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Latest News in Blood Cancer Research

Highlights From the 2014 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 2014 Annual Meeting of the American Society of Hematology, which took place December 6–9 in San Francisco, California.

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.

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Highlights From the 2014 Annual Meeting of the American Society of Hematology

Latest News in Blood Cancer Research

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Leukemia

A number of studies reported at the 2014 Annual Meeting of the American Society of Hematology show important progress in the treatment of leukemia:

Many people with chronic myeloid leukemia (CML) who have no signs of cancer after being treated may safely stop taking their medication, according to the results of three clinical trials. Researchers believe those who have continued to respond well to treatment for up to eight years may have the best chance of successfully stopping treatment without a return of cancer *(page 4)*.

A new diagnostic method called dPCR may be able to predict which patients with CML who are no longer taking medication may be at risk of having their cancer return. Two factors—patient age less than 45 years combined with a

positive dPCR result—predicted the likelihood of relapse with 100 percent accuracy *(page 5).*

Ponatinib was more effective than imatinib in patients with newly diagnosed chronic-phase CML. Ponatinib currently is used to treat people with CML who no longer respond to or cannot tolerate imatinib or other medicines. The results of this study suggest that it also may be beneficial as an initial treatment *(page 6).*

Previously untreated patients with advanced chronic lymphocytic leukemia (CLL) appear to benefit more from the combination of fludarabine, cyclophosphamide and rituximab (FCR) than those who first received bendamustine and rituximab. For people with CLL who are physically fit, FCR remains the standard of care *(page 7).*

Researchers have learned that people whose CLL is in remission after treatment with ofatumumab may benefit from continuing to receive it. Patients who continued taking ofatumumab went longer without their cancer returning than those who stopped taking the drug *(page 8).*

The addition of bortezomib to sorafenib and vorinostat appears to be a promising new treatment for people who have acute myeloid leukemia (AML) that no longer responds to other treatments. Researchers plan to study the combination of these three drugs further to confirm its benefits in this group of difficult-to-treat patients *(page 9).*

According to two clinical trials, a new medicine called tosedostat in combination with cytarabine or decitabine stopped the growth of cancer in older people with previously untreated AML or myelodysplastic syndrome (MDS). Researchers will continue to study tosedostat in people with AML as well as in people with other types of cancer to see who would benefit most from such treatment *(page 10).*

Safely Stopping Treatment of CML

People with CML whose cancer responded to treatment may safely stop taking their medication, according to results from three clinical trials.

In the first study, researchers found that over 200 patients who stopped receiving tyrosine kinase inhibitors (TKIs), such as dasatinib (Sprycel), imatinib (Gleevec) and nilotinib (Tasigna), were much more likely to be completely free of all signs of their cancer (remission) than previously expected. Researchers used a standardized molecular test to check for remission in patients with CML. All of these patients had been treated with a TKI for an average of eight years.

The duration of treatment appeared to play a critical role. In people who were treated with a TKI for more than eight years, the leukemia returned in about 25 percent. In contrast, in those who were treated for less than eight years, signs of CML returned in nearly half. Some of the patients who received imatinib experienced temporary bone or muscle pain after they stopped taking it.

In the second clinical trial, the majority of over 50 patients with CML who stopped taking dasatinib or nilotinib still had no signs of their cancer returning up to two years later. At one year after treatment was stopped, 61 percent of the patients were still in complete remission. At two years, 57 percent remained cancer-free.

In the third trial, which is still gathering data in 115 people with CML, it appears that imatinib treatment may be safely discontinued without leading to a return of their cancer. Patients in this study received treatment with imatinib for an average of seven years. Fifteen months after they stopped taking the drug, almost 75 percent of them remained free of

cancer. Of the 25 percent of patients whose cancer did return, most of them responded to retreatment with imatinib after about three months.

