WHAT PATIENTS SHOULD KNOW ABOUT DEVELOPING MPN TREATMENTS AND RESEARCH



Program Resource Guide

Questions to Ask About Clinical Trial Participation

- What is the clinical trial?
- What is involved in the trial?
- Has the drug been studied in people before?
- What are risks?
- What is the time commitment?
- What is the cost to participate?

MPN Treatments In Development

Therapies Being Studied in Combination With JAK Inhibitors

- Parsaclisib (PI3K Inhibitor)
- Pelabresib (BET Inhibitor)
- Navitoclax (Bcl-xL Cell Inhibitor)
- Luspatercept (Anemia Treatment)

Momelotinib: JAK inhibitor being studied in clinical trials for the treatment of myelofibrosis (MF).

Rusfertide (PTG-300): Being studied for the treatment of phlebotomy-dependent polycythemia vera (PV) patients.

Glossary Terms

Cytoreductive Therapy: Treatment to reduce the amount of cells the bone marrow is producing in order to control blood counts.

Eligibility Criteria: Requirements that must be met to be included in a clinical trial that ensure that the participants are like each other. These specific factors include age, type and stage of cancer, prior treatments, and general health.

Graft-Versus-Host Disease (GVHD): A complication of a bone marrow or stem cell transplant that occurs when the donor cells view the patient's healthy cells as foreign, and attack and damage them. GVHD can be mild, moderate, or severe.

Hydroxyurea (Hydrea): Oral chemotherapy agent used as a cytoreductive therapy in myeloproliferative neoplasms (MPNs) to control the number of white blood cells, red blood cells, and platelets – and to reduce spleen size.

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

Next-Generation Sequencing (NGS): Technology to sequence DNA or RNA to identify genetic variations associated with diseases or other biological phenomena.

JAK Inhibitors: A class of therapy that interferes with the activation of the JAK-STAT pathway **Stem Cell Transplant**, also called a bone marrow transplant, is a procedure in which healthy blood stem cells are used to replace damaged or diseased bone marrow. This procedure can be used to treat certain types of blood cancers.

Tissue Bank: An organization that collects, stores, and distributes human biological specimens for research purposes.

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