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Latest News in Blood Cancer Research

Highlights From the 2013 Annual Meeting of the American Society of Hematology

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This special edition of the Cancer*Care* Connect Booklet Series highlights cutting-edge research presented at the 2013 Annual Meeting of the American Society of Hematology, which took place December 7–10 in Atlanta, Georgia.

Some of the treatments discussed are still in the very early stages of research and may not be available to the general public outside of a clinical trial.

The information contained in this booklet is intended for discussion with your doctor. He or she can let you know whether these advances in the treatment of blood cancers affect your treatment plan and whether a clinical trial is right for you.

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Latest News in Blood Cancer Research

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Lymphoma

Rituximab maintenance treatment after initial chemotherapy helped patients with follicular lymphoma survive longer without their cancer returning. These clinical trial results are encouraging, and researchers plan to study this type of maintenance therapy further to see which patients benefit the most *(page 2)*.

In a small clinical trial, adding lenalidomide to standard chemotherapy with rituximab helped many people with diffuse large B-cell lymphoma (DLBCL) survive longer without their cancer returning. Those who had a certain type of DLBCL—germinal center DLBCL—seemed to benefit most from the new treatment *(page 4)*.

Early studies showed that brentuximab vedotin is likely to be effective in treating people who have DLBCL or other B-cell lymphomas, even when these cancers no longer respond to other treatments. This medication works by helping the body's immune system destroy cancer cells *(page 4)*.

Rituximab Maintenance Treatment for Follicular Lymphoma

Rituximab (Rituxan) maintenance treatment after initial chemotherapy helped patients with follicular lymphoma survive longer without their cancer returning. In a clinical trial known as the PRIMA study, researchers looked at about 1,000 people with follicular lymphoma whose cancer had responded to initial chemotherapy. Most of them (75 percent) had received rituximab and CHOP chemotherapy (cyclophosphamide, doxorubicin, vincristine and prednisone). Half of these patients were then given rituximab maintenance treatment once every three months for about two years, and the others were not.

Six years later, the people who received rituximab maintenance treatment had survived longer without their cancer returning than those who did not receive the maintenance treatment (59 percent versus 43 percent). At this time, nearly 90 percent of both groups of patients were still surviving.

What Patients Need to Know

The PRIMA study is one of the first large clinical trials to look at the merits of giving rituximab as maintenance therapy for patients with follicular lymphoma after their cancer responded to prior chemotherapy. Researchers plan to study rituximab maintenance treatment further to find out which patients might benefit most. After initial treatment, maintenance therapy may be given for a longer time (months and even years) to prevent the cancer from returning. People who have been treated for follicular lymphoma should ask their doctor whether rituximab maintenance treatment might be right for them.

Adding Lenalidomide to Chemotherapy for DLBCL

Researchers have shown that adding lenalidomide (Revlimid) to standard chemotherapy with rituximab helped many people with DLBCL survive longer without their cancer returning. Those who had what doctors call germinal and non-germinal center DLBCL seemed to benefit from the new treatment.

A small Italian study looked at the benefit of adding lenalidomide to standard CHOP chemotherapy plus rituximab (R-CHOP) in nearly 50 older adults with DLBCL (average age was 69 years). This was the first treatment these people had received for their lymphoma. Two years after treatment, more than 90 percent of the patients had survived. About 85 percent of those taking part in the clinical trial had a complete remission—no signs or symptoms of cancer. The 16 people with germinal center DLBCL seemed to benefit most.

What Patients Need to Know

Lenalidomide is an effective treatment for many different types of lymphoma, multiple myeloma, and other blood disorders. Researchers are encouraged by these results and plan to compare adding lenalidomide to R-CHOP with R-CHOP alone in a larger clinical trial. They are especially interested in confirming the benefits of lenalidomide plus R-CHOP in those who have non-germinal center DLBCL, a cancer that is more challenging to treat.

Brentuximab Vedotin for Resistant DLBCL

Early studies showed that brentuximab vedotin (Adcetris) is likely to be effective in treating people who have DLBCL or other B-cell lymphomas, even when these cancers no longer respond to other treatments. Brentuximab vedotin has been

approved by the U.S. Food and Drug Administration for the treatment of T-cell lymphomas that no longer respond to other treatments.

More than 60 patients were given brentuximab vedotin every three weeks intravenously (through a vein). Many of their cancers (65 percent) had no longer responded to treatment, and some of the cancers (nearly 25 percent) had never responded to any prior treatment. Of these patients, 44 had DLBCL, and 18 had other types of B-cell tumors.