What Patients Need to Know

TKIs like imatinib have revolutionized the treatment of CML and helped many patients to survive longer with no signs or symptoms of leukemia. Studies have now shown that some of these people who have responded well to these treatments may safely stop taking them without their cancer returning. Additional studies should help doctors to learn which people with CML would have the best chances of stopping treatment with TKIs and remaining free of cancer. So far, researchers believe those who have been treated with these medications for a longer time and who have responded well to them may have the best chance of successfully stopping their treatment without a return of cancer.

New Diagnostic Method for Predicting Relapse in CML

A new diagnostic method, the digital-polymerase chain reaction (dPCR), may be beneficial in predicting relapse, or a return of cancer, in persons with CML who are no longer taking imatinib. Doctors normally recommend that their patients stop taking imatinib when laboratory tests indicate that their leukemia is in remission (no evidence of cancer).

dPCR is able to provide a detailed picture of a person's DNA. People with CML who have certain changes in their DNA may be more prone to relapse.

The study focused on 108 people with CML who were no longer taking imatinib because they were in remission. After 32 months, the leukemia had come back in nearly 44 percent

of these patients. The majority of relapses occurred in the first nine months after they stopped taking imatinib. Two factors, age less than 45 years combined with a positive dPCR result, predicted the probability of relapse with 100 percent certainty. Patients whose cancer came back resumed taking imatinib.

What Patients Need to Know

Researchers continue to search for ways to identify which persons with CML who are in remission may relapse after no longer taking imatinib. This study showed that dPCR may be a valuable diagnostic tool for predicting relapse in patients with CML and possibly other cancers. However, further research is necessary before dPCR becomes part of standard practice.

Ponatinib Versus Imatinib in Newly Diagnosed CML

Ponatinib (Iclusig) was more effective than imatinib in people with newly diagnosed chronic-phase CML, according to early results from a clinical trial in 306 patients. About 90 percent of patients have chronic-phase CML when first diagnosed.

In this study, approximately half of the people received ponatinib and the other half received imatinib. After three months, patients receiving ponatinib had a higher molecular response rate than those receiving imatinib (31 percent versus 3 percent). Molecular response is a predictor of long-term outcome of treatment in patients with CML. Generally, patients who achieve a high molecular response from their treatment are less likely to have their cancer come back.

Although ponatinib was found to be more effective than imatinib, more patients receiving it had side effects. The most common side effects were rash (38 percent), abdominal pain (36 percent) and headache (33 percent). Other side effects

included constipation, increased levels of lipase (an enzyme produced by the liver), muscle pain, a loss of infection-fighting white blood cells (neutropenia) and blood clots.

What Patients Need to Know

Ponatinib is a powerful medicine approved to treat people with CML who no longer respond to or cannot tolerate other cancer drugs. The researchers who conducted this study found that it may be more effective than imatinib in newly diagnosed people with chronic-phase CML. However, people who receive ponatinib may be at higher risk of blood clots than those who are treated with imatinib. Further studies of ponatinib in people with newly diagnosed CML may help doctors to find the safest, yet most effective, dose of this medication.

Fludarabine, Cyclophosphamide and Rituximab in CLL

Previously untreated people with advanced CLL who are physically fit appear to benefit more from a combination of fludarabine, cyclophosphamide and rituximab (Rituxan), commonly known as FCR, and can go longer without their cancer returning than people who first received bendamustine (Treanda) and rituximab, according to a study in 547 patients.

Approximately half the patients received FCR, and the remainder received bendamustine and rituximab. Forty months after starting treatment, more patients receiving FCR than those receiving bendamustine and rituximab had no sign of cancer (41 percent versus 32 percent). Also, patients who received FCR went longer without their cancer coming back than did those receiving bendamustine and rituximab (54 months versus 43 months). Patients under 65 years of age appeared to benefit the most from FCR treatment.

What Patients Need to Know

For people with CLL who are physically fit, FCR remains the standard of care. Researchers now have more evidence that this combination treatment helps these people to survive longer without their cancer growing. However, many older, fit patients with CLL who are treated with FCR have more side effects and infections, making it difficult for them to remain on their treatment and receive its full benefit. So, older people with CLL might benefit more from treatment with bendamustine and rituximab.

