

Essential Thrombocythemia (ET) or Polycythemia Vera (PV) Treatment:

A Global Patient–Provider Communication Checklist

ET and PV are 2 types of myeloproliferative neoplasms (MPNs). In these conditions, the bone marrow produces too many blood cells. They develop due to acquired genetic changes and typically progress slowly over time.

Essential Thrombocythemia¹

- ▲ The body makes too many platelets
- ▲ Platelets may not function properly, which can lead to:
 - Blood clots
 - Bleeding problems (less commonly)
- ▲ JAK2, CALR, or MPL mutations found in most patients (approximately 9 in 10)
- ▲ Can progress to post-ET myelofibrosis and/or acute myeloid leukemia

Polycythemia Vera¹

- ▲ The body makes too many red blood cells, and sometimes excess white blood cells or platelets
- ▲ Excess red blood cells thicken the blood, increasing the risk of blood clots
- ▲ JAK2 mutations found in nearly all patients (approximately 99 in 100)
- ▲ Can progress to post-PV myelofibrosis and/or acute myeloid leukemia

What treatments could I receive for ET or PV?^{1,2}

Treatment for ET and PV is personalized. It depends on factors such as age, history of blood clots, symptoms, and blood counts. The goals of treatment are to reduce the risk of blood clots, control symptoms, and prevent disease complications.

1. Therapeutic phlebotomy and low-dose aspirin

Low-dose aspirin

- ▲ Aspirin used at a low dose (81–100 mg/day) helps reduce the risk of blood clots by preventing platelets from sticking together

Therapeutic phlebotomy (primarily for PV)

- ▲ Therapeutic phlebotomy involves removing blood from the body to lower the hematocrit (the percentage of red blood cells in the blood) to reduce blood thickness (viscosity), improving blood flow and lowering clot risk
- ▲ Repeated phlebotomy can lead to iron deficiency, which may worsen symptoms such as fatigue or restless legs

2. Cytoreductive therapy (CRT)

- ▲ CRT is used when blood counts remain high despite other treatments, symptoms persist, the spleen becomes enlarged, or there is a higher risk of blood clots

Hydroxyurea³

- ▲ Hydroxyurea is an oral chemotherapy medication that slows down the production of blood cells in the bone marrow
- ▲ Possible adverse events include low blood counts (myelosuppression), mouth sores, skin changes including ulcers and higher risk for skin cancer, and, less commonly, lung or blood vessel toxicity

JAK inhibitors⁴

- ▲ JAK inhibitors block abnormal signaling pathways (JAK-STAT) that drive excessive blood cell and cytokine production
- ▲ Possible adverse events include higher risk of infections (especially respiratory), low blood counts, weight gain, headaches, elevated cholesterol levels, and increased risk for skin cancer

Interferons⁵

- ▲ Interferons are immune therapy that act on the root cause of the MPN to reduce the number of abnormal cells, rather than just managing blood counts
- ▲ Adverse events can include flu-like symptoms, fatigue, mood changes, elevated liver enzymes, autoimmune endocrine and other conditions, and retinal changes

*Discuss these regimens with your doctor, considering the treatment options available at that time.

Questions to Ask Your Doctor After Diagnosis of ET or PV

- ☐ What is my risk of blood clots, bleeding, and progression to myelofibrosis or leukemia?
- ☐ What is my care management plan?
- ☐ What blood count targets should I aim for? How do my current results compare?
 - ☐ Hematocrit (proportion of red blood cells)
 - ☐ White blood cell count
 - ☐ Platelet count
- ☐ What type of tests will I need and how often will I have them?
- ☐ How often should I have follow-up visits?
- ☐ Which symptoms or changes should I report immediately?
- ☐ What complications should I be aware of?
- ☐ Where can I find support and resources for my condition?

Questions to Ask Your Doctor About Your Treatment Options

- ☐ What are my treatment options?
- ☐ What are the possible side effects or complications of treatment?
- ☐ What will happen if I do nothing?
- ☐ How can I get a second opinion on treatment?
- ☐ What are my options if my condition progresses?
- ☐ What financial resources are available?
- ☐ Should I consider clinical trials?

Questions to Ask Yourself About Your Treatment Goals

- ☐ Who in my life will help me with my care decisions (eg, family and/or friends)?
- ☐ How involved do I want them in those decisions (eg, get their advice, decide together)?
- ☐ What are my treatment goals (eg, living longer, feeling better)?
- ☐ What are my biggest concerns about treatment?
 - ☐ Side effects
 - ☐ Emotional impact and mental health
 - ☐ Maintaining ability to work
 - ☐ Maintaining daily responsibilities at home
 - ☐ Impact on my relationships
 - ☐ Transportation to appointments
 - ☐ Scheduling appointments around work or other responsibilities
 - ☐ Arranging childcare/caregiving
 - ☐ Insurance coverage
 - ☐ Out-of-pocket costs

Notes: _____

Notes: _____

References

1. NCCN. Guidelines for patients: myeloproliferative neoplasms. v1.2024. nccn.org.
2. Kuykendall. Clin Lymphoma Myeloma Leuk.2024;24:512.
3. Hydroxyurea PI.
4. Ruxolitinib PI.
5. Ropeginterferon-alfa-2b-njft PI.