mCRPC patients previously treated with docetaxel. The men were randomly assigned to receive one of the two doses (25-mg/m2 or 20-mg/m2) in the same every-three-week schedule. There was no significant advantage found for the higher dose for median overall survival or progression-free survival.

What Patients Need to Know

The results of PROSELICA showed that in addition to being clinically "non-inferior" to the higher dose, the lower dose had a better safety profile, with a lower risk of more serious adverse events.

Liquid biopsies

Circulating tumor cells (CTCs) are malignant cells that have broken off from a tumor and have entered the bloodstream. A liquid biopsy allows for the analysis of CTCs through a simple blood test.

There is increasing interest in studying CTCs as a prognostic or predictive biomarker in several types of cancer, including prostate cancer. The analysis of CTCs has been incorporated into many phase III clinical trials. Understanding the number and characteristics of CTCs may lead to decisions about therapy, and also provide information about the basic mechanisms of tumor biology.

What Patients Need to Know

Changes in CTC counts can provide information about the cancer's growth and invasiveness, and also indicate if the cancer is responding to treatment. This may lead to more personalized treatment for the patient.

Radioactive tracers in diagnosis and treatment

A radioactive tracer is a molecule labeled with radioactive isotopes that can be sent through the body for diagnostic, staging, or treatment purposes.

One example used in prostate cancer is PSMA-617, a small molecule that binds to PSMA (prostate-specific membrane antigen) and can be labeled with various radioactive tracers for diagnostic imaging and therapy. It can bind to prostate tumor cells regardless of their location within the body.

Other types of radioactive tracers are currently being studied. Researchers want to know which molecules are the most sensitive and durable, and therefore can best detect the presence of prostate cancer.

What Patients Need to Know

The ongoing study of radioactive tracers shows much potential promise in understanding how prostate cancer changes biologically and how it responds to treatment.

Sarcoma

Researchers reported a number of important findings in the treatment of sarcoma (cancers of the body's connective tissue) at the 2016 Annual Meeting of the American Society of Clinical Oncology:

- **The TKI regorafenib may improve progression-free survival for patients with certain types of metastatic sarcoma** (page 56).
- **Combining gemcitabine and pazopanib appears to improve progression-free survival in patients with some types of sarcoma** (page 57).
- **A phase II trial showed promising results for the immunotherapy pembrolizumab for certain sarcoma subtypes** (page 57).
- **A phase II study showed that the immunotherapy nivolumab was not effective in the treatment of metastatic leiomyosarcoma of the uterus** (page 58).
- **A phase I/II trial of the TKI crenolanib showed promising results in the treatment of GISTs with the D842V mutation** (page 58).

Regorafenib for some types of sarcoma

The results of a phase II, double blind, randomized trial showed that the tyrosine kinase inhibitor (TKI) regorafenib may improve progression-free survival for patients with certain subtypes of metastatic sarcoma.

Researchers enrolled 181 patients with metastatic sarcoma who had received at least three prior treatments. Sarcomas were grouped by type, which included liposarcoma, leiomyosarcoma, synovial sarcoma, and other types of soft-tissue sarcoma.

What Patients Need to Know

Except for patients with liposarcoma, the study showed an improvement in average progression-free survival for those participants who received regorafenib, compared to those who took a placebo.

The combination-therapy of gemcitabine and pazopanib studied

Results of the phase II trial PAPAGEMO showed that the combination of chemotherapy treatments gemcitabine and pazopanib appears to be a viable treatment option for patients with some types of soft-tissue sarcomas. Improvement in progression-free survival was seen mostly in patients with liposarcoma and, to a lesser extent, leiomyosarcoma.

What Patients Need to Know

In the trial, one group was given only pazopanib, with the other group being given the combination-therapy of pazopanib and gemcitabine. The results indicated that both drugs have activity in the treatment of certain types of sarcomas, but more side effects were seen in the group given the combination therapy.

Pembrolizumab for certain sarcoma subtypes

Pembrolizumab is a type of immunotherapy called a PD-1 checkpoint inhibitor. Researchers reported encouraging results from a phase II investigator-led study of pembrolizumab for certain subtypes of sarcoma. Thirty-three percent of patients

with undifferentiated pleomorphic sarcoma and dedifferentiated liposarcoma treated on this trial had a reduction in tumor size.

Patients in this trial allowed additional tumor tissue to be obtained, which means there may be an opportunity to better understand the potential benefit of this therapy in sarcoma.

What Patients Need to Know

Pembrolizumab is approved by the FDA for other types of cancer, which adds to the promising nature of the study. In this study, pembrolizumab was generally well tolerated.

The study was sponsored by the Sarcoma Alliance for Research through Collaboration (SARC).

Nivolumab and metastatic leiomyosarcoma

Results were reported from a phase II study of the immunotherapy nivolumab in treatment of metastatic leiomyosarcoma of the uterus. The results indicated that nivolumab was not effective in treating this specific type of cancer.

What Patients Need to Know

Nivolumab is approved by the FDA for other types of cancer. Although the results were disappointing, this study is still important, as it provides researchers with additional information on the use of immunotherapy in battling various types of cancer.

GISTs with D842V mutation

Gastrointestinal stromal tumors (GISTs) are usually treated with tyrosine kinase inhibitors (TKIs). However, patients with the D842V mutation don't benefit from currently available TKIs. A phase I/II trial of the TKI crenolanib showed promising early results in the treatment of GISTs.

In related news, BLU-285, an oral investigational drug, is being studied for safety and clinical activity in patients with metastatic and treatment-resistant GIST. BLU-285 inhibits the D842V and KIT Exon 17 mutations; these mutations play a key role in GIST.

What Patients Need to Know

Further studies are needed, but studies such as these are very encouraging, as they help in the development of drugs for specific mutations, leading to more personalized treatment approaches.

Notes

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Resources

Cancer*Care*®
800-813-HOPE (800-813-4673)
www.cancercare.org

American Cancer Society
800-227-2345
www.cancer.org

Cancer.Net
888-651-3038
www.cancer.net

Cancer Support Community
888-793-9355
www.cancersupportcommunity.org

National Cancer Institute
800-422-6237
www.cancer.gov

National Comprehensive Cancer Network
215-690-0300
www.nccn.org

National Library of Medicine
888-346-3656
www.nlm.nih.gov

CLINICAL TRIALS WEBSITES

Coalition of Cancer Cooperative Groups
www.cancertrialshelp.org

EmergingMed
www.emergingmed.com

National Cancer Institute
www.cancer.gov/clinicaltrials

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