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14th Edition

CANCER CLINICAL TRIALS

» **Moving treatments forward, one patient at a time**

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TRANSFORM 2
M20-178

Myelofibrosis Research Study

Do you or someone you know have Relapsed/Refractory Myelofibrosis?

The TRANSFORM-2 study is a research study evaluating the efficacy and safety of an investigational medication (navitoclax) in combination with ruxolitinib compared to best available therapy. Navitoclax is an investigational drug that is not approved by the FDA or any other global health authority. Safety and efficacy have not been established.

Patients must meet the following criteria:

- 18 years of age or older
- Diagnosed with primary myelofibrosis, post-polycythemia vera myelofibrosis, or post-essential thrombocythemia myelofibrosis
- Currently have Intermediate-2 or High-Risk myelofibrosis
- Currently on treatment or have received prior treatment with ruxolitinib
- Have splenomegaly (enlarged spleen)

For more information, ask your doctor about the TRANSFORM-2 Study or visit **www.myelofibrosisresearch.com** and **www.clinicaltrials.gov** (NCT04468984) to see if you qualify.

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CO-EDITORS-IN-CHIEF

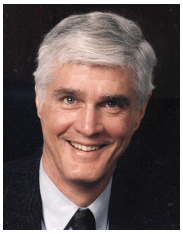
Charles M. Balch, MD, FACS, FASCO

Professor of Surgery, The University of Texas MD Anderson Cancer Center

Editor-in-Chief, Patient Resource LLC

Former Executive Vice President & CEO, American Society of Clinical Oncology

Past President, Society of Surgical Oncology



Paul A. Bunn, MD, FACP, FASCO

Distinguished Professor and James Dudley Endowed Professor of Lung Cancer Research, University of Colorado School of Medicine

Past President, American Society of Clinical Oncology

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Chief Executive Officer	**Mark A. Uhlig**
Co-Editors-in-Chief	**Charles M. Balch, MD, FACS, FASCO** **Paul A. Bunn, MD, FACP, FASCO**
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Office Address	**8455 Lenexa Drive** **Overland Park, KS 66214**
For Additional Information	**prp@patientresource.com**
Advisory Board	**Visit our website at PatientResource.com to read bios of our Medical and Patient Advisory Board.**

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Educate yourself about trials as you consider treatment options

Clinical trials are a critical component of cancer research. Most of the advances made in treating cancer today were once therapies or procedures that were developed, tested and evaluated through the clinical trials process to gain approval from the U.S. Food and Drug Administration (FDA). The ultimate goal is to help you and the entire cancer community enjoy longer, healthier lives.

These highly regulated research studies rely on volunteers to help doctors search for new and better ways to prevent, diagnose, treat and cure cancer. Regardless of where you are in the continuum of care – newly diagnosed or ready for a new option – clinical trials offer possible access to state-of-the-art treatments. And, by participating, you will be a partner in cancer research, helping improve treatments for future patients.

It is a good idea to bring up the topic of clinical trials early in your care, especially if your doctor does not mention it first. Your medical team will guide you through the process if a clinical trial is the next step. You can even help search for clinical trials on your own (see *Searching for a Clinical Trial*, page 3).

As with any cancer treatment, those used in clinical trials present potential risks and extra time commitments. Ask your doctor about possible side effects and the schedule to accommodate the tests and appointments that are required for the trial.

AREAS OF RESEARCH

Clinical trials explore treatment and non-treatment strategies, such as disease prevention, patient screening, diagnostic tools and procedures, genetic risk factors and lifestyle/behavioral changes. Many trials incorporate measures to improve quality of life.

Therapeutic Treatments

Most people are familiar with this type of clinical trial that tests a new medical approach. These treatments may include new drugs, drug combinations, surgeries, medical procedures or devices. Recently, a great deal of focus has been on the identification of therapies that treat cancers with specific biomarkers. Research is also ongoing to find treatments for cancers that do not exhibit a biomarker for a specific mutation.

Disease Prevention and Patient Screening

Designed to evaluate one or more ways to identify or diagnose a particular disease or condition, these trials also find ways to prevent the initial development or recurrence of a disease or condition. These can include medicines, vaccines or lifestyle changes, among other approaches.

