What is New for Lymphoma and CLL *Know Your Treatments*

EDITION 1 JANUARY 2017

Designed to support patients and their families through the various phases of their lymphoma journey.

www.lymphoma.org.au

Lymphoma Australia

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Acknowledgements
Lymphoma Australia would like to give a very special thank you to sponsors, hospitals, doctors, nurses, patients, families and friends that make our educational resources possible. Taking the fear of the unknown out of the Lymphoma journey can make a huge difference.

Lymphoma Australia
Raising awareness. Giving support. Searching for a cure.

Lymphoma Australia is as an incorporated charity and is providing education, support and awareness across Australia.

Lymphoma Australia relies on grants, fund raising initiatives, donations and sponsorships to continue our work, support research, and to develop our free resources for patients, hospitals and cancer clinics. We are a member of the Lymphoma Coalition which provides global support to the millions of people around the world living with cancer of the lymphatic system. Lymphoma Australia is endorsed with deductible gift recipient (DGR) status from the ATO and receives no government funding.

The feather in our logo symbolises a guardian angel because our founding patient group wanted to make sure no one felt alone in their lymphoma journey. It is also a symbol of hope to those with lymphoma, and reflects the fact that people are searching for cures for this group of cancers everyday.

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What is New for Lymphoma and CLL?

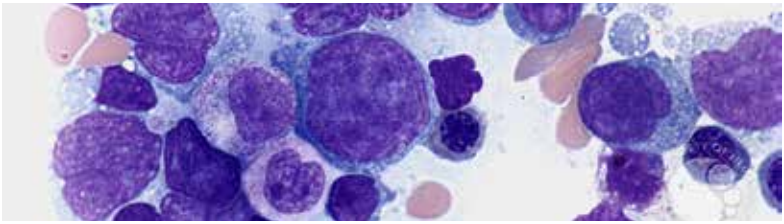
There have been many exciting advances in the field of Lymphoma and CLL in the way that we are able to diagnose, monitor and treat these diseases. Up until early 2000, treatment options for Lymphoma and CLL were mostly limited to chemotherapy and radiotherapy. Now when people are diagnosed with Lymphoma and CLL there are newer more targeted treatment options that are also available. These newer treatment options may be used on their own or in combination with chemotherapy and radiotherapy. The purpose of this booklet is to help you understand these new advances in the treatment of Lymphoma and CLL and what they mean. As there are now over 100 subtypes of lymphoma this booklet will refer to lymphoma in general and mention specific subtypes when required. Lymphoma is currently the 5th most common cancer in Australia with more then 5000 diagnoses every year and CLL has another 1200 every year. CLL is now recognised as a subtype of lymphoma bringing the total diagnoses a year to over 6200. In this book we will reference 'lymphoma' but this means all subtypes of lymphoma including CLL.

Diagnosis and Monitoring

When a doctor suspects that someone has a lymphoma, they need to get a biopsy of a piece of tissue from where they suspect the lymphoma may be to look at this tissue under the microscope to confirm a diagnosis. This biopsy may be from a lymph node or any other body organs such as bowel, bone, skin or blood. It is critical to obtain this

information to diagnose which specific subtype a person has to tailor the treatment to that particular lymphoma. As there are over 100 different subtypes of lymphoma there are many different treatment types.

The most common forms of biopsy used to diagnose lymphoma are a core biopsy or excisional biopsy as rarely does a biopsy using a fine needle aspirate give a sufficient result. The tissue from the biopsy will be analysed by a specialist pathologist who is experienced in diagnosing lymphoma to determine which of the 100 plus subtypes is. A pathologist is a doctor who specialises in interpreting laboratory tests and evaluating cells, tissues, organs and blood to diagnose a disease.



Biopsies to obtain tissue may be repeated to help guide treatment options or determine if there has been a relapse of the lymphoma. Further tests that are required at diagnosis may include PET scan, CT scan, bone marrow biopsy and blood tests to assess where the lymphoma is and baseline tests of some organ functions such as the heart and lungs may also be required.

Know Your Treatments

There are many treatments for lymphoma and this book will go through what is new but it is also important to understand that when having treatment for these diseases, you may start with standard chemotherapy, radiotherapy, before moving onto some of the new treatments mentioned in this book.

