



MYELOPROLIFERATIVE NEOPLASMS

# CLINICAL TRIAL

# CORNERSTONE DIRECTORY



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## WHAT ARE THE GOALS OF MPN CLINICAL TRIALS?

- Create truly curative therapies
- Develop therapies that can work when others have failed
- Decrease the side effects of treatment

## TIPS FOR LEARNING ABOUT & FINDING CLINICAL TRIALS

- Know your MPN status and obtain a copy of your pathology report
- Learn about eligibility criteria such as age and physical fitness
- See if there are financial resources that can help assist with travel and other costs
- Ask what the risks are to participating

## MYELOFIBROSIS (MF)

Here's a list of treatments in clinical trials for myelofibrosis (MF).

- **JAK inhibitors.** JAK inhibitors are under study in clinical trials and have shown effectiveness in reducing spleen size and symptoms such as fatigue and night sweats, and potentially anemia. Pacritinib (Vonjo or SB1518) is under study to lower platelet counts, and momelotinib is under study for the treatment of anemia.
- **Epigenetic drugs.** Epigenetic drugs alter the organization of genes to make them more or less accessible for utilization by cells.
- Recent studies with epigenetic drugs have shown that the HDAC inhibitor givinostat and the hypomethylating agents azacitidine (Onureg or Vidaza) and decitabine (Dacogen) were minimally effective in MF treatment in early studies (in contrast to their effectiveness in treating PV). Another HDAC inhibitor called panobinostat (Farydak) is currently under study.
- **Antifibrotic agents.** Antifibrotic agents interfere with the tissue repair process and fibrosis. PRM-151 is an antifibrotic therapy that is under study to see whether it prevents and/or reverses fibrosis in patients with MF.
- **Lysyl oxidase (LOX) and lysyl oxidase-like (LOXL).** Two additional antifibrotic medications under study in clinical trials.

## MYELOFIBROSIS (MF) CONTINUED

List of treatments in clinical trials for myelofibrosis (MF)

- **Pomalidomide (Pomalyst).** Pomalidomide has shown effectiveness in treating anemia in early studies. It targets the patient's immune system to attack abnormal cells to make space for the normal cells that make red blood cells. With enhanced anti-cancer activity and lower toxicity compared to other drugs in its class, pomalidomide has shown promise in initial studies and is now in Phase III clinical trials for its use as a first-line therapeutic for treating anemia in MF patients with the V617F mutation.
- **Everolimus (Afinitor).** Everolimus (also known as RAD001) is an inhibitor of the mTOR/AKT pathway, which is highly active in MF blood-producing cells and appears to contribute to abnormal cell growth. Everolimus was well-tolerated and able to reduce both spleen size and systemic symptoms in Phase I and II clinical trials.
- **Telomerase inhibitor.** A drug called imetelstat is under study for use in MF treatment to improve bone marrow fibrosis, blood cell counts, and bone marrow function.
- **Panobinostat (Farydak), pracinostat (SB939), umbralisib (Ukoniq), bomedemstat (IMG-7289), and navitoclax (ABT-263).** For patients who no longer respond to ruxolitinib (Jakafi), researchers are studying the use of these drugs, either in combination with ruxolitinib or as single-agent therapies.
- **Momelotinib, luspatercept (Reblozyl), and sotatercept.** Use of these drugs is under study to help patients with chronic anemia.
- **Bomedemstat (IMG-7289).** This drug is under study to help patients with high platelet counts.

## ESSENTIAL THROMBOCYTHEMIA (ET)

Here's a list of treatments in clinical trials for essential thrombocythemia (ET).

