
Midostaurin for Acute Myeloid Leukaemia (AML)

**A Guide for
Patients**

Introduction

Midostaurin (sold by Novartis under the name Rydapt®) is a multi-targeted protein kinase inhibitor. Midostaurin has been approved for the treatment of adult patients with AML.

This booklet is designed to provide you with information about midostaurin as a treatment for AML.

If you need specific advice or have any questions about this treatment, please contact your medical team or Clinical Nurse Specialist (CNS).

Booklet compiled by our Patient Information Writer, Isabelle Leach and peer reviewed by a medical professional who is specialised in leukaemia, Dr Emmanouil Nikolousis. We are also grateful to a leukaemia patient, Julie Quigley, for her valuable contribution as a reviewer.

If you would like any information on the sources used for this booklet, please email communications@leukaemiacare.org.uk for a list of references.

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About Leukaemia Care

Leukaemia Care is a national charity dedicated to ensuring that people affected by blood cancer have access to the right information, advice and support.

Our services

Helpline

Our helpline is available 9.00am - 10.00pm on weekdays and 9.00am - 12.30pm on Saturdays. If you need someone to talk to, call **08088 010 444**

Nurse service

We have two trained nurses on hand to answer your questions and offer advice and support, whether it be through emailing **nurse@leukaemicare.org.uk**, over the phone on **08088 010 444** or via LiveChat.

Patient Information Booklets

We have a number of patient information booklets like this available to anyone who has been affected by a blood cancer. A full list of titles – both disease specific and general information titles – can be

found on our website at **www.leukaemicare.org.uk/support-and-information/help-and-resources/information-booklets/**

Support Groups

Our nationwide support groups are a chance to meet and talk to other people who are going through a similar experience. For more information about a support group local to your area, go to **www.leukaemicare.org.uk/support-and-information/support-for-you/find-a-support-group/**

Buddy Support

We offer one-to-one phone support with volunteers who have had blood cancer themselves or been affected by it in some way. You can speak to someone who knows what you are going through. For more information

on how to get a buddy call **08088 010 444** or email **support@leukaemicare.org.uk**

Online Forum

Our online forum, **www.healthunlocked.com/leukaemia-care**, is a place for people to ask questions anonymously or to join in the discussion with other people in a similar situation.

Patient and carer conferences

Our nationwide conferences provide an opportunity to ask questions and listen to patient speakers and medical professionals who can provide valuable information and support.

Website

You can access up-to-date information on our website, **www.leukaemicare.org.uk**, as well as speak to one of our care

advisers on our online support service, LiveChat (9am-5pm weekdays).

Campaigning and Advocacy

Leukaemia Care is involved in campaigning for patient well-being, NHS funding and drug and treatment availability. If you would like an update on any of the work we are currently doing or want to know how to get involved, email **advocacy@leukaemicare.org.uk**

Patient magazine

Our free quarterly magazine includes inspirational patient and carer stories as well as informative articles by medical professionals. To subscribe go to **www.leukaemicare.org.uk/communication-preferences/**

What is Midostaurin?

Midostaurin (sold by Novartis under the name Rydapt®) is a multi-targeted protein kinase inhibitor. Protein kinase inhibitors block the protein kinase enzymes that are involved with cell growth, and work by preventing the growth of cancer cells.

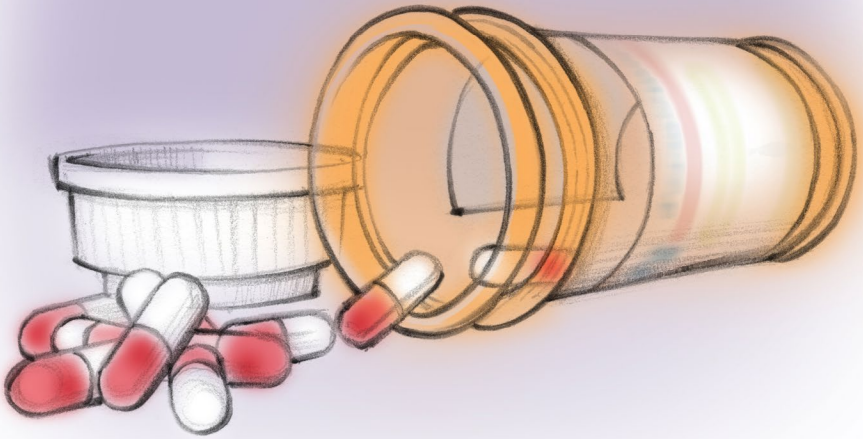
Midostaurin is indicated for the following patients:

- Adult patients with newly-diagnosed acute myeloid leukaemia (AML) who have the FLT3 mutation.
- Adult patients with aggressive systemic mastocytosis (ASM), systemic mastocytosis with associated haematological neoplasm (SM-AHN), or mast cell leukaemia (MCL).

In April 2017, midostaurin was approved by the Food and Drug Administration in the United States for use in combination with chemotherapy for newly-diagnosed AML patients who have the specific gene mutation, FLT3.

In September 2017, midostaurin was approved in the European Union for the treatment of adult patients with newly-diagnosed AML who are FLT3 mutation-positive. Midostaurin was launched in the UK in April 2018.

