



BUSULFAN

WHAT IS BUSULFAN?

The drug busulfan is known by several names and also goes under the brand name Myleran®. Busulfan is used to treat all three main types of MPNs: polycythaemia vera (PV), essential thrombocythaemia (ET) and myelofibrosis (MF). Busulfan is available as a 2 mg oral white round tablet.

HOW DOES BUSULFAN WORK IN MPNS?

Blood cells originate from stem cells which are master cells that divide and mature into different types of blood cells: red blood cells, white blood cells and platelets. Our bone marrow acts as a factory for division and maturation of these stem cells into blood cells. Each new blood cell contains DNA that carries instructions the cell needs to grow and function. Once matured, blood cells leave the bone marrow and enter our blood stream.

Busulfan works by interfering with the DNA in bone marrow cells. The drug prevents the cells from growing and maturing, which ultimately leads to the death of these cells. When you take busulfan you will be less likely to suffer a blood clot or thrombosis because your blood cell counts will be lower. Busulfan is classed as a chemotherapy treatment because it causes the death of some cells in the body. You may be asked to sign a consent to treatment form before you start taking this drug for this reason.

HOW CAN BUSULFAN TREAT MY MPN?

Busulfan is usually recommended to treat individuals who are unable to tolerate other drugs that treat MPNs. It is usually given as either a single one-time dose or on a daily basis for a short period of time (e.g 4-6 weeks). It is rare for busulfan to be given over long periods of time.

Very close monitoring of your blood counts is essential whenever busulfan is prescribed.

If you have polycythaemia vera (PV) or essential thrombocythaemia (ET)

Busulfan can control your blood counts, which helps to reduce risk of both blood clots and bleeding. When you take this medication you may notice a reduction in MPN-related symptoms such as visual problems, headache, fatigue, tingling in fingers or toes, night sweats, breathlessness, bleeding, gout and itching.

If you have myelofibrosis (MF)

If your spleen is enlarged, taking busulfan can help to reduce its size. Busulfan can also reduce the likelihood that you will experience a blood clot. Reducing the size of your spleen can reduce pain, discomfort, nausea and eating problems that you may experience with an enlarged spleen. You may notice a reduction in other symptoms of myelofibrosis such as night sweats, fevers and fatigue.

ARE THERE ANY DRAWBACKS?

As with any medicine you may experience side effects whilst taking busulfan. Possible side effects are listed in the 'Side effects' section over the page.

You will need frequent blood tests and monitoring whilst taking busulfan as this medication is usually ceased once the blood counts normalise or improve significantly.

A small number of people will develop a resistance to busulfan over a period of years so that the drug is no longer effective. This may require a switch to another treatment.

If you are sexually active during this course of treatment, you need to use a condom. This is to protect your partner in case there is any chemotherapy in semen or vaginal fluids.



ARE THERE ANY SIDE EFFECTS?

Most people taking this drug tolerate it well and have few side effects. The side effects listed below are relevant to individuals taking the low doses of busulfan that are given for MPNs. It is important to inform your doctor or nurse if you are experiencing any of the side effects listed below or any other new symptoms, even if mild.

Common side effects - affect approximately 1-10% of patients taking busulfan:

- **Reduced red blood cells:** If your red blood cells drop too low (anaemia) you may notice that you are becoming breathless and tire easily.
- **Reduced platelets:** If your platelet count drops too low you may experience nose bleeds, bleeding gums when you clean your teeth, a rash of tiny red spots or increased bruising.
- **Reduced white blood cells:** If your white blood cells drop too low you may have an increased risk of developing infections. You may experience a high temperature, fever, shivers or chills

If you experience any of the above side effects you must contact your treating haematologist immediately. Although uncommon, sudden unexpected changes in blood cell levels may occur. It is important to attend appointments to have blood counts checked and discuss symptoms with your haematologist.

Less common side effects – affects approximately one person in 10 up to one person in 100:

- Diarrhoea
- Nausea or vomiting
- Mouth ulcers
- Heart problems
- Inflammation of lungs which can cause breathlessness, cough or raised temperature. Such inflammation may have a gradual onset after long-term use of busulfan
- Dark patches on skin
- Liver disease
- Infertility

Uncommon side effects -affects approximately one person in 100 to one person in 1000:

- Early menopause/periods stopping

Rare side effects -affects approximately one person in 1000 to one person in 10,000:

- Severe drop in all blood cells (pancytopenia)
- Liver abnormalities
- Yellowing of the eyes/jaundice
- Hair loss
- Dry mouth and lips
- Skin reactions/rash and itching/very dry skin
- Mouth ulcers
- Diarrhoea

Very rare side effects -affects less than one person in 10,000:

- Muscle weakness in arms and legs/difficulty in speaking
- Drooping eyelids (myasthenia gravis)
- Breast enlargement in men
- Weight loss
- Weakness

The above list is not fully comprehensive and some other side effects can occur. Please consult the product information sheet (available from your pharmacist) if you are experiencing any side effects and it is important that patients inform their doctor if they do experience any side effects.