- **Peginterferon alfa-2a (Pegasys).** Previously, interferon use was restricted because it was not well-tolerated by patients. There is new interest in the ability of peginterferon to induce a molecular response (remission) in some patients.
  - This type of treatment response occurs when a decrease is observed in the number of abnormal blood cells with genetic mutations.
  - New formulations of peginterferon are under study that may be more well-tolerated by patients than older formulations. Ongoing studies are comparing peginterferon alfa-2a with hydroxyurea (Hydrea) in essential thrombocythemia patients.
  - Researchers are also analyzing if achieving a molecular response with interferon reduces the risk of ET progressing to myelofibrosis (MF) or acute myeloid leukemia (AML).
- **Ruxolitinib (Jakafi).** Ruxolitinib has been shown effectiveness in patients with primary myelofibrosis and polycythemia vera (PV).
  - Researchers are now studying ruxolitinib use in ET patients who are refractory or intolerant to hydroxyurea, to see whether it decreases disease-related symptoms and platelet counts.

## ESSENTIAL THROMBOCYTHEMIA (ET) CONT.

Here's a list of treatments in clinical trials for essential thrombocythemia (ET).

- **Bomedemstat (IMG-7289).** Bomedemstat inhibits lysine-specific demethylase 1 (LSD1), an essential enzyme in the production and function of megakaryocytes, so it prevents excess platelet production.
  - The FDA has granted a fast-track designation for the development of bomedemstat for essential thrombocythemia treatment.
  - This FDA process is designed to facilitate the development and to expedite the review of drugs to treat serious conditions that fill an unmet medical need. Its purpose is to make vital new drugs available sooner to patients.



- **Aspirin.** The effects of aspirin regimens are under evaluation by researchers.
  - The studies include the use of two or three doses per day, compared to once-daily aspirin regimens, to prevent thrombosis in patients with intermediate-risk and high-risk essential thrombocythemia.

## POLYCYTHEMIA VERA (PV)

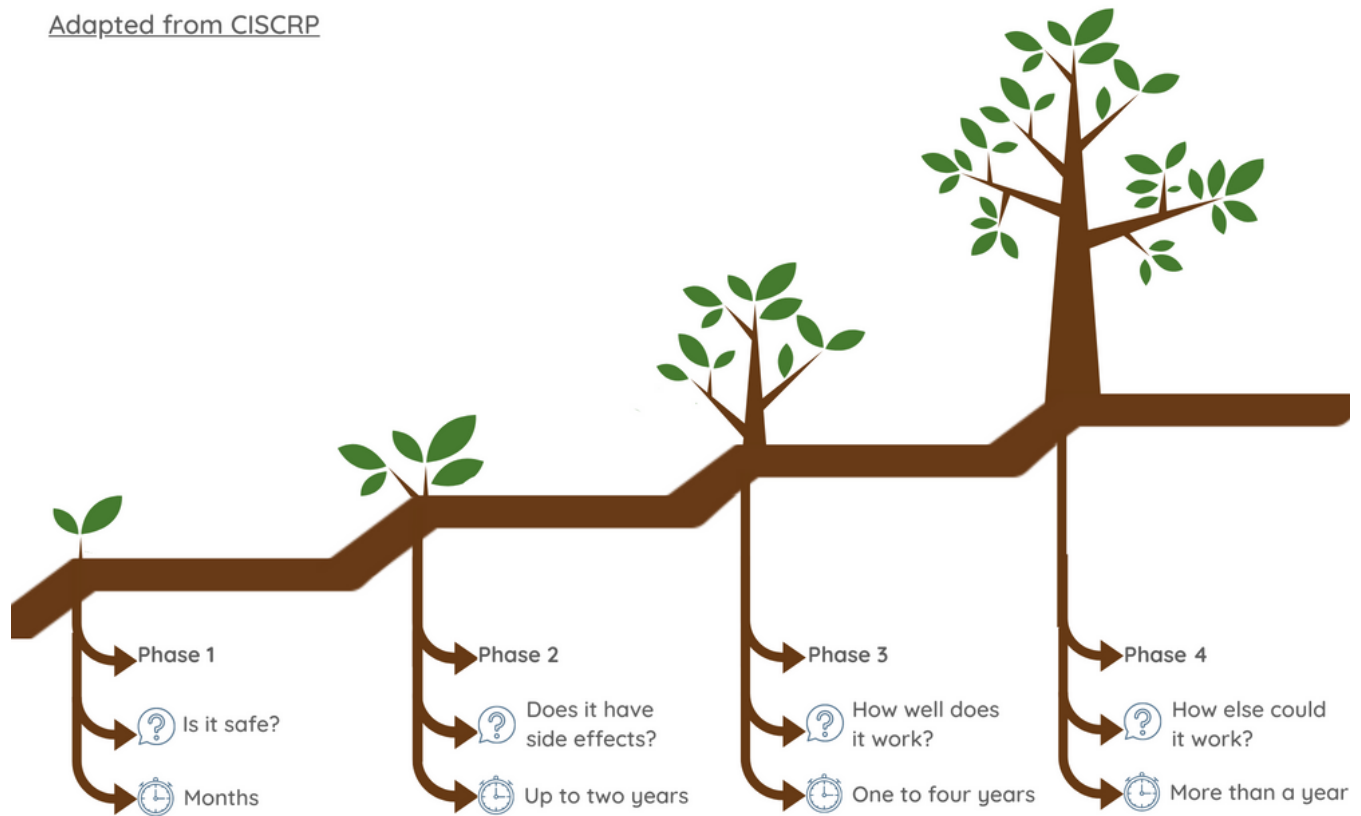
Here's a list of treatments in clinical trials for polycythemia vera (PV).