On 4th May 2018, the National Institute for Health and Care Excellence (NICE) recommended the use of midostaurin in combination with daunorubicin and cytarabine for induction therapy for AML, and in combination with high-dose cytarabine for consolidation therapy to destroy any remaining cancer cells. For patients who show a complete response to this initial treatment, NICE also recommends midostaurin on its own for maintenance therapy to lower the chance of the cancer coming back (this is called a relapse).



Who receives Midostaurin?

Midostaurin has been approved for the treatment of patients with AML, MDS, ASM, SM-AHN, or MCL. It is only indicated for adult patients because the safety and efficacy of midostaurin in children and adolescents below 18 years has yet to be established.

When used for the treatment of AML, midostaurin is indicated for newly-diagnosed patients with the FLT3 mutation who are aged less than 60 years of age. There is little experience of the use of midostaurin in patients with AML aged 60-70 years, and none in patients over 70 years. For patients aged 60 years or older, midostaurin should be used only in patients who can tolerate intensive induction therapy to achieve remission of the AML, and patients who do not have any other serious illnesses.

If you wish to have further information on AML, please view our collection of patient information booklets that are available on our website at www.leukaemiacare.org.uk

How is Midostaurin administered?

The daily dose of midostaurin is normally determined by your consultant according to the stage of the AML and your symptoms. However, the usual daily dose of midostaurin for patients with AML is 50mg (2 capsules) twice daily (12 hours apart).

For patients with ASM, SM-AHN or MCL, the usual daily dose of midostaurin is 100mg (4 capsules) twice a day.

Depending on the response to midostaurin, the daily dose may be lowered, or the treatment temporarily interrupted.

You should follow the steps below when taking midostaurin:

- Take midostaurin at the same time each day to help you to remember to take your treatment.
- Midostaurin should be taken with food.
- Take midostaurin twice a day at about 12-hour intervals (for example, at breakfast and with your evening meal).
- Swallow the capsules whole with a glass of water. Do not open, crush or chew them to make sure you get the full dose and avoid any unpleasant taste of the capsule content.
- If taken with chemotherapy, it is very important to follow your doctor's instructions on how to take midostaurin.
- If you bring up the capsules after you swallow them, do not take any more capsules and wait until your next scheduled dose.

What are the side effects of Midostaurin?

You may get some side effects when you first start taking midostaurin. They are usually mild or moderate and will generally disappear after a few weeks of treatment.

Some people have very few side effects, whereas other people experience more. Midostaurin is still working even if you don't have any side effects. Your doctor can prescribe medicines to help with particular side effects if they become troublesome.