Ofatumumab for Maintenance Treatment of CLL

In patients with CLL that is resistant to standard treatment, maintenance treatment with ofatumumab (Arzerra) was effective in stopping their cancer from coming back, according to a clinical trial in 474 patients. Maintenance treatment often is given to people who are in remission to keep their cancer from returning.

Half of the patients received ofatumumab for up to two years. The remaining patients received no medication but continued to be observed for any sign that their cancer had returned. Patients who received ofatumumab were able to go an average of 29 months without their cancer coming back. In contrast, those who were observed but didn't receive medication went an average of only 15 months without their cancer returning.

Patients receiving ofatumumab had higher rates of side effects than patients who were simply monitored. The most common were neutropenia and pneumonia.

What Patients Need to Know

Ofatumumab is an approved treatment for people with CLL. It is an effective medicine for those who have not received treatment before for CLL and for those who have. Researchers are now pleased to learn that people whose CLL is in remission may benefit from continuing to take ofatumumab. The patients in this study will be followed to see whether maintenance treatment with ofatumumab also can help them to survive longer.

Bortezomib in Difficult-to-Treat AML

Adding bortezomib (Velcade) to sorafenib (Nexavar) and vorinostat (Zolinza) was found to be effective in a small group of patients with difficult-to-treat AML, according to an ongoing clinical trial in 15 patients. Patients identified as having difficult-to-treat AML have a gene mutation (change) known

as FLT3-ITD or have other changes in their chromosomes that decrease the odds of their cancer responding to treatment. Fifty-nine percent of the patients had the FLT3-ITD mutation, and 53 percent had other changes that put them at risk.

All of the patients in this small study had AML that either resisted other treatment or relapsed after being treated with other medications or stem-cell transplantation. Forty percent of the patients responded to the combination of bortezomib with sorafenib and vorinostat, and 27 percent had no signs of leukemia after being treated.

What Patients Need to Know

The combination of bortezomib, sorafenib and vorinostat appears to be a promising treatment for people who have AML that no longer responds to other medications or stem-cell transplantation. However, these results are from a very early phase of research with a small number of patients, so researchers plan to study the combination treatment further to confirm these encouraging results.

Tosedostat in Previously Untreated AML or MDS

A new medicine called tosedostat combined with cytarabine (Cytosar-U and others) or decitabine (Dacogen and others) stopped the growth of cancer in older people with previously untreated AML or MDS, according to two clinical trials. People with AML or high-risk MDS with certain gene mutations have a poorer response to chemotherapy and a higher risk of relapse after treatment.

A total of 34 people 60 years and older participated in the first study. Twenty-nine of these patients had AML, and five had high-risk MDS. Half of them received tosedostat

and cytarabine, and the other half received tosedostat and decitabine. The drugs were given for an average of three months. More than half of these patients responded to treatment with tosedostat combined with cytarabine or decitabine, with 14 patients showing no signs of AML or MDS after treatment.

In the second clinical trial, 33 elderly patients (average age, 75 years) with AML received tosedostat combined with low doses of cytarabine. Half of these patients had previously untreated AML, and the other half of them had AML that had returned (relapsed) after prior treatment.

More than half (54 percent) of the patients responded to tosedostat and cytarabine, with 15 of the 33 patients showing no signs of AML after being treated. More than half of these patients were still cancer free after an average of 10½ months, and two other patients showed no signs that their cancer was still growing. The study was reported in June, 2015 at the 20th Congress of the European Hematology Association.

What Patients Need to Know

These two small studies show that adding tosedostat to cytarabine or decitabine may prove to be an effective way to treat older people with previously untreated or relapsed AML. In both studies, more than half of the patients who received tosedostat responded to treatment. In addition, many of these patients did not have to be admitted to the hospital to receive treatment.