Diagnostic Tools and Procedures

These trials are conducted to examine new and improved methods for identifying a condition or the risk factors for that condition.

Genetic Risk Factors

Researchers seek to learn more about the genetic disorders and disease-related mutations that cause various types of cancer.

Lifestyle/Behavioral Changes

These trials are designed to explore and measure ways to make people more comfortable as they manage a chronic condition. Some of the studies test the effect lifestyle changes have on lowering the risk of cancer and on current cancer treatments. ■

Clinical trials need a wide variety of participants

▸ **Doctors understand that people can experience** the same disease differently because each patient is biologically and genetically unique. It is vital that new medications are tested in clinical trials where participants reflect real-world populations to get a clear understanding of the drug's safety and efficacy. For everyone to get the most benefit from cancer research, volunteers from many different backgrounds and life experiences are necessary. Volunteers of all ages, genders, locations, races and ethnicities, weights, sexual orientations and socioeconomic groups are needed.

Recent studies have shown that the quality of research increases when the volunteers come from diverse groups because different life experiences add valuable perspectives to these projects.

Many factors influence how people react to certain drugs, medical devices and treatment plans. Therefore, drawing from a diverse pool of volunteers allows researchers to use science-driven strategies to determine which therapies are best for specific groups of people. This research can also take into account how a therapy works in people who are at higher risk for certain conditions such as heart disease and diabetes.

The rise of precision medicine is also driving the need for more specific information about the potential users of certain types of therapy so that treatments can be tailored for them and their condition. Precision medicine uses information about a person's own genes or proteins to prevent, diagnose and/or treat disease.

Following are additional benefits of including more diversity within clinical trials:

- Reduces biases
- Promotes health equity
- Contributes to producing innovative treatments
- Improves public perception about clinical trials
- Builds public confidence about participating

Progress is being made to include a wider variety of people in clinical trials. This is due in part to new guidelines and strategic plans, which are encouraging scientists and clinical trial planners to include more diverse groups of people in research.

The National Institutes of Health Revitalization Act of 1993 was signed into law to establish guidelines to include women and persons from racial and ethnic minority populations in clinical research. More recently, the National Institutes of Health Minority Health and Health Disparities Strategic Plan 2021-2025 was created to address disparities in health care. This patient-centric approach to cancer research will help bring new and improved therapies to the populations that will be using them to live longer.

The path to finding a clinical trial

When looking for a clinical trial, there are some specific steps that you must take. Start by having a conversation with your medical team so you understand the types of trials they may be considering and then become an active part of your treatment team by joining the search. Follow the path outlined on this page to help guide your efforts and use the resources below to help you connect with advocacy groups and trial search databases. ■

1

Gather your diagnosis and treatment information.

Enter your diagnosis & details to narrow your search (age, preferred location).

2

3

4 Review the list of clinical trials & print the protocols for any that you think may be a good fit.

Search by cancer type online or by phone.

5 Select a trial & call the clinical trial team for more information. Ask lots of questions.

6 Discuss it with your doctor to see if it would be a treatment to consider.

7 Undergo testing to see whether you meet the eligibility criteria.

8 Talk it over with your loved ones. Review and sign the Informed Consent form.

9 Begin the clinical trial!

10 Continue to be an advocate for your care....

CLINICAL TRIAL RESOURCES

- **Cancer Support Community:** www.cancersupportcommunity.org/find-clinical-trial, 888-793-9355
- **Center for Information & Study on Clinical Research Participation:** www.searchclinicaltrials.org, 617-725-2750
- **ClinicalTrials.gov:** www.clinicaltrials.gov
- **Lazarex Cancer Foundation:** www.lazarex.org, 877-866-9523, 925-820-4517
- **National Cancer Institute:** www.cancer.gov/clinicaltrials
- **NCI Cancer Information Service:** 800-422-6237
- **WCG CenterWatch:** www.centerwatch.com, 866-219-3440

Get the facts and move forward with confidence

Every clinical trial includes a safety measure known as Informed Consent. It is designed to protect participants throughout the clinical trial. Once you and your doctor find a trial that may fit into your treatment plan, you will be provided with an Informed Consent document to review. This document can be lengthy, sometimes 20 pages or more, but do not worry. A member of the clinical trial team will guide you, answering your questions along the way.

