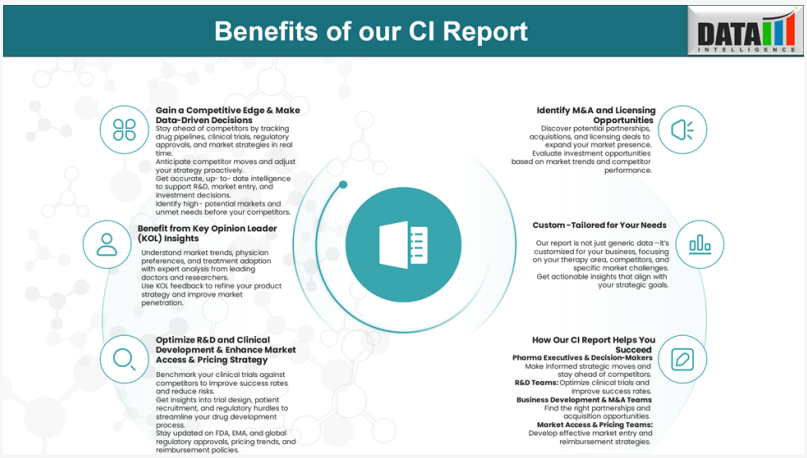
Polycythemia Vera Market Shifts Toward Novel Mechanisms and Long-Term Disease Control | Competitive Intelligence

The PV Market is shifting beyond JAK inhibitors, with new therapies targeting iron metabolism & epigenetics to improve efficacy, safety, & patient convenience.



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AUSTIN, TX, UNITED STATES, June 4, 2025 /EINPresswire.com/ -- Polycythemia vera(PV), a rare chronic blood cancer under the myeloproliferative neoplasm (MPN) umbrella, is undergoing a transformative shift in its treatment landscape. As understanding deepens around disease biology and patient needs, next-generation therapies are being designed to go beyond traditional control of hematocrit and splenomegaly — aiming instead for durable molecular remission, symptom relief, and improved quality of life. With the global prevalence of Polycythemia Vera estimated at approximately 22 per 100,000 individuals and a significant proportion of patients requiring lifelong treatment, the demand for more convenient, safer, and potentially disease-modifying options is intensifying.

> “PV care is at a turning point as novel therapies emerge to address unmet needs beyond current JAK and interferon-based treatments. Patient-centered innovation is now the defining battleground.”
>
> *DataM Intelligence*

PV predominantly affects older adults, particularly men above the age of 60. The disease is driven by mutations—most notably the JAK2V617F mutation—that lead to excessive production of red blood cells and, frequently, increased white blood cells and platelets. While phlebotomy remains a foundational intervention for hematocrit management, its burden on patients has led to the widespread adoption of pharmacological interventions.

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Current Standard of Care: Besremi and Jakafi Hold Ground
Currently, two drugs dominate the PV treatment landscape: Besremi® (ropeginterferon alfa-2b-njft) by PharmaEssentia and Jakafi® (ruxolitinib) from Incyte/Novartis. Besremi, a long-acting, monopegylated interferon, is approved in both the U.S. and Europe as a first-line therapy. Its appeal lies in its disease-modifying potential—evidenced by sustained molecular and hematologic responses—and its relatively infrequent dosing regimen (biweekly to monthly). Despite its clinical efficacy, Besremi is often associated with flu-like symptoms and psychiatric side effects, limiting its tolerability in some patients.

Jakafi, a JAK1/JAK2 inhibitor, is approved for patients who are intolerant to or fail hydroxyurea. It remains the most widely used second-line option, credited for rapid hematocrit control, spleen size reduction, and symptom relief. However, it brings risks of cytopenia and infection, and does not provide a molecular response—factors that may limit its long-term utility.

Emerging Pipeline: New Mechanisms, Fewer Side Effects, Greater Convenience
The PV pipeline is now bursting with innovative agents designed to overcome the limitations of existing treatments. At the forefront are non-JAK, non-interferon mechanisms that target the root causes of PV or its symptom burden, including iron metabolism, epigenetic modulation, and cytokine signaling.

Among the most promising candidates is rusfertide, a hepcidin mimetic from Protagonist and Takeda, currently in Phase III trials (VERACITY). Hepcidin, the body's natural iron regulator, plays a central role in red cell production. Rusfertide mimics its function, helping reduce phlebotomy dependence and maintain hematocrit levels without cytoreductive therapy. Its safety profile and targeted mechanism offer a compelling option, especially for patients burdened by frequent blood draws.

Bomedemstat, developed by Merck (originally Imago Biosciences), is an epigenetic LSD1 inhibitor currently in Phase III. By modulating chromatin remodeling, it aims to suppress the malignant clone at a genetic level, potentially reducing the JAK2V617F allele burden. As a first-in-class epigenetic therapy, bomedemstat could reshape the long-term management strategy for PV, especially if it proves effective in modifying disease progression.

Another candidate, Givinostat, from Italfarmaco, is an HDAC inhibitor also in Phase III trials. It specifically targets the JAK2 mutation pathway and holds promise for molecular response and symptom reduction. With its oral formulation, it could add a convenient, targeted option for long-term disease control.

Meanwhile, Sapablursen (ONO-0530), developed by Ono Pharmaceutical, introduces an antisense oligonucleotide strategy targeting thrombopoietin receptor mRNA. By modulating platelet production and inflammation, it offers a novel anti-inflammatory approach that could

work either as monotherapy or in combination with existing agents.

Competitive Dynamics: Shifting from Control to Cure
The evolving treatment landscape is driving competition not just on efficacy, but on convenience, tolerability, and long-term outcomes. A key framework for evaluating these pipeline agents is the Target Opportunity Profile (TOP) — a strategic tool that benchmarks what emerging therapies must demonstrate to outpace leaders like Jakafi and Besremi.

According to the TOP, next-gen PV therapies should aim for:
- Superior or equivalent hematocrit control without the need for regular phlebotomy
- Molecular response, specifically reduction in JAK2 allele burden
- Improved safety and tolerability, particularly in elderly or frail patients
- Oral or infrequent subcutaneous dosing, favoring long-term adherence
- Competitive or value-based pricing to enhance payer adoption

The unmet need for faster onset of action, reduced symptom burden, and durable remissions provides significant opportunity for pipeline agents that can deliver across multiple dimensions.

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Strategic Outlook: Redefining First-Line and Beyond
As the PV landscape continues to mature, PharmaEssentia's Besremi is expected to retain a stronghold in the front-line setting, especially among patients prioritizing disease modification. In contrast, Jakafi's dominance in the second-line space remains unchallenged in the short term, though its vulnerability lies in its lack of molecular impact and long-term tolerability concerns.

By 2026 and beyond, drugs like rusfertide and bomedemstat could gain regulatory approval and offer serious competition—either displacing current therapies or creating combination opportunities aimed at deeper remission and improved quality of life.

Ultimately, success in the PV market will hinge on addressing the holistic needs of patients—not just hematologic metrics, but daily life impacts, psychological well-being, and financial accessibility.

Conclusion
The polycythemia vera treatment paradigm is undergoing a fundamental transformation. From the historic reliance on hydroxyurea and phlebotomy to biologics like Besremi and Jakafi, and now to targeted, patient-friendly, disease-modifying agents, the market is rapidly diversifying. As pipeline candidates mature and new mechanisms of action gain traction, the future of PV care may prioritize not only efficacy and safety, but also convenience, adherence, and sustained remission—key elements of the Target Opportunity Profile (TOP) that will define market success in the years ahead.

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