Researchers will continue to study tosedostat in people with AML as well as in people with other types of cancer to see who would benefit most from such treatment.

Lymphoma

At the 2014 Annual Meeting of the American Society of Hematology, researchers reported important progress in lymphoma treatment:

Positive outcomes from two clinical trials suggest promising new roles for brentuximab vedotin in the treatment of people with Hodgkin lymphoma. Brentuximab vedotin added to doxorubicin, vinblastine and dacarbazine improved outcomes and was more effective than supportive care only after autologous stem-cell transplantation *(page 13).*

The combination of brentuximab vedotin and bendamustine was found to be safe and effective in patients with Hodgkin lymphoma that was resistent to initial treatment or had returned after initially responding. However, further study of

the potential benefits and risks of this combination in more patients is needed *(page 14)*.

Combining bendamustine with rituximab may be of benefit in people with slow-growing lymphomas and in elderly people with mantle cell lymphoma. The combination treatment was found to be more effective than standard treatment known as R-CHOP *(page 15)*.

Two new medications, polatuzumab vedotin and pinatuzumab vedotin, appear to be safe and effective when combined with rituximab in patients with non-Hodgkin lymphoma. These patients' cancer had returned after previous treatment or resisted treatment with other drugs *(page 16)*.

Brentuximab Vedotin in Hodgkin Lymphoma

Brentuximab vedotin (Adcetris) was found to be safe and effective in people with advanced Hodgkin lymphoma, according to early results from two clinical trials.

The standard treatment for Hodgkin lymphoma is a combination of four drugs—doxorubicin, bleomycin, vinblastine and dacarbazine (DTIC-Dome and others)—commonly known as ABVD. In the first study, researchers replaced the bleomycin in ABVD with brentuximab vedotin and gave it to 26 patients with newly diagnosed Hodgkin lymphoma that had already reached a late stage. Three years after diagnosis, all but one of the patients had no signs of cancer, and all 26 survived. No major side effects occurred by substituting bleomycin with brentuximab vedotin.

In the second study, 329 patients with Hodgkin lymphoma were divided into two groups. Half of them received brentuximab vedotin for 48 weeks after autologous stem-cell transplantation (ASCT); the other half received placebo (a

look-alike product with no active ingredients) and supportive care only after ASCT. Patients who received brentuximab vedotin after ASCT survived significantly longer without their cancer returning than those who had ASCT and supportive care alone (43 months versus 24 months).

ASCT is a form of treatment for blood disorders and cancers such as lymphoma. During ASCT, healthy stem cells are removed from a patient's blood, stored and later given back to the patient to make new blood cells. Approximately 50 percent of eligible patients with Hodgkin lymphoma are cured after they receive ASCT.

What Patients Need to Know

Brentuximab vedotin currently is used to treat Hodgkin lymphoma in patients who are not cured by ASCT or whose cancer has resisted treatment with chemotherapy and are not candidates for transplantation. The results of these two studies suggest promising new roles for brentuximab vedotin in improving the outcomes of chemotherapy and ASCT, particularly for people with advanced lymphomas.

Brentuximab Vedotin With Bendamustine in Hodgkin Lymphoma

For people with Hodgkin lymphoma whose cancer has resisted initial treatment or returned afterward, the combination of bendamustine (Treanda) with brentuximab vedotin was found to be safe and effective.

Forty-five patients with Hodgkin lymphoma that had resisted or returned after initial treatment took part in this clinical trial. Of those who received the full treatment course, 94 percent showed improvement after receiving brentuximab vedotin and

bendamustine. In 82 percent, all signs of Hodgkin lymphoma disappeared after treatment with the two drugs.

The most common side effects were chills, shortness of breath and redness of the face and neck. Taking anti-allergic medication before the two drugs were injected helped prevent these reactions.

What Patients Need to Know

The combination of bendamustine and brentuximab vedotin may be an option for people with Hodgkin lymphoma that no longer responds to previous treatment. Further study is required to determine the best dose of bendamustine and brentuximab vedotin when they are used in combination. Patients should speak with their doctors before deciding if this treatment is right for them.