WHAT IS INCLUDED IN INFORMED CONSENT?

Because every clinical trial is unique, each has its own Informed Consent form that has been reviewed and approved by several groups and individuals. It contains customized information about that trial to help you decide whether it is right for you.

Generally, the following key information is included on every Informed Consent form:

- The trial and its goals.
- The best treatment regimen, referred to as standard-of-care treatment, for the stage of your disease, regardless of the doctor or institution.
- The modality (method of treatment) and the dosage/frequency.
- Possible risks and benefits. As with any type of cancer treatment, potential risks as well as benefits may occur. Keep in mind that there is no guarantee that the treatment will be effective for you.
- How you will be monitored.
- The schedule of appointments.
- Potential side effects to expect.
- The costs that are covered by the trial and those that will be your responsibility.
- The safeguards in place (see *Safety Measures*, page 7).
- How to withdraw from the trial. Even after you begin a clinical trial, you are not locked in. Participation is always voluntary. You can leave the trial at any time and for any reason, and opt for standard-of-care treatment.

You should have a reasonable amount of time to read the information at home. As you do, take notes and make a list of questions to ask.

Next, schedule an in-person or telehealth meeting with the clinical trial team to talk in detail about the trial and ask your questions. You are encouraged to have a family member or friend attend with you. It helps to have another person hear the same information. If English is not your first language, this is the time to request the assistance of a translator at the meeting.

If at any time you find the medical terminology or any other aspect of the trial confusing, simply ask. A member of the team will explain it in easy-to-understand language. Be sure to take notes to refer to later.

After the meeting, check with your health insurance provider to determine the procedures that are covered and those you will be expected or required to pay out of pocket (see *Financial Considerations*, page 7). Although many components of the trial may be covered, other expenses, such as travel, lodging, food, parking and more, may be your responsibility. It is important for you to have this discussion before you begin participating in the trial.

You may request additional time to consider what you have learned or to talk it over with loved ones. If you choose to move forward, let the team know that you are ready to sign the Informed Consent form. Ask for a copy of the signed document for your files.

It is important to know that even after signing the form, you may withdraw from the trial at any time and for any reason.

When changes are made to the clinical trial, such as modifications to procedures or changes to the risks and benefits, the Informed Consent form is revised. As another safety measure, participants must be made aware of any information that may help them decide whether they should continue with the trial. ■

The Four Phases of Treatment Trials

➥ Treatment trials have traditionally been designed in four phases, an approach that ensures only rigorously researched treatments are approved for the public. The information gained in each phase adds to the knowledge of the next phase. This process allows researchers to ask and answer questions in a way that produces the most reliable information and provides the most protection to trial participants.

Based on the successes of other trials, the FDA recently has shortened its approval times for certain new therapies and will consider approvals at any phase of research, giving patients earlier access to lifesaving treatments.

Phase I	Phase II	Phase III	Phase IV
▸ Evaluates a new drug (or other type of treatment) to see if it is safe for use in people. The goal is to determine how and when the drug should be given, and the dosage that will be most effective for killing diseased cells while causing the fewest side effects.	▸ Determines how well a treatment works and how safe it is in a greater number of patients.	▸ Compares the new treatment with the current standard of care to see whether it is more effective or has fewer side effects.	▸ Tests a drug that has already been FDA-approved for the market to gather more information about its effect in different populations and learn about long-term side effects.

R E S E A R C H S T U D Y

The ENVELOP Study

A Clinical Research Study for Myeloproliferative Neoplasms and Chronic Myelomonocytic Leukemia

We are conducting a research study to evaluate the safety and effectiveness of an investigational medication called navitoclax in adults with the following types of blood and bone marrow conditions: Polycythemia Vera (PV), Essential thrombocythemia (ET), Myelofibrosis (MF), and Chronic Myelomonocytic Leukemia (CMML).

Patients must meet the following criteria:

- 18 years of age or older
- Documented diagnosis of primary or secondary Myelofibrosis (MF), Essential Thrombocythemia (ET) or Polycythemia Vera (PV) or Chronic Myelomonocytic Leukemia (CMML)
- Have declined or not had success with at least one standard treatment
- Additional study entrance criteria will apply

ENVELOP

Scan Here to Learn More

Navitoclax, an investigational agent, is under clinical development and is not approved by regulatory health authorities. Its safety and effectiveness are under evaluation.