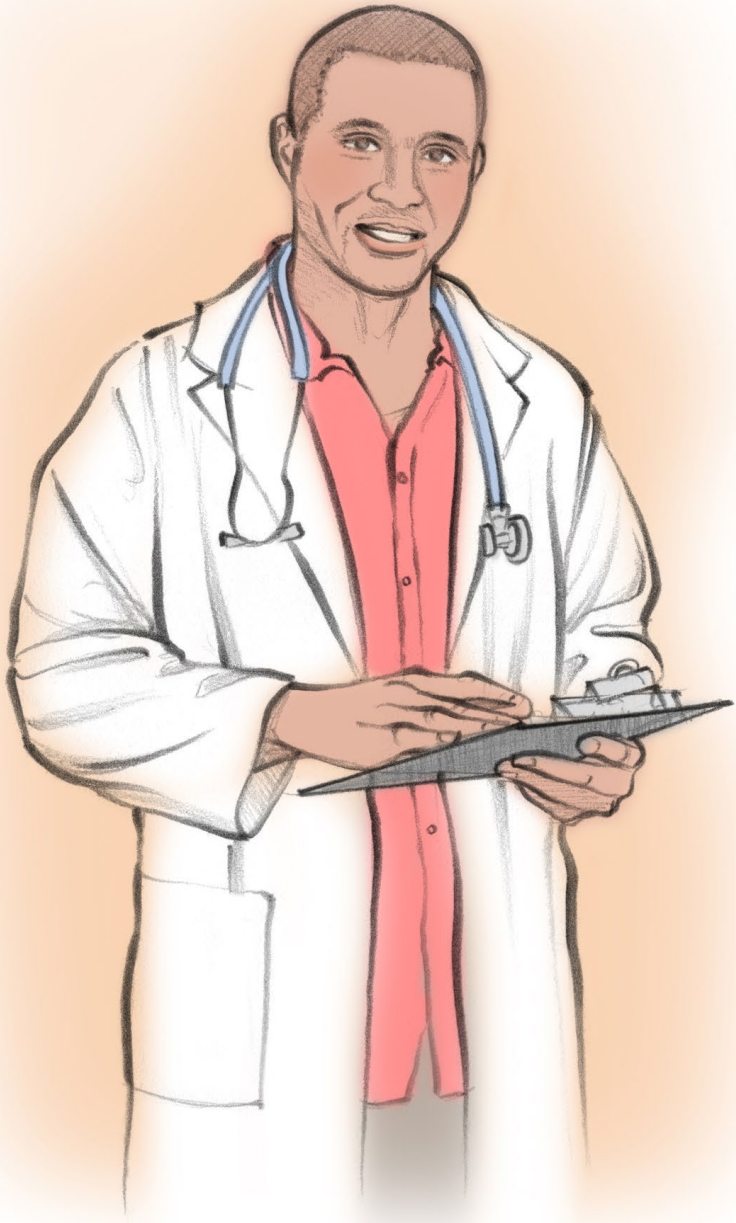
Common side effects

- Nausea and vomiting (both are less common if you take midostaurin with food)
- Diarrhoea
- Headache
- Fever
- Inflammation of the mucous membranes (transparent red skin that lines, for example, your mouth, nose or ears)
- Nose bleeds, bleeding gums, or reddish purple, flat, pinhead spots under the skin (petechiae)

- Upper respiratory tract infections such as colds, coughs, and sinusitis

Contact your doctor if you have any of the following side effects as you may need treatment:

- Raised temperature (over 38°C/100.4°F) depending on the advice given by your consultant
- Bruising or bleeding that you can't explain such as nose bleeds, bleeding gums, blood spots, rashes (your levels of platelets may be low)
- Eye pain, dry or watery eyes (you may have changes in vision)
- Feeling very tired or short of breath (you may have anaemia)



What happens if Midostaurin doesn't work for me?

Your doctor will closely monitor how well midostaurin is working by taking regular blood tests.

If midostaurin isn't working or stops working for you, your consultant will decide the best option to take with you. A different protein kinase inhibitor or another type of cancer medicine for your AML could be used.

Leukaemia Care offers nationwide support groups for people affected by a diagnosis of a blood or lymphatic cancer. Visit www.leukaemiacare.org.uk, or call **08088 010 444**, to find out more and to find a group near you.



Glossary

Acute Myeloid Leukaemia (AML)

Acute Myeloid Leukaemia (AML) is a type of blood cancer that starts from young white blood cells called granulocytes or monocytes in the bone marrow.

Anaemia

A medical condition in which the red blood cell count or haemoglobin is less than normal.

Chemotherapy

A type of cancer treatment that uses one or more drugs with a powerful chemical to kill growing cancer cells.

Clinical trial

A highly regulated research study, which assigns patients and non-patients to participate in the study and to evaluate the efficacy of a drug or a combination of drugs.

Leukaemia

A cancer of the blood with many different subtypes. Some forms are acute (develop quickly) and others are chronic (develop slowly). Leukaemia is an excess number of abnormal cells in the blood, usually white blood cells, which stop the bone marrow working properly.

Platelets

A disc-shaped element in the blood. During normal blood clotting, the platelets clump together (aggregate).

Relapse

The return of a disease or the signs and symptoms of a disease after a period of improvement.

Tell us what you think!

If you would like to give us some feedback about this patient information booklet, please hover over the code to the right using your phone or tablet's camera. Click the link as it appears and this will take you to a short web form to fill in.

Suitable for Android, iPhone 7 and above.



Useful contacts and further support

There are a number of helpful sources to support you during your diagnosis, treatment and beyond, including:

- Your haematologist and healthcare team
- Your family and friends
- Your psychologist (ask your haematologist or CNS for a referral)
- Reliable online sources, such as Leukaemia Care
- Charitable organisations

There are a number of organisations, including ourselves, who provide expert advice and information.

Leukaemia Care

We are a charity dedicated to supporting anyone affected by the diagnosis of any blood cancer.

We provide emotional support through a range of support services including a helpline, patient and carer conferences, support group, informative website, one-to-one buddy service and high-quality patient information. We also have a nurse on our help line for any medical queries relating to your diagnosis.

Helpline: **08088 010 444**
www.leukaemicare.org.uk
support@leukaemicare.org.uk

Bloodwise

Bloodwise is the leading charity into the research of blood cancers. They offer support to patients, their family and friends through patient services.

020 7504 2200
www.bloodwise.org.uk

Cancer Research UK

Cancer Research UK is a leading charity dedicated to cancer research.

0808 800 4040
www.cancerresearchuk.org

Macmillan

Macmillan provides free practical, medical and financial support for people facing cancer.

0808 808 0000
www.macmillan.org.uk

Maggie's Centres

Maggie's offers free practical, emotional and social support to people with cancer and their families and friends.

0300 123 1801
www.maggiescentres.org

Citizens Advice Bureau (CAB)

Offers advice on benefits and financial assistance.

08444 111 444
www.adviceguide.org.uk

Leukaemia Care is a national charity dedicated to providing information, advice and support to anyone affected by a blood cancer.

Around 34,000 new cases of blood cancer are diagnosed in the UK each year. We are here to support you, whether you're a patient, carer or family member.

Want to talk?

Helpline: **08088 010 444**

(free from landlines and all major mobile networks)

Office Line: **01905 755977**

www.leukaemiacare.org.uk

support@leukaemiacare.org.uk

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One Birch Court,
Blackpole East,
Worcester,
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Registered charity
259483 and SC039207

Leukaemia Care
YOUR Blood Cancer Charity