These different treatments can be used alone or in combination. Most blood cancers are treated with chemotherapy and/or radiotherapy. Other treatments, such as hormone therapy and immunotherapy, can also be used. Sometimes targeted therapy is used instead of or with chemotherapy.

To understand some of these treatments please visit the Lymphoma Australia website www.lymphoma.org.au

Types of Treatments

Treatment	Description
Chemotherapy	The use of cytotoxic medicines to treat cancer cells or by slowing their growth
Radiotherapy	The use of radiation, usually free x-rays, to kill cancer cells or injure them so they can no longer grow and multiply
Immunotherapy	The use of medications to activate the immune system to attack cancer cells.
Targeted Therapy	Treatment that attacks specific particles within cells that allow cancer to grow. Some immunotherapy and hormone medicines are types of targeted therapy.

New Medicines

As researchers have learned more about the changes in cells that cause lymphoma, they have been able to develop new medicines that specifically target these changes. These medicines are often referred to as targeted therapies and immunotherapies. As they work differently to chemotherapy they may have different side effects.

For a new medicine to be routinely used in Australia it needs to be approved by the Therapeutic Goods Administration (TGA) and reimbursed by the Pharmaceutical Benefits Scheme (PBS). PBS approval specifies which subtype of lymphoma the medicine can be prescribed for and indicates whether this medicine can be given when first diagnosed as the first round of treatment or if it is used after the disease has come back (relapsed) this is called or second or third line treatment. Only some of the new medicines listed in this booklet are PBS approved at time of printing however you may still be able to access these therapies through clinical trials or other access programs and self-funding options from the pharmaceutical companies discussed later on in this booklet. Please use the specified links in the 'Where to go for further information' section at the end of this booklet for updated approval information and speak to your specialist doctor about whether these new therapies may be useful for you and your lymphoma.

The following is a guide to some new medicines for lymphoma and how they work. As these new medicines

become more widely available and more information on how best to use them comes out they will be used more to treat lymphoma.

This resource is not intended to be a comprehensive list of new therapies and clinical trials that are available in Australia to treat lymphoma but as a guide to prompt your thinking and interactions with your specialist doctor and treating team about what treatments may be appropriate for you.

Overview of Therapies

Targeted therapies are medicines that block the growth and spread of lymphoma by interfering with specific molecules that are involved in the growth, progression and spread of lymphoma. The following table outlines the main differences between targeted therapies and chemotherapy.

Targeted Therapy	Chemotherapy
Works on specific molecular targets within the cells associated with cancers	Works on all normal and cancerous cells that are rapidly dividing. Chemotherapy is unable to tell the difference between the two different cells
Specifically chosen or designed to interact with a particular type of cell	Identified because they kill cells
They block cancer cells being able to divide and multiply this can be referred to as 'cytostatic'.	Are 'cytotoxic' as they kill cancer cells and other normal healthy cells

The development of targeted therapies requires the identification of good cell molecular targets that play a role in lymphoma cell growth and survival. One way to look at this is to identify good molecular targets by comparing the amounts of individual proteins in lymphoma cells with those in normal cells. Proteins that are present in

lymphoma cells but not in normal cells are potential targets, especially if they are known to be involved in lymphoma cell growth or survival. Another approach to identify potential target cells is to determine whether the lymphoma cells produce mutated proteins mutated proteins that drive the progression of lymphoma drive the progression of the lymphoma.

Once a target protein has been identified the next step is to develop a therapy that interferes with the cells ability to grow and survive. The targeted therapy could then work on the lymphoma cells and stop the lymphoma cells from multiplying or even surviving.

The following are examples of targeted therapies and newly approved chemotherapy medicines that are being used. Please ask your specialist doctor if any of these new medicines may be useful for treating your lymphoma subtype.