Bendamustine With Rituximab in Lymphoma

Combining bendamustine with rituximab (Rituxan) benefited people with slow-growing lymphomas and elderly people with mantle cell lymphoma.

According to a clinical trial in 514 patients, more patients with slow-growing lymphomas who received the combination of bendamustine with rituximab survived longer without their cancer coming back than did patients receiving standard treatment (72 percent versus 62 percent). In patients with mantle cell lymphoma, there was less of a difference between patients receiving bendamustine and rituximab and those receiving standard treatment.

Standard treatment for these types of lymphomas is a combination of five drugs—rituximab, cyclophosphamide, doxorubicin, vincristine and prednisone—called R-CHOP.

What Patients Need to Know

Combining bendamustine and rituximab may be a treatment option in patients with slow-growing lymphomas and some elderly patients with mantle cell lymphoma. Further research is needed, however, before the combination of bendamustine and rituximab can become a part of standard treatment in these patients.

Polatuzumab Vedotin and Pinatuzumab Vedotin With Rituximab in Relapsed or Resistant Non-Hodgkin Lymphoma

Two new medications, polatuzumab vedotin and pinatuzumab vedotin, were effective in patients with non-Hodgkin lymphoma (NHL) that returned (relapsed) after treatment with other drugs or had resisted other treatment. Like brentuximab vedotin, these new medications are known as antibody-drug conjugates that specifically target cancer cells.

An ongoing clinical trial in people with relapsed or resistant NHL is exploring the safety and effectiveness of these new medications in combination with rituximab. So far, 59 patients have been treated with polatuzumab vedotin plus rituximab and 63 with pinatuzumab vedotin plus rituximab. Two thirds of these patients have diffuse large B-cell lymphoma (DLBCL); the other third have follicular lymphoma (FL).

Of those who received polatuzumab vedotin with rituximab, 56 percent of patients with DLBCL and 70 percent of those with FL responded to treatment. Among the patients who received pinatuzumab vedotin plus rituximab, 57 percent of those with DLBCL and 62 percent of those with FL responded. Between 10 percent and 40 percent of these patients had no signs of their cancer 10 months after starting treatment.

However, patients who received polatuzumab vedotin or pinatuzumab vedotin with rituximab did experience side effects, including fatigue, diarrhea, nausea, temporary nerve damage (such as weakness, numbness or pain), neutropenia (a loss of white blood cells that protect against infection), constipation and decreased appetite. More than 40 percent of the patients stopped taking these drugs due to side effects.

What Patients Need to Know

Polatuzumab vedotin or pinatuzumab vedotin combined with rituximab may be a treatment option in people with NHL that has resisted treatment with other medications or returned. Further research is needed before these two treatments can become part of standard therapy.

Multiple Myeloma

At the 2014 Annual Meeting of the American Society of Hematology, researchers reported important progress in the treatment of multiple myeloma:

Adding carfilzomib to lenalidomide and dexamethasone helped to stop cancer from returning in patients with multiple myeloma. The combination of these three drugs is even more effective in stopping the return of cancer in people with resistant multiple myeloma *(page 18)*.

Maintenance treatment with ixazomib in patients whose multiple myeloma was in remission was found to be safe and effective. Ixazomib is a promising new treatment for multiple myeloma that has resisted or returned after other treatment *(page 19)*.

Carfilzomib in Relapsed Multiple Myeloma

In people with multiple myeloma whose treatment was no longer effective, the addition of carfilzomib (Kyprolis) to lenalidomide (Revlimid) and dexamethasone, known as KRd, was more effective in stopping their cancer from returning than treatment without carfilzomib, according to the results of a clinical trial in 792 patients. Lenalidomide with dexamethasone (a type of steroid) is the standard of care for people with relapsed multiple myeloma.

