

CLINICAL TRIALS AS AN MPN TREATMENT OPTION: WHAT YOU SHOULD KNOW

Program Resource Guide



Clinical Trial Phases

- **Phase I:** The goal is to test the safety of the drug, finding the appropriate dose that produces the fewest side effects.
- **Phase II:** Further assesses the safety of the drug and the effectiveness of the treatment.
- **Phase III:** Compares the efficacy of the new treatment to the standard-of-care treatment.
- **Phase IV:** Study that looks at drugs that have already been approved to get additional safety information and to learn more about long-term benefits and side effects.

Types of Clinical Trials

Controlled Clinical Trial: Study that includes a control (comparison) group, in which the control group receives a treatment, no treatment, or a placebo.

Double-Blind Study: Clinical trial in which neither the participants nor the researcher knows which intervention the participants are receiving until the end of the clinical trial, reducing the likelihood of biased results.

Observational Study: A study in which participants are observed or certain outcomes are measured. No treatment is administered, and there are no efforts to affect the study outcome.

Randomized Clinical Trial: Trial in which participants are randomly divided into separate groups that compare different treatments or other interventions. The randomization means that the groups will be similar so that treatment effectiveness they receive can be compared more fairly.

Common Clinical Trial Terminology

Adverse Event: Any possible side effect, unfavorable change in health, or even that occur in trial participants during the clinical trial or within a specified period following the trial.

Health Insurance Portability and Accountability Act (HIPAA): A set of national rules that help protect the privacy of a patient's personal medical information, provide patients with access to their medical records, and help people get health insurance for themselves and their family members.

Informed Consent: Provides a thorough explanation of the purpose of the research, including the role of the patient and how the trial will work.

Institutional Review Board (IRB): Committees that ensure that the risks of trial participation are reduced and outweighed by potential benefits in order to protect the rights and safety of research participants. Most clinical trials, but not all, in the United States are approved and monitored by an IRB both before the research starts and as it proceeds.

Placebo: An inactive drug or treatment with no therapeutic benefit.

Protocol: The written description of a clinical study that includes the objectives, design, and methods. The protocol may also include scientific background and statistical information that pertains to the study.

Standard of Care: An established guideline that is consensus among experts as the most appropriate and/or effective treatment for a specific type and stage of cancer.

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Questions to Ask Your Healthcare Team About Clinical Trials

- What is it about my disease that makes a clinical trial a consideration?
- Why is this particular trial right for me?
- What medication is it, and how does it help?
- What are the short-term risks?
- What are the long-term risks?
- If the treatment is successful, what can I expect?
- What does participation entail?
- What other options are available?
- How are study results and safety of participants being monitored?
- How long will the trial last?
- How frequently do I need to come in for visits?
- What costs might I expect?
- Who will fund the study?
- Who has reviewed and approved the study?
- Who will tell me about the results of the study, and how will I be informed?

Potential Barriers to Clinical Trial Participation

- Access to technology
- Education / Lack of awareness
- Eligibility criteria
- Financial barriers
- Insurance coverage
- Logistical issues
- Prior health conditions

Resources for MPN Clinical Trial Information

ClinicalTrials.gov

MPN Research Foundation: mpnrf.org

MPN Advocacy & Education International: mpnadvocacy.com

MPN Hub: mpn-hub.com

National Organization for Rare Disorders (NORD): rarediseases.org

The Leukemia & Lymphoma Society: LLS.org

MORE TOOLS FOR EMPOWERMENT

- Digitally Empowered™
- PEN-Powered Activity Guide
- Empowered Blog
- Empowered! Podcast



MPN Clinical Trials 201 is brought to you by the Patient Empowerment Network. Funding is provided by an unrestricted educational grant from AbbVie, Inc. and through generous donations from people like you.



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