- **Ropeginterferon alfa-2b (Besremi).** Previously, the use of interferon was restricted because it was not well-tolerated by patients. However, there is interest in interferon's ability to induce a molecular response (remission) in some polycythemia vera patients.
  - This type of treatment response occurs in PV patients when there is a decrease in the number of cells with the abnormal JAK2 gene mutation.
  - New formulations of interferon may be effective in bringing on a molecular remission with less frequent doses and fewer side effects.
  - Researchers are analyzing pegylated formulations of interferon that can be given less frequently, since they remain in the body longer.
  - High molecular response rates have been shown in studies.
  - In 2019, ropeginterferon alfa-2b was approved by the European Medicines Agency for PV treatment in patients without symptoms of an enlarged spleen. It is now under study in clinical trials in the U.S.



# CLINICAL TRIALS & PHASES

Adapted from CISCRP



**Planting the seed** - In Phase I trials, researchers test a new drug or treatment in a small group of people (20-80) for the first time to evaluate its safety, determine a safe dosage range, and identify side effects.



**Laying down roots** - In Phase II trials, the study drug or treatment is given to a larger group of people (100-300) to see if it is effective and to further evaluate its safety.



**Preparing for harvest** - In Phase III trials, the study drug or treatment is given to large groups of people (1,000-3,000) to confirm its effectiveness, monitor side effects, compare it to commonly used treatments, & collect information that will allow the drug or treatment to be used safely.



**Expanding the yield** - In Phase IV trials, studies look at real-world experience over a long time and provide additional information on the drug's risks, benefits, and optimal use.

Feel more in control of your health journey and gain confidence around your treatment decisions no matter your geography.

# MPN Vignettes



**My Polycythemia Vera Journey to Empowerment**

[Read more >](#)



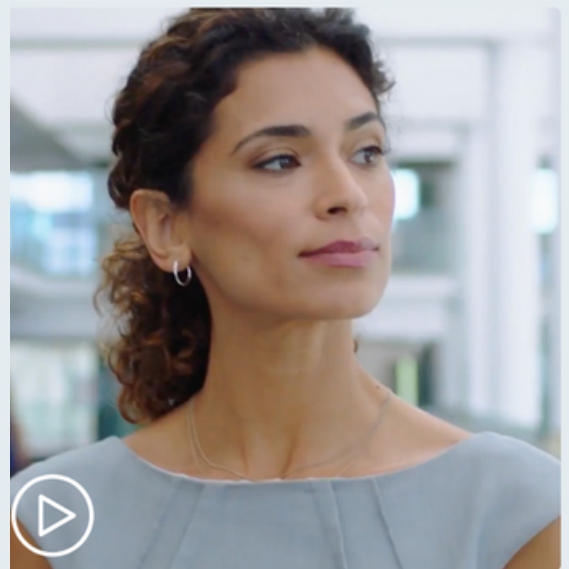
**Living With an MPN and Being Your Own Best Advocate**