Of the 43 people with DLBCL who have been evaluated so far, cancer in 40 percent has responded to treatment. There was a complete remission of the cancer in seven of these patients. For people with other types of B-cell tumors, 22 percent of the cancers responded to treatment.

What Patients Need to Know

Not only is brentuximab vedotin effective in treating many people with T-cell lymphomas, it now seems to benefit some people with B-cell lymphomas such as DLBCL that have continued to grow with other treatments, including rituximab. The reason may be that this drug targets a protein on the surface of cancer cells called CD30, which has been found on both T-cell and B-cell lymphomas. Brentuximab vedotin works by helping the body's immune system seek out and destroy the CD30 protein on cancer cells.

Leukemia

The new drug obinutuzumab combined with the standard medication chlorambucil seems to be an effective treatment for people who have chronic lymphocytic leukemia (CLL) that has not yet been treated. These encouraging results with obinutuzumab were among the most exciting developments reported at the meeting *(page 7)*.

The combination of idelalisib and rituximab seems to be more effective in treating people with CLL than rituximab alone. This new treatment may also help people with CLL survive longer *(page 8)*.

Researchers in France believe that some people with chronic myelogenous leukemia (CML) whose cancer responds well to imatinib and remains in remission for at least two years

may be able to safely stop taking the drug. More studies are needed to help doctors know which patients would have the most success in stopping treatment and whether re-treatment, if the CML returns, is effective for the long term *(page 9).*

A large clinical trial confirmed that people with resistant CML and a type of acute lymphoblastic leukemia (ALL) may benefit from treatment with ponatinib. This newer, more powerful targeted leukemia drug may improve outcomes for patients with CML or Philadelphia chromosome-positive ALL that has stopped responding or returned after treatments with other medications *(page 11).*

A treatment that uses a person's own T cells to fight resistant B-cell leukemias such as ALL has shown promising early results. After treatment, there were no signs or symptoms of cancer in the blood of 10 of 12 patients evaluated; their cancers responded very quickly, within seven to 14 days after treatment *(page 12).*

Obinutuzumab and Chlorambucil for CLL

The new drug obinutuzumab (Gazyva) combined with the standard medication chlorambucil (Leukeran) seems to be an effective treatment for people who have CLL that has not yet been treated. More than 600 people with CLL took part in a clinical trial in which half of the patients were given obinutuzumab plus chlorambucil, and the others were given rituximab (Rituxan) plus chlorambucil.

After 18 months, the CLL in more patients responded to obinutuzumab plus chlorambucil than to rituximab plus chlorambucil (78 percent versus 65 percent). About 20 percent of those who received obinutuzumab and chlorambucil had no signs or symptoms of cancer, compared

with seven percent of those who received rituximab and chlorambucil. It also appears that obinutuzumab may help patients survive longer.

What Patients Need to Know

These encouraging results with obinutuzumab were among the most exciting developments reported at ASH 2013. Rituximab has been an effective drug, helping many people survive with B-cell lymphoma. Now it seems that newer medications like obinutuzumab may be even more effective when given with chlorambucil, which is used more commonly in Europe than in the United States. In November 2013, the U.S. Food and Drug Administration (FDA) approved this combination for people who have not yet received treatment for their CLL.

Idelalisib and Rituximab for Resistant CLL

The combination of idelalisib and rituximab seems to be more effective in treating people with CLL than rituximab alone, according to the results of a recent clinical trial. This new treatment may also help people with CLL survive longer.

The more than 200 people with CLL who took part in this study had other medical conditions, which made it more challenging for them to tolerate chemotherapy. Some of these patients were given idelalisib plus rituximab, and the others were given rituximab and a placebo (a look-alike substance with no active ingredient).

The cancer in many more people who received idelalisib and rituximab responded to treatment than did the cancer in those who received rituximab and placebo (81 percent versus 13 percent). After six months of treatment, the leukemia continued to respond or remain unchanged in 93 percent

of those given idelalisib plus rituximab, compared with 46 percent of those given rituximab with a placebo. Twelve months after treatment, 92 percent of those who received idelalisib and rituximab had survived, compared with 80 percent of those in the other group.

Idelalisib belongs to a new class of medications called PI3K inhibitors. These drugs block the PI3K pathway, which plays an important role in the growth of different types of lymphoma cells, especially B-cell lymphomas and CLL. (A pathway is a series of chemical events that can lead to cell growth or other changes.)

