

Polycythemia Vera *Fact Sheet*

What is polycythemia vera (PV)?

Polycythemia vera (PV) is a rare and incurable blood cancer associated with an overproduction of blood cells in the bone marrow. This condition is part of a group of related blood cancers known as myeloproliferative neoplasms (MPNs)¹.

Normal bone marrow produces stem cells that develop into healthy blood cells, which are carefully regulated by the body. In PV, the mechanism used by the body to control the production of these blood cells functions abnormally, ultimately resulting in their overproduction¹.

The exact causes of PV are unknown; however, almost all patients have a mutation in the Janus kinase 2 (JAK 2) gene, which can cause a dysregulation and overproduction of blood cells¹.

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What are symptoms and complications of PV?

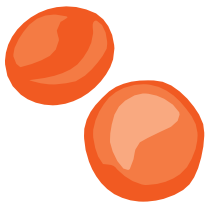
Signs, symptoms and complications of PV result from too many red blood cells. An abundance of red blood cells, in particular, can lead to a thickening of the blood and an increased risk of clots, which can cause serious cardiovascular complications, such as stroke and heart attack¹. PV can persist for many years and in some cases evolve to myelofibrosis (post-PV MF) or acute myeloid leukemia (AML)².

Common symptoms and complications of PV may include^{1,3}:



- Pruritus (itchy skin)
- Shortness of breath
- Fatigue and weakness
- Unexplained weight loss
- Headaches, visual disturbances and vertigo
- Bleeding, bruising and/or blood clots
- Enlarged spleen
- Angina (chest pain) or heart failure
- Painful inflammation of the joints (gout)

How is PV diagnosed?



Globally, PV affects up to three in every 100,000 people each year and is often discovered during a routine blood test^{1,4}. A Complete Blood Count (CBC) is the first diagnostic test used to help detect PV and provides information about the types and numbers of cells in the blood. Specifically, a CBC can measure red blood cell concentration through hematocrit, hemoglobin and red blood cell count, which are usually elevated in PV patients. Other common PV diagnostic tests include a erythropoietin (EPO) or bone marrow test^{1,5}.

Why is hematocrit control important in PV?

PV is typically characterized by an elevated hematocrit, a measure of the volume percentage of red blood cells in whole blood. Hematocrit is used to help diagnose PV and is a key measure of a patient's response to therapy. PV patients with an elevated hematocrit are at an increased risk of cardiovascular complications and death as well as debilitating symptoms. In order to help control PV,



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it is important for patients to maintain a hematocrit level below 45%, which is a common treatment target for individuals with the disease^{1,6}.

What is inadequately controlled PV?

Approximately 25% of patients with PV develop resistance to or intolerance of hydroxyurea and are considered to have uncontrolled disease⁷. This is typically defined as hematocrit levels greater than 45%, elevated white blood cell count and/or platelet count, and may be accompanied by debilitating symptoms and/or enlarged spleen⁸⁻¹⁰. Elevated white blood cell count and hematocrit are also associated with an increased risk of blood clots¹¹. Among patients who are resistant to hydroxyurea, the median overall survival is approximately five years⁷.

What treatments are available for PV?

The goal of PV treatment is to control symptoms and decrease the risk of complications. Common types of PV treatments include^{1,8,10}:

- **Phlebotomy:** A procedure to remove blood from the body to reduce the concentration of red blood cells, which is used to help maintain a hematocrit level below 45%. However, phlebotomy is usually unsuitable as a permanent treatment option due to its inability to control symptoms or effectively manage the overproduction of red blood cells.
- **Cytoreductive therapy:** Typically used to treat high-risk PV patients or used in combination with phlebotomy when phlebotomy is unable to control symptoms and blood counts alone.

References

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