As an avid weightlifter, body builder and biking enthusiast, Richard Bagdonas leads a healthy lifestyle. A Stage IV mantle cell lymphoma diagnosis at 45 shocked him. He joined an immunotherapy clinical trial and today he is considered to be in complete metabolic remission. He is passionate about being a patient advocate and offers support to others facing a cancer diagnosis.

When it comes to cancer, this survivor is "crushing it"

Odd as it sounds, Montezuma's revenge helped me to discover I had mantle cell lymphoma. While on vacation with my family in Mexico, I ate some fruit that had the bacteria that causes the condition. When the discomfort continued after we returned home, I called my doctor, who referred me to a gastroenterologist. He performed a colonoscopy and found a snippet of inflammation that he sent to pathology. But he felt everything else looked good.

After the colonoscopy, my symptoms persisted. Three weeks later, the doctor called and wanted me to come into the office right away. Not realizing the seriousness of the situation, I thought I'd set up an appointment for two weeks later, but he insisted he needed to talk to me. He said I had pulled the golden lottery ticket because they had figured out that I had lymphoma, specifically mantle cell lymphoma. I was confused. I wasn't sure why having lymphoma was lucky. No one feels lucky to get cancer.

He explained that the sample he took was examined by the pathology lab and they found cells they didn't recognize. They had another lab take a look, and they couldn't identify them either. The sample was then sent to a lab in Boston where they identified the strange cells as mantle cells. The theory was that because lymphoma is a disease of the lymphatic system, mantle cells were created and then drained into my gut, and the cells were found in my colon. It was lucky and unusual that they'd been able to determine this through a colonoscopy.

Once I heard the diagnosis, my world collapsed. I read online that the life expectancy was three to five years and it was a fast-growing cancer. I didn't want to accept this. Friends and family helped me search for a specialist, and we found an expert in mantle cell lymphoma. I called him and was able to see him in person four days later.

The specialist performed multiple tests and concluded I had Stage IV mantle cell lymphoma that had spread to my bone marrow and lymph nodes in my neck and groin. He told me I could do the standard treatment at the time, but I'd likely only have 10 years. Being that I was still a relatively young man at 45 years old, he recommended I explore a clinical trial that was testing two immunotherapy drugs. It was starting in a few months.

Before joining the trial, I sought a second opinion with another oncologist. She told me she could only offer me the current approved treatment and confirmed that my best option was to join a clinical trial. With the help of my specialist, I enrolled as the first patient.

During the trial, I focused on having a positive mindset, used affirmations and meditation, and continued to work out and eat healthy. In meditation, I would focus on believing that I was past the cancer and was cured, that my body just needed to catch up. Throughout the trial, I never had any adverse symptoms.

When the second month of treatment ended, they performed a PET and discovered I was in complete metabolic remission, meaning tests could not find any lymphoma in my body. The trial then put me on a targeted therapy to help my body get rid of any remaining "dead" lymphoma cells. Thankfully, I responded well and went on with my life.

Now I am a spokesperson for the hospital that conducted that clinical trial. I share my experience to inspire others to consider clinical trials. One of the highlights of my life was meeting Jim Allison, Ph.D., who won the Nobel Prize in Physiology or Medicine for developing a type of immunotherapy for treating cancer.

I recommend patients consider participating in clinical trials 110 percent. I think patients should look for one in the beginning because some trials require that you haven't had a previous treatment. And, it's a good idea to research this option before you need it.

Having support is critical, and surrounding yourself with people with positive energy is so helpful. Keeping a positive attitude is also important. I offer my support to give back to others. To share some of my tips for keeping healthy during treatment, I wrote a book, *Fit for any Battle*. I believe that our bodies have to be as fit as possible to be ready for treatment, so strengthening it beforehand is a great strategy. Before starting treatment, try to be in the best health possible. And you don't have to be a weightlifter like me to get the benefits of exercise during cancer treatment. Anyone who has recently been diagnosed with cancer can contact me at richard@fitforanybattle.com for a free copy of my book.