Table of New Medicines by Subtype	
Subtype of Lymphoma	**Medicine**
Relapsed or refractory systemic Anaplastic Large Cell Lymphoma	Brentuximab Vedotin
Chronic Lymphocytic Leukaemia (CLL)	Bendamustine Ibrutinib Idelalisib Obinutuzumab Venetoclax
Cutaneous T Cell Lymphoma (CTCL)	Romidepsin Vorinostat
Diffuse Large B Cell Lymphoma (DLBCL)	Lenalidomide
Follicular Lymphoma (FL)	Obinutuzumab (rituximab refractory) Lenolidomide Idelalisib
Relapsed or refractory Hodgkin's Lymphoma	Brentuximab Vedotin Lenolidomide
Mantle Cell Lymphoma (MCL)	Bendamustine Ibrutinib Lenolidomide
Peripheral T Cell Lymphoma (PTCL)	Romidepsin

Bendamustine (Ribomustin ™)

Bendamustine is a chemotherapy medicine that belongs to the group of agents used to treat indolent lymphomas such as Follicular Lymphoma and CLL. Bendamustine works by damaging the DNA in the lymphoma cell so that it is unable to multiply and when it loses the ability to multiply it dies. Unfortunately, this chemotherapy does not know the difference between the lymphoma cells and normal healthy cells in the body which is why you can get certain side effects of the healthy cells in the body.

Bendamustine is given by intravenous infusion which means it goes directly into the vein through a needle in your arm or through a central line if you have one in place. Bendamustine is given every four weeks either alone or in combination with rituximab depending on your subtype of lymphoma. Bendamustine in combination with rituximab has been found to be more effective and less toxic than using the standard chemotherapy combination of CHOP with rituximab for certain subtypes of lymphoma.

Bendamustine may have the following side effects:

- Increased risk of infections
- Lowering of blood counts
- Nausea

I was diagnosed with Follicular Lymphoma 5 years ago and was given Bendamustine and Rituximab. I didn't even lose my hair and I have been cancer free ever since then" – *Laura*

- Skin rash

Bendamustine is approved by the TGA and listed on the PBS for the following indications;

- CLL as a first line treatment
- Indolent Non Hodgkin's Lymphoma as a first line treatment in combination with rituximab
- Mantle Cell Lymphoma as a first line treatment in combination with rituximab in patients ineligible for autologous stem cell transplant

Brentuximab Vedotin (Adcetris ™)

Brentuximab vedotin is an immunotherapy and chemotherapy combined into one medicine used to treat Anaplastic Large Cell Lymphoma and Hodgkin Lymphoma with a promising outlook in treatment of other subtypes of lymphoma. Brentuximab vedotin works by targeting a particular marker on the cell which is referred to as CD30. It works by destroying particular areas in the cell and this causes the cell to die.

Brentuximab vedotin is given by intravenous infusion which means it goes directly into the vein through a needle in your arm or through a central line if you have one in place. Brentuximab vedotin is given every three weeks either alone or in combination with chemotherapy depending on your subtype and disease status of lymphoma.

Brentuximab vedotin has the following side effects;

- Lowering of blood counts
- Nausea
- Peripheral neuropathy (numbness and tingling of the hands and feet)
- Tiredness

We are so grateful our son was able to receive this new treatment when his lymphoma was not responding to any other treatment" – ***Paula with son Mitch***

Brentuximab Vedotin is approved by the TGA for the following indications:

- Relapsed or refractory systemic Anaplastic Large Cell Lymphoma
- Relapsed or refractory Hodgkin's Lymphoma

Brentuximab Vedotin is listed on the PBS for the following indications:

- Relapsed or refractory systemic Anaplastic Large Cell Lymphoma
- Relapsed or refractory Hodgkin's Lymphoma

Ibrutinib (Ibruvica ™)

Ibrutinib is an immunotherapy medicine used in treatment of CLL, SLL and Mantle Cell Lymphoma with a promising outlook in treatment of other subtypes of lymphoma. Ibrutinib works by blocking activity of a specific protein called Bruton's tyrosine kinase (BTK). By blocking this protein BTK ibrutinib may help move abnormal cells out of their nourishing environments in the lymph nodes, bone marrow and other organs where is gets excreted. BTK is also found in normal healthy B cells so this action of blocking may cause side effects.

Ibrutinib is an oral tablet that you take every day.

Ibrutinib may have the following side effects;

- Bruising
- Diarrhoea
- Fever
- Irregular heart beat (Atrial fibrillation)
- Lowered blood counts
- Muscle and bone pain

- Nausea
- Rash
- Tiredness

Some patients may be able to enrol in a clinical trial testing ibrutinib. Ask your doctor if there are any clinical trials suitable for you.