Half of the patients in this study received KRd. The other half received only lenalidomide and dexamethasone. Patients who received KRd went longer without their cancer coming back than those receiving lenalidomide and dexamethasone alone (26 months versus about 17½ months). In addition,

the patients who received KRd were more likely to have a complete or partial response than those who received standard treatment (nearly 85 percent versus 45 percent).

The side effects experienced by both groups of patients were similar in type and frequency. They included diarrhea, fatigue, cough, shortness of breath, peripheral neuropathy (weakness, numbness, tingling or a prickly sensation in the hands and feet) and lowered blood-cell counts.

What Patients Need to Know

The standard of care for people with relapsed multiple myeloma has been treatment with lenalidomide and dexamethasone. When carfilzomib was approved for treating people with multiple myeloma who no longer responded to standard treatment, doctors had another effective choice. Now, researchers are pleased to learn that adding carfilzomib to the standard treatment is even more effective in stopping the return of cancer in people with resistant multiple myeloma. In fact, this three-drug combination treatment, which appears to be as well tolerated as the standard treatment, provides a new option for patients and caregivers.

Ixazomib as Maintenance Treatment in Multiple Myeloma

Maintenance treatment with a new medicine, ixazomib (Ninlaro), was safe and effective in patients with multiple myeloma who were in remission (under control). Ixazomib was recently approved by the U.S. Food and Drug Administration (FDA) as a new treatment for multiple myeloma in combination with lenalidomide and dexamethasone. Maintenance treatment is the ongoing use of medication to help lower the risk of cancer recurrence.

Fifty patients with previously untreated multiple myeloma first received ixazomib, lenalidomide and dexamethasone. Twenty-one of those patients whose cancer was in remission received maintenance treatment with ixazomib. Eleven of them (52 percent) showed no evidence of multiple myeloma while receiving ixazomib as maintenance treatment, and six others had a partial response to ixazomib. The average length of response was 26½ months.

Ixazomib did have side effects, however, including mild-to-moderate diarrhea (38 percent), nausea (14 percent), pain in the hands and feet (14 percent), anemia (10 percent) and headache (10 percent). None of the patients stopped taking the drug because of its side effects.

What Patients Need to Know

Ixazomib belongs to a class of cancer medications known as proteasome inhibitors, which include carfilzomib and bortezomib (Velcade). Myeloma cells depend on proteasomes to make large amounts of myeloma proteins and survive. What's different about ixazomib is that it can be taken as a pill, making it an ideal drug for long-term maintenance treatment.

Researchers now know that extending treatment with ixazomib may help people with multiple myeloma respond better to combination treatment, and this is very good news. Lenalidomide is the currently accepted maintenance drug used by patients with multiple myeloma.

Further studies of maintenance treatment with ixazomib will determine whether it will prove to be a convenient, well-tolerated alternative to lenalidomide maintenance for people with multiple myeloma.

Myeloproliferative Disorders

At the 2014 Annual Meeting of the American Society of Hematology, researchers reported important advances in treating polycythemia vera and myelofibrosis:

Switching patients with polycythemia vera from hydroxyurea to ruxolitinib led to greater control of their symptoms, a reduction in the size of their spleen and improvement in their quality of life. Recently approved by the U.S. Food and Drug Administration (FDA) for treating polycythemia vera, ruxolitinib holds promise for people who no longer respond to hydroxyurea *(page 21).*

Imetelstat is a promising new medicine being studied in people with myelofibrosis. Although it stopped this blood disorder from coming back in certain patients, it caused significant suppression of the patients' immune system. Further research is needed before imetelstat becomes a standard treatment for myelofibrosis *(page 23).*

Ruxolitinib in Polycythemia Vera

Switching people with polycythemia vera from hydroxyurea (Hydrea and others) to ruxolitinib (Jakafi) led to greater control of their symptoms, a reduction in the size of their spleen and improvement in their quality of life. All 222 patients who participated in this clinical trial either still had symptoms after treatment with hydroxyurea or were unable to tolerate its side effects.

