About Myelofibrosis

What is Myelofibrosis?

Myelofibrosis (MF) is a potentially fatal cancer of the bone marrow impacting the normal production of blood cells.[i]

It is characterised by the buildup of excessive scar tissue in the bone marrow, which interferes with the production of healthy blood cells and can lead to severe anaemia, weakness, fatigue and a condition known as splenomegaly or an enlarged spleen.[ii] In patients with MF, anaemia is commonly associated with poor prognosis, reduced quality of life and shortened overall survival.[iii,iv]

MF is one of a related group of blood cancers known as myeloproliferative neoplasms (MPNs), in which the bone marrow makes too many red blood cells, white blood cells, or platelets.[v]

Patients & prevalence

20,000
MF affects approximately 20,000 patients in the US[vi]

1 in 500,000
MF occurs in 1 in 500,000 people worldwide[vii]

Men & Women
It affects both men and women in equal numbers[vii]

50+
It is usually diagnosed in people over the age of 50, however, MF can occur at any age[ii]

Causes

MF can occur independently (primary MF) or as a result of another bone marrow disorder (secondary MF).[ii]

MF develops when a **genetic mutation occurs in blood stem cells in the bone marrow**, which reproduce and divide, passing the mutation into specialised cells that make up the blood. The cause of this genetic mutation is unknown. MF is not an inherited disease.[viii]

Over time, the production of abnormal cells causes **scarring in the bone marrow**, outpacing the bone marrow's ability to produce enough normal blood cells, resulting in severe anaemia, weakness, bone pain, fatigue **and increased risk of infection**.[ix]

Signs & symptoms

MF usually develops slowly. As disruption of normal blood cell production increases, signs and symptoms may include:[vii]

Symptoms related to low blood cell counts

- Feeling tired, weak or short of breath, due to a low red blood cell count (anaemia)
- Easy bleeding or bruising, due to low platelet count
- Frequent infections, due to a low white blood cell count

Symptoms related to splenomegaly

- Pain or fullness below the ribs on the left side

Constitutional symptoms

- Night sweats
- Fever
- Bone or joint pain
- An enlarged liver
- Pale skin
- Weight loss

About anaemia in MF

40%
At diagnosis, approximately 40% of patients are already anaemic[iii]

24%
At diagnosis, almost a quarter of patients are transfusion dependent[iii,x]

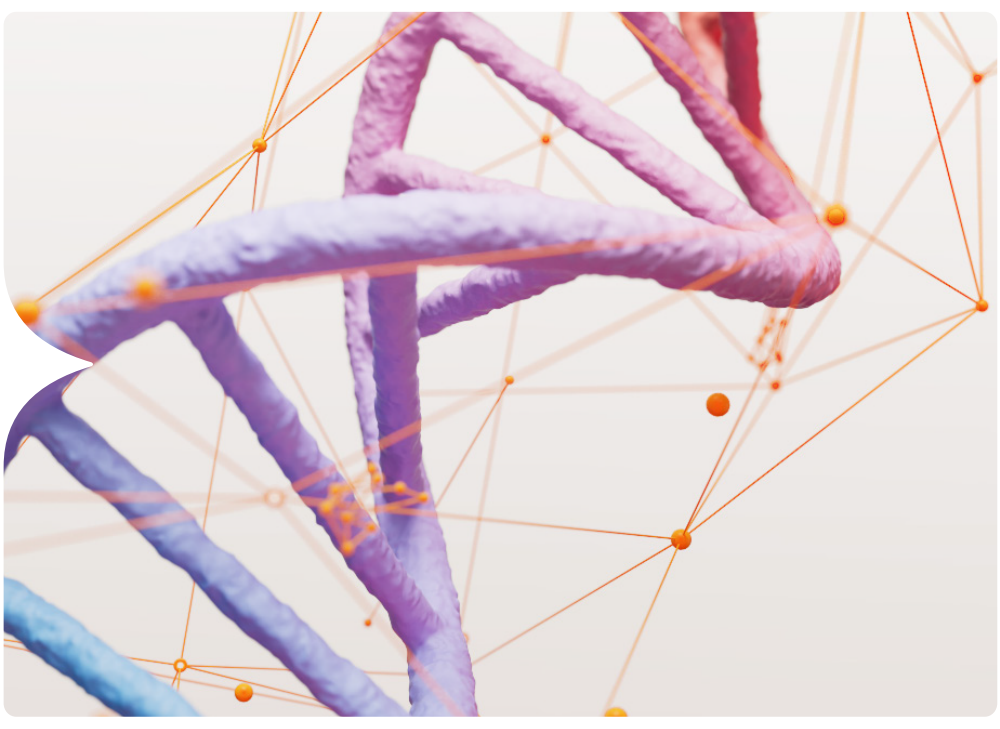
50%
About half of patients require transfusions within the first year following diagnosis[xi]

~100%
Nearly all myelofibrosis patients will eventually develop anaemia[iii]

Diagnosis

Several specialised tests and procedures may be used to diagnose myelofibrosis, including:[xi]

- Blood tests, including a complete blood count (CBC)
- Imaging tests, such as X-rays and MRIs
- Bone marrow exams, such as biopsy and aspiration
- Genetic testing



Treatment and treatment challenges

Since MF remains an incurable disease at this time, treatment goals primarily include symptom relief and reducing the risk of complications.[x] However, currently-approved treatments do not address the full spectrum of symptoms and can negatively impimpact blood cell production, leading to worsening anaemia.[xii]

Over the course of their disease, patients may be treated with one or more of these therapies:[x]

- Blood transfusions
- Hormone therapy
- Immunomodulatory drugs
- Targeted therapy
- Chemotherapy
- Splenectomy (surgical removal of the spleen)
- Radiation therapy
- Stem cell transplant

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