[Read more >](#)



**My Journey of Living With a Myeloproliferative Neoplasm for 30 Years**

[Read more >](#)



**My MPN Journey, Getting the Best Care After ET and MF Diagnoses**

[Read more >](#)

Best MPN Care No Matter Where You Live is an advocacy and empowerment program for patients and families facing a myeloproliferative neoplasm (MPN) diagnosis where many factors, including age, ethnicity, socioeconomic status, gender and insurance type may impact disease outcomes.

Feel more in control of your health journey and gain confidence around your treatment decisions no matter your geography.



**ADVICE FOR HESITANT MPN CLINICAL TRIAL PARTICIPANTS**

What should MPN patients know about clinical trials? Dr. Claire Harrison from Guy’s and St. Thomas’ Hospital in London shares information about the varying degrees of clinical trials and advice to those who are hesitant about clinical trials.



**HOW CAN MPN PATIENTS BECOME MORE PROACTIVE IN THEIR CARE?**

How can MPN patients become more empowered and active in their care? Dr. Claire Harrison from Guy’s and St. Thomas’ Hospital in London shares advice for patients to gain confidence to become a more active participant for optimal care.



**NEW DEVELOPMENTS IN MPN TREATMENT LANDSCAPE**

For the MPN treatment landscape, what are the latest developments? MPN expert Dr. Claire Harrison from Guy’s and St. Thomas’ Hospital in London shares how the treatment landscape is changing and diagnostic criteria to be published on how each specific diagnosis should be used to optimize care.



**HOW CAN I GET THE BEST MYELOPROLIFERATIVE NEOPLASM (MPN) CARE?**

Internationally respected MPN expert Dr. Claire Harrison from Guy’s and St. Thomas’ Hospital in London shares information about research updates, treatment strategies, clinical trials, and how to achieve patient-centered care for you or your loved one.

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## CLINICAL TRIAL RESOURCES

MPN patients and care partners do not have to face their journey alone. Take advantage of support resources to locate clinical trials and financial assistance programs.

### **Clinicaltrials.gov**

Provides information on publicly and privately supported clinical studies on a wide range of diseases and conditions. The website is maintained by the National Library of Medicine at the National Institutes of Health.

**Each clinical trial record presents summary information about a study protocol and includes the following:**

- Disease or condition
- Intervention (for example, the medical product, behavior, or procedure being studied)
- Title, description, and design of the study
- Requirements for participation (eligibility criteria)
- Locations where the study is being conducted
- Contact information for the study locations

## NIH National Cancer Institute

**Contact:** Call 1-800-422-6237, live chat through [LiveHelp](#) or email [NClinfo@nih.gov](mailto:NClinfo@nih.gov)

Search portal to find National Cancer Institute (NCI)-supported clinical trials. Search by cancer type or keyword, your age (to determine which trials you could be eligible for) or U.S. ZIP code



## Clinical Connection

**Contact:** 1-800-887-0639; email [info@clinicalconnection.com](mailto:info@clinicalconnection.com)

Founded by a team of medical researchers whose goal has been to efficiently connect patients with clinical trial opportunities that are relevant and timely. Options to create a free member account to be notified when clinical trials that match your health interests become available in your area.

Search for trials (both in U.S. and internally) by ZIP code, keyword, or distance (select distance ranges starting from within 5 miles up to over 250 miles).



## CISCRP

(Center for Information and Study on Clinical Research Participation)

**Contact:** 877-MED-HERO (633-4376) or [info@ciscrp.org](mailto:info@ciscrp.org).

Provides education and information about clinical trials. Search Clinical Trials is a free service designed to help people find clinical trials that are relevant to their needs.