What Patients Need to Know

There are fewer treatment options for people with CLL who have other medical conditions, because chemotherapy side effects pose a particular health risk for them. So, researchers are encouraged by promising results with idelalisib and rituximab; these medications improve outcomes for these patients and are well tolerated. Other studies have shown that idelalisib may be an effective treatment for resistant non-Hodgkin lymphoma as well. Researchers predict that in the future, targeted treatments like idelalisib could change the way lymphoma and CLL are treated. (To destroy cancer cells, targeted treatments focus on specific cell mechanisms thought to be important for the growth and survival of tumor cells. Unlike chemotherapy, targeted treatments are designed to spare healthy tissues and tend to cause less severe side effects.)

Stopping Imatinib Treatment for CML

Based on the results of two clinical trials, researchers in France believe that some people with CML whose cancer responds well to imatinib (Gleevec) and remains in remission

for at least two years may be able to safely stop taking imatinib. (Remission is a period during which the signs and symptoms of cancer are reduced or disappear.) These patients' remissions are what doctors call a sustained deep molecular response. They may have a good chance for ongoing remission and long-term survival, without the need for further treatment.

In the first clinical trial, nearly 125 patients with CML who had a sustained deep molecular response to imatinib for more than two years stopped treatment. One year later, the cancer had not returned in 76 of these people. Researchers also found that CML in all patients responded to restarting treatment with imatinib or another drug in its class, either nilotinib (Tasigna) or dasatinib (Sprycel).

In the second clinical trial, 80 patients with CML who had no measurable cancer cells for at least two years stopped imatinib treatment. When researchers evaluated these people more than two years later, there was no significant return of the cancer or loss of deep remission in about two-thirds of the patients. Those whose cancer did return benefited from restarting imatinib treatment.

What Patients Need to Know

Imatinib, the first targeted treatment for blood cancer, and the related drugs that followed it dramatically changed the outlook for people with CML. Many of the first patients to take imatinib have remained in remission for more than 10 years.

Researchers were encouraged to see that a number of the people whose CML responded well to imatinib and stayed in remission for several years could safely stop treatment. They also learned that restarting imatinib after a period of discontinuing treatment was still beneficial for patients with

CML whose disease returned. Although these study results suggest that in the future some people may be able to stop treatment after a period of time, more studies are needed to help doctors know which patients would have the most success in stopping treatment and whether re-treatment, if the CML returns, is effective for the long term.

Ponatinib for Resistant CML and ALL

A large clinical trial known as PACE confirmed that people with resistant CML and a type of ALL called Philadelphia chromosome-positive (Ph+) ALL may benefit from treatment with ponatinib (Iclusig). This newer and more powerful targeted leukemia drug may improve outcomes for patients with CML or Ph+ ALL that has stopped responding to treatment or returned after the use of several medications.

In the PACE trial, over half of the more than 200 patients who could not tolerate other treatments or whose CML had become resistant to them benefited from ponatinib. The

improvement was seen not only in the blood of these patients, but also in the bone marrow, where red blood cells and many white blood cells originate. Overall, the leukemia did not return or grow in 80 percent of patients in the clinical trial; 94 percent of them were still surviving one year after treatment with ponatinib.

Resistant CML and Ph+ ALL often have a BCR-ABL/T315I gene mutation (change), which had remained a challenge to treat. But these types of cancer (particularly those in the chronic or longer term phase) appear to respond well to ponatinib.

What Patients Need to Know

In late 2012, the FDA approved ponatinib as a treatment for people with resistant CML or Ph+ ALL. Then the FDA temporarily withdrew its approval of ponatinib to review information about certain side effects of the drug (mainly blood vessel complications). However, in late 2013, ponatinib returned to the market with a modified label that included guidance for doctors about the side effects of ponatinib and details on who might be best suited for the drug. With these encouraging results from the PACE trial, ponatinib still appears to be an effective way to treat some patients with resistant CML or Ph+ ALL, provided doctors use it as directed and monitor patients closely for side effects.

CAR T-Cell Therapy for Resistant B-cell ALL

A treatment that uses a person's own T cells to fight resistant B-cell leukemias such as ALL has shown promising early results. T cells belong to a group of white blood cells known as lymphocytes, which help the body's immune system fight infections and diseases such as cancer.

In a small clinical trial, 13 patients with resistant ALL were first given chemotherapy. Then they were treated with "CAR" T cells. This treatment involved a few steps. First, T cells were taken from the blood of the patients with ALL. Next, the T cells were "reprogrammed" to recognize and destroy the leukemia cells. Then, these strengthened T cells were given back to the patients. CAR T cells recognize and target a protein called CD19, which is found on the surface of all B cells—both healthy and cancer cells.