Busulfan and cancer

There is a possibility that the use of busulfan over the course of several years may increase the risk of developing cancers including acute leukaemia. This risk is particularly increased when more than one chemotherapy agent has been used, for instance if busulfan is used in combination with, or following drugs such as hydroxycarbamide.



TAKING BUSULFAN

How to take busulfan

- Take either before or after food, in the morning or evening, but at the same time each day
- Wash your hands thoroughly before and after taking the tablets
- Swallow whole with plenty of water
- Do not crush, break or chew tablets

Dosage

Your haematologist will give dosage instructions. He or she may recommend that you take the medication every day over a short period of time, for instance over a limited period of 7 to 14 days, as a single dose, or very rarely on a continuous basis (every day for an indefinite period of time). Be sure to follow the directions precisely.

Non-compliance - It is important that you follow the directions precisely. Failure to do so could result in disease related complications and may increase your risk of vascular and thrombotic complications.

Keeping track

It may be helpful to keep a record or diary to remember when to take your tablets and to record any side effects.

Will I need follow up?

You will need more frequent blood tests while on busulfan to determine how your body is responding to the medication. Your kidney and liver function may also be monitored with blood tests.

Storage and disposal of busulfan

- Store in a dry place at room temperature
- Keep your medication in its original packaging
- Busulfan can be dangerous to others. Keep your tablets in a secure location, well out of the reach of children and pets
- Return any unused tablets to your local pharmacy or hospital. Do not dispose of them in the bin or flush them down the toilet
- If you use a pill box or Dosette box the busulfan must be kept separate from other medications

INTERACTIONS WITH OTHER MEDICATIONS OR VITAMINS, HERBAL SUPPLEMENTS OR REMEDIES?

Whenever you take busulfan (or in fact any medication), always provide names of other medicines prescribed for you as well as any over-the-counter medications (e.g vitamins, herbal supplements or remedies) to the health care team who are treating you. It can be very helpful to carry a list of the names and dosages of all your medicines to show to your haematologist at appointments.

[The MPN AA has a wallet card which provides for medications to be listed.](#)

Medications that may interact with busulfan include:

- Cyclophosphamide
- Itraconazole
- Metronidazole
- Phenytoin
- Chemotherapy drugs

WHAT SHOULD I EXPECT?

How fast does it work and how will I feel?

Busulfan may take a couple of weeks to begin having an effect on your blood cells. You will probably not feel any benefits until your counts are under control. As your blood counts reduce you may notice a reduction in your MPN-related symptoms. Most people tolerate the drug and have relatively few side effects.

Will I need follow up?

You will need more frequent blood tests while on busulfan to determine how your body is responding to the medication. Your kidney and liver function may also be monitored with blood tests.



WHAT IF I HAVE OTHER MEDICAL CONDITIONS?

Busulfan should be used under supervision if you have now or have had any of the following conditions:

- Allergies to any of the ingredients in the medicine (these will be listed on the information leaflet that came with your tablets)
- If you have ever experienced seizures, fits or epilepsy (even if in the past)
- If you are planning pregnancy
- If you have radiation therapy planned
- If you have lung disease

If you think you may have one of these conditions please discuss this with your doctor.

FREQUENTLY ASKED QUESTIONS

Can I eat and drink normally?

Yes. We recommend that you eat a normal, healthy diet and drink plenty of water.

Can I drink alcohol?

While it is safe to drink alcohol in moderation whilst taking busulfan, Australian NHMRC guidelines state that for healthy women and men drinking no more than two standard drinks on any day reduces your risk of harm from alcohol-related disease. Alcohol can cause dehydration, and it is important to avoid becoming dehydrated if you have an MPN. Please ask your nurse or doctor if you require more information regarding alcohol consumption.

What if I want to have a child?

- It is strongly recommended that you use contraception whilst taking busulfan, because this medication can be harmful to a developing foetus
- When planning to conceive or to father a child, you should stop taking busulfan for a period of at least three months to allow the drug to clear from your system before trying to conceive
- It is imperative to discuss your plans together with your haematologist prior to becoming pregnant or fathering a child. Your haematologist can recommend treatment options for you that will not cause harm to your developing foetus and will increase your chance of a successful pregnancy
- If you or your partner becomes pregnant while taking this drug please contact your haematologist immediately for further advice.