CISCRP staff will work with you to understand your options and will help you find local clinical trials in your community, or as far as you would be comfortable traveling.



## Antidote

1-888-509-1308 (US) or +44 808-196-0665 (UK)

**Email:** [hello@antidote.me](mailto:hello@antidote.me)

Search for clinical trials by condition, city or ZIP code, age, and gender. Receive list of clinical trials that could be a match for you by answering series of questions. Watch educational webinars and patient stories.

## FINANCIAL ASSISTANCE

### Lazarex Cancer Foundation

**Contact:** 877-866-9523 or 925-820-4517

**Other language(s):** Spanish, Mandarin, Korean

Helps cancer patients navigate clinical trial options by offering financial assistance (such as lodging and transportation costs) for participation in FDA-approved clinical trials; call for eligibility details. Also provides community outreach and education.

### MPN Research Foundation

**Contact:** 877-866-9523 or 925-820-4517

**Other language(s):** Spanish, Mandarin, Korean

Finding an MPN clinical trial that matches your diagnosis and treatment history is easier than ever with MPN Research Foundation's personalized clinical trial finder powered by Trialjectory.

Also provides community outreach and education.

## **21st Century C.A.R.E.**

Get immediate financial assistance for incidental expenses related to active cancer treatments. Must be referred by a physician to be considered for assistance.

Applications are processed without delay. Once the application is approved, then you are eligible for financial assistance for incidental expenses related to: transportation to and from treatments, follow-up visits related to cancer-care, childcare during treatment, temporary housing due to geographical distance from the treatment center, medical supplies, and much more.

**Medicare and the National Cancer Institute** provides information on Medicare coverage for clinical trials.

**Contact:** 1-800-633-4227 or 1-877-486-2048 for hearing impaired

## **CenterWatch offers online tools to:**

- Search clinical trials
- Receive email notifications about specific clinical trials
- Review results from completed clinical trials
- Search drug information
- Learn about the informed consent process
- Read an overview of the clinical trials process
- Find disease-specific health associations and other educational resources



# UNDERSTANDING CLINICAL TRIALS:

## A JARGON BUSTER GUIDE

By: Marie Ennis-O'Connor



When it comes to cancer treatment, you or a loved one may be considering participating in a clinical trial as a treatment option. Clinical trials are designed to evaluate the safety and effectiveness of a treatment. They may involve researchers administering drugs, taking blood or tissue samples, or checking the progress of patients as they take a treatment, according to a study's protocol.

Learning about clinical trials can be a steep learning curve – not the least because the process comes with a lot of new terms, acronyms, and jargon. To help you, we've put together this list of the most common terms you will find when you are researching clinical trial information. This is not an exhaustive list, but it is a helpful starting point. At the end of this article, you will see links to find more information.

## ADVERSE EFFECTS (AE)

Also called Adverse Events, or Adverse Drug Reaction, AEs are any harmful event experienced by a person while they are having a drug or any other treatment or intervention. In clinical trials, researchers must always report adverse events, regardless of whether or not the event is suspected to be related to or caused by the drug, treatment, or intervention.



## ARM

Subsection of people within a study who have a particular intervention.

## **BIAS**

Bias is an error that distorts the objectivity of a study. It can arise if a researcher doesn't adhere to rigorous standards in designing the study, selecting the subjects, administering the treatments, analyzing the data, or reporting and interpreting the study results. It can also result from circumstances beyond a researcher's control, as when there is an uneven distribution of some characteristic between groups as a result of randomization.

## **BLINDING**

Blinding is a method of controlling for bias in a study by ensuring that those involved are unable to tell if they are in an intervention or control group, so they cannot influence the results. In a single-blind study, patients do not know whether they are receiving the active drug or a placebo. In a double-blind study, neither the patients nor the persons administering the treatments know which patients are receiving the active drug.