After treatment, there were no signs or symptoms of cancer in the blood of 10 of the 12 patients evaluated. Cancer in these patients responded very quickly, within seven to 14 days after treatment with the CAR T cells. Researchers also found that the three patients who had Ph+ ALL benefited from treatment.

What Patients Need to Know

Although B-cell ALL first responds to treatment, it returns in many people and then no longer responds to chemotherapy, even medication that targets Ph+ cancer cells. So early success with CAR T-cell treatment is an exciting story in the treatment of these resistant types of leukemias. Researchers plan to study this new treatment strategy in more people with resistant leukemias to confirm these results. They also hope to learn more about CAR T cells, such as how long the benefits may last, which people with ALL may benefit most and how to use these cells in other (non–B-cell) cancers.

Myeloproliferative Disorders

Identifying mutations (changes) in a newly discovered gene called calreticulin may help doctors diagnose myeloproliferative tumors in some patients. The mutations can be found through blood testing. In the future, this and other related gene findings may be useful in identifying and treating people with myeloproliferative disorders *(page 14).*

A drug called imetelstat appears to be a promising new treatment for myelofibrosis. Although doctors do not know exactly how this drug works, it seems to affect the cancer cells in the bone marrow that drive myelofibrosis *(page 16).*

Myeloproliferative disorders (MPDs) include:

- Some types of blood cancers such as chronic myelogenous, chronic neutrophilic and chronic eosinophilic leukemias;
- Polycythemia vera, in which red blood cells are overproduced in the bone marrow, raising the risk of blood clots from thickened blood;
- Essential thrombocythemia, which involves overproduction of platelets and overactivity of platelet-forming cells, which also can lead to thickened blood;
- Primary myelofibrosis, characterized by scarring of the bone marrow.

Calreticulin to Help Diagnose MPDs

Identifying mutations (changes) in a newly discovered gene called calreticulin may help doctors diagnose MPDs in some people. The mutations can be found through blood testing. In the future, these gene findings may also be useful for treating myeloproliferative disorders.

This information is the result of two studies. In the first study, researchers examined blood samples from more than 1,100 patients with MPDs. They found 36 different types of mutations in calreticulin.

Among people with essential thrombocythemia who did not have the JAK2 V617F gene mutation (change), which is common in patients with MPDs, nearly 70 percent did have changes in their calreticulin genes. Among those with primary myelofibrosis who did not have the JAK2 gene change, nearly 90 percent did have mutations in their calreticulin genes.

Patients with mutations in the calreticulin gene appeared to survive longer than those who had mutations in the JAK2 gene. People with essential thrombocythemia and gene mutations in calreticulin had a lower risk of blood clots than those who did not have mutations in this gene.

In the second study, researchers found mutations in the calreticulin gene in 26 of the 31 patients who had MPDs and no mutations in the JAK2 gene or in MPL, another important gene in these disorders. They also found mutations in the calreticulin gene in about 70 percent of 112 patients with essential thrombocythemia and in 56 percent of the 32 patients with myelofibrosis but no mutations in either JAK2 or MPL. Importantly, there was no overlap between the presence of JAK2 mutations and that of calreticulin mutations.

What Patients Need to Know

Mutations in the newly discovered gene calreticulin are found in most people with MPDs who do not have the JAK2 gene change. Finding out whether a person has mutations in the calreticulin gene can help doctors correctly diagnose—and then treat—these types of MPDs. And now, with a better understanding of the biology behind these blood disorders,

researchers may develop more effective treatments in the near future.

Imetelstat for Myelofibrosis

A drug called imetelstat appears to be a promising new treatment for myelofibrosis, according to the results of a small clinical trial. Imetelstat belongs to a new class of treatments called telomerase inhibitors. They target the protein telomerase, which is found in higher amounts in most cancer cells compared with healthy cells.

In this study, 18 people with myelofibrosis were given imetelstat intravenously (through a vein) either once every three weeks or weekly for four weeks followed by one dose every three weeks. Seven of the patients needed to have regular blood transfusions because of their condition.

So far, the myelofibrosis in eight of these people responded to treatment with imetelstat, with improvement in symptoms and blood cell counts. More than three months after treatment, 16 of the patients remained on treatment. Two of the seven people who needed regular blood transfusions at the start of the study no longer needed them after treatment.