Can I breastfeed while taking busulfan?

It is strongly recommended against breastfeeding whilst taking busulfan. Busulfan is a very strong drug that inhibits blood cell development. It can be secreted in breast milk and this may affect your baby's development.

Can I drive?

Busulfan does not usually cause drowsiness that impacts upon your ability to drive, however if you are feeling drowsy or fatigued for any reason do not drive.

Do I need to take any special precautions?

Your skin may be more sun-sensitive whilst you are taking busulfan. It is recommended that you protect your skin by limiting sun exposure, using SPF 30+ sunscreen and wearing protective clothing and a hat.

Can I have vaccinations such as the flu and COVID-19 jabs while taking busulfan?

Yes, you can have most vaccinations including the flu and COVID-19 vaccines whilst taking busulfan. Some vaccinations are live vaccines and these should not be taken with busulfan. It is important you tell the person giving you the vaccine that you are taking busulfan so they can verify it is safe for you to be vaccinated.

COVID-19 - Can I take antivirals while on busulfan?

If you test positive for COVID-19, you may be eligible for antiviral treatments. The antiviral most suitable for you will depend on what other medications you are taking and your kidney function. Treatment must be commenced within 5 days of symptom onset, or as soon as possible if you have no symptoms but test positive. Please contact your GP or haematologist to arrange antiviral therapy immediately if required.



WHAT TO DO IF...

You have taken too much medicine/someone else has taken your medicine

If you have taken extra tablets or if another person has taken your busulfan please contact your haematologist.

You were sick shortly after taking your tablets

If this happens just once, take your next dose as usual. If you are sick over a few days contact your haematologist.

You forget to take a dose

If you have forgotten to take a dose, do not take any extra but take your next dose as normal. If you have forgotten to take a few doses, start taking them again and contact your haematologist.

You need to have a medical procedure or operation

You may occasionally be required to adjust the dose of busulfan if you need an operation. It is important that you inform the doctor or dentist planning the procedure or operation that you are taking busulfan and that they co-ordinate your treatment with your haematologist. We always recommend that you inform your haematologist if you have any procedures or operations planned.

You feel anxious about taking busulfan?

If you have concerns, please discuss this with your haematologist or GP.

WHAT IF I DO NOT WANT TO TAKE THIS MEDICATION?

Whether or not to take busulfan is entirely your decision. If you feel uncertain or prefer not to take this medication, you can choose not to take it, but it is essential that you discuss this decision with your haematologist. If you decide not to take busulfan or if you elect to stop after you begin treatment, it is important to inform your haematologist of your decision. He or she can recommend alternatives or other suggestions if necessary to safeguard your health.

WHAT CAN I DO TO HELP MYSELF?

If you have an MPN it's important to take good care of yourself. Many things help you feel better.

- Good nutrition is important. Eat a balanced diet including lots of fresh fruit and vegetables, lean protein and whole grains. Drink plenty of water and be careful to prevent dehydration by avoiding excessive alcohol and caffeinated drinks.
- Maintain a normal weight and maintain your muscle mass to help keep your cholesterol and blood sugar within normal limits.
- Exercise is very beneficial for MPN patients and helps fight fatigue. Be sure to check with your GP and haematologist before launching on any new programme and start slowly and gently if you have not exercised before.
- Stop smoking. Ask your GP if you need help.
- Consider wellness activities such as yoga, aerobic activity, strength training, meditation, massages, support groups, improved nutrition etc – an international study of hundreds of MPN patients showed wellness activities had a pattern of decreased levels of symptom burden, fatigue, depression, and a higher quality of life for MPN patients. (Survey of integrative medicine in myeloproliferative neoplasms – the SIMM study).

More information on living well with an MPN is available at www.mpnallianceaustralia.org.au

MAKING THE ADJUSTMENT

It can be disconcerting to start a new medication or find you must increase your dosage. You may feel that your MPN is getting worse or that you are at greater risk of serious medical problems. You may also feel concerned about the long and short term risks of taking this medication. It is normal to feel this way and many people with MPNs have had similar experiences. It's worth bearing in mind that many people with MPNs have a long life expectancy, and that the treatments are very effective at controlling blood cell production. You may want to seek support via one of the sources listed on the MPNAA website under the Australian support section.

GENERAL ADVICE This leaflet is intended to give you general information about taking busulfan, or as a reference for people already taking this medication. It is important that in addition to this leaflet you read the product information available from your pharmacist about busulfan and discuss any concerns with your general practitioner or haematologist.