With the help of clinical trials and tons of support, I was able to crush cancer. ■

Understand the financial obligations of being in a clinical trial

Every cancer treatment, even those used in a clinical trial, has associated costs. Typically, some of the expenses for treatment in a trial are covered by the sponsor. Before you proceed with a trial, read the detailed list of costs included in the Informed Consent form. Also contact the trial coordinators to request a list of the services and tests that will take place at each study visit. Knowing what to expect will help you plan your budget as you move forward.

Costs related to clinical trials generally fall into two categories:

1. Routine patient care applies to any type of cancer treatment, including those used in clinical trials. It usually involves expenses related to doctor visits, hospital stays and some testing procedures.
2. Research costs are directly related to the clinical trial and are typically covered by the trial sponsor. They include drugs, laboratory and imaging tests for research purposes, and procedures.

INSURANCE COVERAGE

After learning which costs are not covered by the trial sponsor, contact your insurance company to verify whether those costs will be covered by your health insurance plan or whether they will be your personal responsibility.

GOVERNMENT ASSISTANCE

The following federal and state government programs may offer assistance with the costs associated with a clinical trial:

- The Patient Protection and Affordable Care Act (ACA), a federal law that regulates health plans and insurance, covers routine clinical trial costs. Clinical trials covered under the ACA must be designed to study new ways to prevent, detect or treat cancer or other life-threatening illnesses.
- Medicare covers portions of clinical research studies, such as a cancer drug's effectiveness. Medicare Part A and/or Part B may cover some items, such as office visits and tests, in certain qualifying studies. Medicare Advantage plans may pay the difference in your out-of-pocket costs between it and traditional Medicare.
- TRICARE is the Department of Defense's health care program in partnership with the National Cancer Institute (NCI).
- The U.S. Department of Veterans Affairs (VA) allows eligible veterans to participate in NCI-sponsored clinical trials at VA medical centers.

OTHER TYPES OF ASSISTANCE

Whether or not you are insured or underinsured, ask the trial's administrators about assistance programs offered by the pharmaceutical companies that manufacture the drugs being tested in the trial, and flip this guide over for a list of resources in *Assistance & Support,* page 25. ■

Participant safety is the top priority

One of the most common concerns of people who are exploring clinical trials is safety. Rest assured, by the time a clinical trial is offered as a potential treatment option for you, it has gone through extensive testing. Multiple safety measures and guidelines make up the safety net of support that continues to protect you throughout the trial.

The safeguards established include a set of rules called a protocol. The protocol states what the study will do, how it is done and why it is done. It includes the trial's eligibility criteria, specifies the tests and procedures, describes the medications and dosages, outlines the information that will be collected, and establishes the duration of the study. A scientific review panel evaluates the protocol carefully to make sure the trial is based on sound science. All participating clinics, hospitals, universities, cancer centers and medical offices must follow the same protocol.

Following are some of the layers of safety involved with clinical trials.

The **U.S. Food and Drug Administration** (FDA) is responsible for the safety, efficacy and security of drugs. The FDA monitors the drug development process and works closely with pharmaceutical companies to ensure the integrity of new treatments and medications. All drugs must pass a series of tests and undergo a rigorous evaluation process by the FDA's Center for Drug Evaluation and Research (CDER).

Institutional Review Boards (IRBs) are groups that review each clinical trial's protocols before the study begins and monitor the trial's ongoing progress from beginning to end.

Data and Safety Monitoring Boards (DSMBs) review the progress of a clinical trial while monitoring the participants. They also review data on the effectiveness of the trial interventions.

Physicians, research experts and investigators directly supervise all studies. This extra safety measure is designed to ensure compliance with all scientific and ethical guidelines. ■

NATIONAL RESEARCH ACT

This act established regulations that led to the creation of these basic ethical guidelines:

Respect for people: All people, including those who require assistance to make their own decisions, should be respected and have the right to choose which treatments they receive.

Beneficence: People are treated in an ethical manner by respecting their decisions, protecting them from harm and making efforts to secure their well-being. Additionally, people should be protected from harm by maximizing benefits and minimizing risks in the research study.

Justice: All people should share the benefits and burdens of research.