What Patients Need to Know

Imetelstat is the first telomerase inhibitor to be studied in clinical trials for people with myelofibrosis. Researchers are encouraged so far with the results from this early stage of study. Although doctors do not know exactly how this new drug works, it seems to affect cancer cells in the bone marrow that drive myelofibrosis. Further clinical trials on imetelstat for more people with blood disorders should help doctors understand how best to use it and who might benefit most from treatment with it.

Multiple Myeloma

Combining two anti-cancer drugs—carfilzomib and lenalidomide—with dexamethasone may be an effective way to treat people with early myeloma. Because about 75 percent of people diagnosed with early myeloma are at risk of developing multiple myeloma five years later, it's important to find ways to stop the progression of the cancer *(page 18)*.

Older adults with multiple myeloma who are not candidates for a stem cell transplant may benefit from continuous treatment with lenalidomide. When this treatment was combined with dexamethasone, the patients experienced fewer side effects than with a three-medication treatment that is often used *(page 19)*.

Pomalidomide and carfilzomib are proving to be effective against multiple myeloma in people whose cancer returns

after other treatments. That's important because there are few treatment options for people with resistant multiple myeloma, especially for those who have certain genetic mutations, or changes *(page 21)*.

A new oral medication called ixazomib appears to be a promising treatment for people with resistant multiple myeloma. Like carfilzomib, ixazomib tends to cause fewer side effects than bortezomib, such as nerve damage to the hands and feet *(page 22)*.

The combination of a new medication called panobinostat with bortezomib and dexamethasone seems to benefit people whose multiple myeloma no longer responds to standard treatments. Studies have shown that panobinostat and bortezomib seem to work better together than either drug alone *(page 24)*.

Carfilzomib, Lenalidomide and Dexamethasone for Early Myeloma

Combining two anti-cancer drugs—carfilzomib (Kyprolis) and lenalidomide (Revlimid)—with the steroid dexamethasone may prove to be an effective way to treat people with early myeloma. The U.S. Food and Drug Administration (FDA) has approved carfilzomib for treatment of multiple myeloma that does not respond to other drugs such as bortezomib (Velcade) and thalidomide (Thalomid). Lenalidomide is an FDA-approved treatment for another blood cancer called mantle cell lymphoma and for people with multiple myeloma.

In a small clinical trial, 12 patients with early myeloma were given carfilzomib, lenalidomide and dexamethasone. Thus far, nine patients have completed four cycles of treatment, and the cancer in all nine responded. Seventy-five percent of these

nine people had no signs or symptoms of early myeloma after receiving the combination treatment.

What Patients Need to Know

Carfilzomib belongs to a new and exciting class of drugs called proteasome inhibitors, which are designed to block the growth of tumor cells. These types of medications tend to cause fewer side effects than standard treatments. Although further studies of this promising combination treatment are needed, researchers believe that treating people with early myeloma before they develop severe signs of multiple myeloma may help improve their outcomes later.

Lenalidomide for Older Adults With Multiple Myeloma

People older than age 65 and those who are not candidates for a stem cell transplant may benefit from continuous treatment with lenalidomide, according to the results of a French clinical trial. In one of the largest studies of this group of patients with multiple myeloma, the combination of lenalidomide and dexamethasone seems to be more effective in stopping the growth of cancer than the standard treatment of melphalan (Alkeran and others), prednisone and thalidomide (MPT).

In the study, more than 1,500 patients were treated for newly diagnosed multiple myeloma. Two-thirds of them were given lenalidomide and dexamethasone, and the others were given MPT.

About three years after treatment, more cancers responded to treatment with lenalidomide than the standard MPT treatment (75 percent versus 62 percent). Patients treated with lenalidomide survived longer without their cancer growing than those treated with MPT. Also, patients who received

lenalidomide had a lower risk of developing a second cancer than those who received MPT.

Another clinical trial, conducted in Italy with the same type of patients, showed similar results. Nearly 650 older people with newly diagnosed multiple myeloma took part in this study. Two-thirds of these patients received either melphalan, prednisone and lenalidomide (MPR) or cyclophosphamide, prednisone and lenalidomide (CPR). The other third received lenalidomide and dexamethasone.

The cancer in 75 percent to 80 percent of all patients in the study responded to treatment. Nearly two years after treatment, the cancer had not continued to grow in approximately half of all the patients—regardless of which treatment they received. About 80 percent of all patients in the study had survived two years after treatment. However, those who were treated with MPR or CPR had more side

effects affecting their blood cells than those who were treated with lenalidomide and dexamethasone (51 percent versus 29 percent).