Half of the patients received ruxolitinib and the remainder received the best available treatment, as determined by their physician. Best available treatment may have included hydroxyurea, interferon or peginterferon, pipobroman,

anagrelide (Agrylin and others), lenalidomide (Revlimid), thalidomide (Thalomid) or no medication.

At 32 weeks, nearly half of the patients receiving ruxolitinib (49 percent) reported better control of their symptoms and improvement in their quality of life than patients receiving the best available treatment (5 percent). These symptoms included tiredness, itching, muscle ache, night sweats, sweating during the day, vision problems, dizziness, difficulties concentrating, headache, numbness or tingling in their hands or feet, ringing in their ears, skin redness and stomach discomfort.

In addition, more people receiving ruxolitinib experienced a reduction in the size of their spleen than those receiving the best available treatment (21 percent versus 1 percent). The spleen is an organ near the stomach that plays a vital role in defending the body against infection and removing old or damaged red blood cells. An enlarged spleen can be a sign of a number of blood disorders and cancers, including uncontrolled polycythemia vera.

People receiving treatment with ruxolitinib after first receiving hydroxyurea or other treatment also showed improvement in clinical outcomes. After 32 weeks of best available treatment, only 25 percent of patients did not require a phlebotomy

(removal of blood from a vein) to reduce the volume of red blood cells packing their circulation (hematocrit) or the size of their spleen. In contrast, 74 percent of patients receiving ruxolitinib from the start and 79 percent of those who switched to ruxolitinib did not require a phlebotomy.

The degree of spleen enlargement before the patients started taking ruxolitinib had no effect on how well the drug reduced their hematocrit or the size of their spleen.

Patients receiving ruxolitinib were able to go an average of one year before needing a phlebotomy. By comparison, among patients who received best available treatment, the average time they could go before needing phlebotomy was less than five months. Furthermore, patients receiving ruxolitinib achieved meaningful improvements in their symptoms, regardless of the change in their hematocrit, whereas patients treated with best available treatment showed worsening or no improvement in symptoms.

What Patients Need to Know

Researchers are pleased with the positive results of this study because they spell good news for people with polycythemia vera. In December 2014, ruxolitinib was approved by the FDA for treating people with polycythemia vera who no longer respond well to hydroxyurea or cannot tolerate its side effects. Many patients in this study who received ruxolitinib reported relief from several symptoms and improvement in their quality of life regardless of changes in their blood tests. Ruxolitinib also seemed to help people with polycythemia vera go longer without needing a phlebotomy.

Imetelstat in Myelofibrosis

Although imetelstat, a new treatment being studied in people with myelofibrosis, stopped cancer from coming back in

certain patients, it significantly lowered the ability of their immune system to fight infection, according to the results of a clinical trial in 33 patients.

Patients received imetelstat through a vein via intravenous infusion. Of the 33 patients, seven (21 percent) responded fully (no evidence of cancer) or partially (some evidence of cancer) after one to nine treatments. Six of these patients remained free or partially free of cancer for an average of 11 months. Three patients had a complete molecular response—the complete absence of any gene mutations (changes) associated with myelofibrosis in circulating blood cells.

Imetelstat also improved clinical outcomes in these patients. Twelve of 23 patients experienced a greater than 50 percent reduction in the size of their spleen, and four of 13 patients who depended upon receiving transfusions of red blood cells no longer required them.

However, treatment with imetelstat led to serious side effects in some patients. These side effects included low levels of red blood cells (27 percent), platelets (21 percent) and infection-fighting white blood cells (18 percent).

What Patients Need to Know

Researchers have long searched for effective treatments for people with myelofibrosis and are encouraged by the early promise of imetelstat. This medication is the first of a new class of treatments that block an enzyme called telomerase, which seems to be active in cancer cells and less so in healthy cells. Researchers will study imetelstat further—in blood cancers such as myelofibrosis as well as in breast cancer—to see who might benefit most from treatment with this drug and how best to reduce its side effects.

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