

## **Myelofibrosis (MF) - Symptoms and Treatment** <sup>[1]</sup>

### **About myelofibrosis**

Myelofibrosis is a rare and life-threatening blood cancer in which the bone marrow is replaced by scar tissue.<sup>1</sup> It affects approximately 1 in every 100,000 people and has similar survival rates as other malignancies, such as breast cancer and colon cancer.<sup>2-6</sup> In myelofibrosis, bone marrow does not function properly, causing other organs to take over the production of blood cells, including the spleen, which often becomes enlarged.<sup>7</sup> Patients may also suffer debilitating symptoms, including fatigue/weakness, shortness of breath, fever, night sweats, itchy skin, bone/joint pain, abdominal pain and/or weight loss.<sup>1,8</sup> Patients living with myelofibrosis may have a poor quality of life and shortened survival rate.<sup>6,8</sup>

Myelofibrosis is part of a group of related blood cancers known as myeloproliferative neoplasms (MPNs).<sup>1</sup> Approximately 90% of patients with myelofibrosis have mutations that directly or indirectly activate the JAK/STAT signaling pathway, which may explain the development of the disease.<sup>9</sup>

### **Treating myelofibrosis**

The goal of treatment in myelofibrosis is to prolong survival, and if possible, a cure, as per European LeukemiaNet guidelines. However, for most patients, treatment does not offer a cure. If prolongation of survival or a cure is not possible, the treatment goal is to reduce symptoms and improve quality of life.<sup>10</sup>

It is important that patients receive the best available treatment early in the course of their disease to ensure that they achieve the most optimal outcome.<sup>11</sup> If left untreated, patients with myelofibrosis can experience a significantly higher disease burden and may have a lower life expectancy than patients who receive treatment. The disease can also progress more rapidly, eventually becoming fatal.<sup>12,13</sup> Common types of treatments include:

- **Bone marrow or stem cell transplant:** The only potential curative treatment option presented to younger patients whose disease state is more advanced. Because this treatment option involves high levels of chemotherapy or radiation therapy, it is accompanied with harmful side effects and not recommended for older patients.<sup>14</sup>
- **Cytoreductive therapy:** This treatment helps to decrease high platelet and white blood cell counts and treat complications of an enlarged spleen and other myelofibrosis symptoms. Patients who take this treatment alone can experience worsening symptoms or become resistant to or intolerant of treatment.<sup>7</sup>
- **Splenectomy (removal of the spleen):** Procedure carried out to reduce severe pain and the feeling of fullness experienced by patients with myelofibrosis who have an enlarged

spleen. Risks associated with splenectomy include infection and potential long term cardiovascular complications.<sup>7</sup>

- Radiotherapy: This procedure is useful for a small number of patients to reduce spleen size and help alleviate other myelofibrosis-related symptoms. Radiation of the spleen is an option if a splenectomy cannot be performed on a patient.<sup>14</sup>

Patients should discuss with their hematologist the available treatments that best address their individual circumstances.

## Questions to ask your doctor

- What is my prognosis and how will myelofibrosis impact my quality of life?
- What are my treatment choices?
- What are my treatment plan and the treatment goal?
- What are the expected benefits of each kind of treatment?
- What are the risks of each treatment?
- What are the side effects of each treatment?
- How will each treatment affect my daily life?
- Is there a benefit to starting treatment immediately and prior to the development of debilitating symptoms?
- How will we know if the treatment is working?
- How can I assess my symptoms?
- Are there new treatments or clinical trials I should consider?

## Additional resources

Learn more about myelofibrosis:

- [Infographic: Myelofibrosis by the Numbers \(PDF 0.2 MB\)](#) <sup>[2]</sup>

## Spotlight on MPN



Visit [SpotlightonMPN.com](http://SpotlightonMPN.com) <sup>[3]</sup> to learn more about myeloproliferative neoplasms, such as myelofibrosis.

### Footnotes:

1. Leukemia & Lymphoma Society. "Myelofibrosis Facts." Available at: [http://www.lls.org/sites/default/files/file\\_assets/FS14\\_Myelofibrosis\\_Fact%20Sheet\\_Final9.12.pdf](http://www.lls.org/sites/default/files/file_assets/FS14_Myelofibrosis_Fact%20Sheet_Final9.12.pdf) <sup>[4]</sup>. Accessed March 2016.
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12. Mesa RA, Shields Alan, Hare Thomas, et al. Progressive Burden of Myelofibrosis in Untreated Patients: Assessment of Outcomes in Patients Randomized to Placebo in the COMFORT-I study. *Leuk Res.* 2013 May 17; 37: 911– 916.
13. Tefferi A, Lasho TL, Jimma T, et al. One Thousand Patients with Primary Myelofibrosis: The Mayo Clinic Experience. *Mayo Clin Proc.* 2012;87(1):25-33.
14. National Cancer Institute. Myeloproliferative Neoplasm Treatments. Available at: [http://www.cancer.gov/types/myeloproliferative/patient/chronic-treatment-pdq#section/\\_234](http://www.cancer.gov/types/myeloproliferative/patient/chronic-treatment-pdq#section/_234) [8]. Accessed March 2016.

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#### Links

- [1] <https://www.novartis.com/our-focus/cancer/oncology-disease-areas/myelofibrosis>
- [2] <https://www.novartis.com/sites/www.novartis.com/files/myelofibrosis-infographic.pdf>
- [3] <http://www.spotlightonmpn.com/>
- [4] [http://www.ils.org/sites/default/files/file\\_assets/FS14\\_Myelofibrosis\\_Fact%20Sheet\\_Final9.12.pdf](http://www.ils.org/sites/default/files/file_assets/FS14_Myelofibrosis_Fact%20Sheet_Final9.12.pdf)
- [5] <http://www.cancer.org/acs/groups/cid/documents/webcontent/003090-pdf.pdf>
- [6] <http://www.cancer.org/acs/groups/cid/documents/webcontent/003096-pdf.pdf>
- [7] <http://www.cancer.net/navigating-cancer-care/how-cancer-treated/when-first-treatment-doesnt-work>
- [8] [http://www.cancer.gov/types/myeloproliferative/patient/chronic-treatment-pdq#section/\\_234](http://www.cancer.gov/types/myeloproliferative/patient/chronic-treatment-pdq#section/_234)