What Patients Need to Know

Melphalan and prednisone are combined more often with other drugs in Europe, where these studies took place, than in the United States. Lenalidomide and dexamethasone are used more in the United States to treat people who are newly diagnosed with multiple myeloma. Three-medication combinations generally tend to cause more side effects than two-medication combinations. Researchers are encouraged by the long-term benefits seen with lenalidomide and dexamethasone compared with the three-medication treatment in these older people with multiple myeloma.

Pomalidomide for Resistant Multiple Myeloma

Several small clinical trials with pomalidomide (Pomalyst) show that it may be an effective treatment when combined with other medications for people who have resistant multiple myeloma—even for those with gene mutations (changes) that usually make treatment more challenging. Pomalidomide has been approved by the FDA for multiple myeloma that no longer responds to two other treatments (including lenalidomide and bortezomib).

In one study, 19 patients had multiple myeloma that no longer responded to treatment with many other medications, including lenalidomide. They all were given pomalidomide, bortezomib and dexamethasone. Of the 12 patients evaluated so far, 10 (83 percent) have responded to treatment. In fact, there were no signs or symptoms of cancer in four of them.

In another clinical trial, 72 people with resistant multiple myeloma were treated with pomalidomide, the newer medication carfilzomib and dexamethasone. Of these patients, 16 had the genetic change known as del(17p), and six had the genetic change known as t(4;14).

Of the 67 patients evaluated so far, cancer in almost two-thirds has responded to treatment. The average time it took for the cancer to continue growing in all of the people taking part in the study was 12 months. For those who had genetic mutations, the time it took for the cancer to continue growing was nearly 10 months. Yet another small clinical trial showed the promising benefits of pomalidomide and dexamethasone in many people with resistant multiple myeloma that had genetic mutations.

What Patients Need to Know

There are few treatment options for people with resistant multiple myeloma, especially for those who have certain genetic mutations. Drugs such as pomalidomide and the newer proteasome inhibitor carfilzomib are proving to be effective against multiple myeloma in people whose cancer returns after other treatments. Researchers are calling these early results with pomalidomide and carfilzomib "remarkable" and plan to study these treatments further in more people with multiple myeloma to confirm these benefits.

Ixazomib for Resistant Multiple Myeloma

A new oral medication, the proteasome inhibitor called ixazomib, appears to be a promising treatment for people with resistant multiple myeloma. Thirty-three people with resistant multiple myeloma took part in a clinical trial of ixazomib. The medication was given once a week for three out of four weeks.

Of the 32 patients evaluated so far, the cancer shrank in 15 percent. The results improved when dexamethasone was given to those who had not benefited at first. In these patients, the cancer in 35 percent responded to ixazomib. Six months after treatment, 96 percent of all the people who received ixazomib were surviving.

What Patients Need to Know

Other studies have shown that ixazomib and dexamethasone may prove to be an effective combination treatment for people with resistant multiple myeloma. This proteasome inhibitor can be taken by mouth, making it convenient for patients to use. Other promising oral medications in this class are still being studied in earlier stages of research. Like carfilzomib, ixazomib tends to cause fewer side effects, such as nerve damage to the hands and feet, than the proteasome inhibitor bortezomib.

Panobinostat and Bortezomib for Resistant Multiple Myeloma

The combination of a new medication called panobinostat with bortezomib and dexamethasone seems to benefit people whose multiple myeloma no longer responds to standard treatments. Studies have shown that panobinostat and bortezomib seem to work better together than either drug alone.

More than 700 people with resistant multiple myeloma took part in two related clinical trials known as PANORAMA 1 and 2. In the first study, cancer in more than 650 patients had initially responded to some type of bortezomib treatment. In the second study of 55 patients, their cancer did not respond to bortezomib.

Of the patients evaluated so far, the combination of panobinostat, bortezomib and dexamethasone extended the time it took for the cancer to continue growing. Cancer in about 50 percent of those in the second clinical trial responded to treatment, shrinking in 19 patients.

What Patients Need to Know

Panobinostat belongs to a class of medications called histone deacetylase (HDAC) inhibitors. They work by destroying cancer cells or stopping their growth. The first HDAC inhibitor approved was vorinostat (Zolinza), which is an effective treatment for those with a blood cancer known as cutaneous T-cell lymphoma. Researchers hope that giving the newer HDAC inhibitor panobinostat with bortezomib may prove to be even more effective than any single drug in treating people with resistant multiple myeloma. Further studies with panobinostat will help doctors learn how best to use it in the future.

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