## **COMPARATOR**

When a treatment for a specific medical condition already exists, it would be unethical to do a randomized controlled trial that would require some participants to be given an ineffective substitute. In this case, new treatments are tested against the best existing treatment, (i.e., a comparator). The comparator can also be no intervention (for example, best supportive care).

## **COMPLETED**

A trial is considered completed when trial participants are no longer being examined or treated (i.e., no longer in follow-up); the database has been "locked" and records have been archived.

## **CONTROL**

A group of people in a study who do not have the intervention or test being studied. Instead, they may have the standard intervention (sometimes called "usual care") or a dummy intervention (placebo). The results for the control group are compared with those for a group having the intervention being tested. The aim is to check for any differences. The people in the control group should be as similar as possible to those in the intervention group, to make it as easy as possible to detect any effects due to the intervention.



## **EFFICACY**

How beneficial a treatment is under ideal conditions (for example, in a laboratory), compared with doing nothing or opting for another type of care.

A drug passes efficacy trials if it is effective at the dose tested and against the illness for which it is prescribed.

## **ELIGIBILITY CRITERIA/INCLUSION AND EXCLUSION CRITERIA**

Eligibility criteria ensures patients enrolling in a clinical trial share similar characteristics (e.g., gender, age, medications, disease type, and status) so that the results of the study are more likely due to the treatment received rather than other factors.

## **FOLLOW-UP**

Observation over a period of time of participants enrolled in a trial to observe changes in health status.

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**INFORMED CONSENT** A process (by means of a written informed consent form) by which a participant voluntarily agrees to take part in a trial, having been informed of the possible benefits, risks and side effects associated with participating in the study.

**INTERVENTION** The treatment (e.g., a drug, surgical procedure, or diagnostic test) being researched. The intervention group consists of the study participants that have been randomly assigned to receive the treatment.

**INVESTIGATOR** A person responsible for the conduct of the clinical trial at a trial site. If a trial is conducted by a team of individuals at a trial site, the investigator is the responsible leader of the team and may be called the principal investigator (PI).

**MULTICENTER TRIAL** A clinical trial conducted according to a single protocol but at more than one site, and therefore, carried out by more than one investigator.

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## **NUMBER NEEDED TO TREAT (NNT)**

The average number of patients who need to receive the treatment or other intervention for one of them to get the positive outcome in the time specified.

## **OUTCOME MEASURES**

The impact that a test, treatment, or other intervention has on a person, group, or population.

## **PLACEBO**

A fake (or dummy) treatment given to patients in the control group of a clinical trial. Placebos are indistinguishable from the actual treatment and used so that the subjects in the control group are unable to tell who is receiving the active drug or treatment. Using placebos prevents bias in judging the effects of the medical intervention being tested.

## **POPULATION**

A group of people with a common link, such as the same medical condition or living in the same area or sharing the same characteristics. The population for a clinical trial is all the people the test or treatment is designed to help.

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## **PROTOCOL**

A plan or set of steps that defines how something will be done. Before carrying out a research study, for example, the research protocol sets out what question is to be answered and how information will be collected and analyzed.

### **Randomized Controlled Trial (RCT)**

A study in which a number of similar people are randomly assigned to two (or more) groups to test a specific drug, treatment, or other intervention. One group has the intervention being tested; the other (the comparison or control group) has an alternative intervention, a placebo, or no intervention at all.

Participants are assigned to different groups without taking any similarities or differences between them into account. For example, it could involve using a computer-generated random sequence.

RCTs are considered the most unbiased way of assessing the outcome of an intervention because each individual has the same chance of having the intervention.



## **RELIABILITY**

The ability to get the same or similar result each time a study is repeated with a different population or group.

## **SAMPLE**

People in a study recruited from part of the study's target population. If they are recruited in an unbiased way, the results from the sample can be generalized to the target population as a whole.

## **SUBJECTS**

In clinical trials, the people selected to take part are called subjects. The term applies to both those participants receiving the treatment being investigated and to those receiving a placebo or alternate treatment.

## **TRIAL SITE**

The location where trial-related activities are conducted.