IMBRUVICA (Ibrutinib) is TGA listed for the treatment of:

- Adult patients with CLL/SLL who have received at least one prior therapy, or adult patients with previously untreated CLL/SLL
- Patients with CLL/SLL with deletion 17p
- Patients with MCL who have received at least one prior therapy
- Adult patients with Waldenstrom's macroglobulinaemia (WM) who have received at least one prior therapy, or in first-line treatment for patients unsuitable for combination chemo-immunotherapy

Idelalisib (Zydelig ™)

Idelalisib is a targeted therapy medicine that has a protein called P3K (phosphatidyloinositil 3 kinase) used in the treatment of Follicular Lymphoma (FL) and CLL. Idelalisib works by stopping the P3K enzyme in the body which is over active in some B cell lymphomas driving the cells to multiply, move to other areas and survive.

Idelalisib is an oral tablet you take every day.

Idelalisib may have the following side effects;

- Altered liver function tests
- Diarrhoea, fever and rash

Idelalisib is approved by the TGA for the following indications;

- Follicular Lymphoma as a third line treatment
- CLL/SLL as a first line treatment with a 17p deletion or TP53 mutation
- CLL/SLL as a second line treatment in combination with an anti CD20 immunotherapy medicine

Being on this treatment meant that I could still go to work and look after my children which has the made the world of difference for me and my family" – *Adele*

Lenalidomide (Revlimid ™)

Lenolidomide is an immunotherapy medicine used in treatment of CLL, SLL and Mantle Cell Lymphoma with a promising outlook in treatment of other subtypes of lymphoma in the future. Lenolidomide works by activating the body's immune system to target and kill the lymphoma cells and helps prevent the lymphoma cells from growing by cutting off their blood supply that they require to exist.

Lenalidomide is an oral tablet that you take every day.

Lenalidomide may have the following side effects;

- Constipation
- Diarrhoea
- Fever
- Itching
- Lowered blood counts
- Nausea
- Rash
- Swelling of the limbs and skin

- Tiredness

Lenolidomide is approved by the TGA for the following indications;

- Mantle Cell Lymphoma as a second line treatment

Other types of lymphoma are currently being reviewed for listing on the PBS and access to lenalidomide can also be found in clinical trials in Australia and by access programs through the pharmaceutical company.

Nivolumab (Opdivo ™)

Nivolumab is an immunotherapy medicine that is currently being used in the treatment of Relapsed and Refractory Hodgkin's Lymphoma after autologous stem cell transplant (ASCT) and treatment with brentuximab vedotin. Nivolumab enables the immune system to recognise and attack cancer cells, which often find ways to disguise themselves as normal cells or 'switch off' the immune system to avoid detection. Nivolumab is known as a checkpoint inhibitor because it blocks an immune-suppressing protein called PD1.

Nivolumab is given by intravenous infusion which means it goes directly into the vein through a needle in your arm or through a central line if you have one in place. Nivolumab is given every two weeks either alone or in combination with chemotherapy depending on your subtype of lymphoma.

Nivolumab may have the following side effects;

- Diarrhoea
- Inflammation and joint pain
- Nausea
- Tiredness
- Skin rash

Nivolumab is generally a well tolerated treatment but side effects can be serious if not treated early. Patients should notify their doctor about a possible side effect as soon as possible.

At the time of publication, nivolumab is approved by the TGA for advanced melanoma, lung cancer and kidney cancer and is listed on the PBS for advanced melanoma. Eligible patients may be able to access nivolumab through a clinical trial or by access programs through the pharmaceutical company.

Obinutuzumab (Gazyva ®)

Obinutuzumab is an immunotherapy medicine used in the treatment of CLL with a promising outlook in treatment for other subtypes of lymphoma in the future. Obinutuzumab works by binding to a protein on the surface of certain white cells known as B lymphocytes and by bonding to this protein the abnormal growth in the B lymphocyte is stopped therefore decreasing the number of CLL cells in the body. Once attached to the CD20 protein Obinutuzumab helps the immune system destroy the CLL cells and also destroys the CLL cells on its own by directly killing them.

Obinutuzumab is the only monoclonal antibody with proven superiority to rituximab for the treatment for CLL when used in combination with chlorambucil as proven in clinical trials.

Obinutuzumab is given by intravenous infusion which means it goes directly into the vein through a needle in your arm or through a central line if you have one in place. Obinutuzumab is given every week for 3 weeks then once every four weeks in combination with chlorambucil depending on your subtype of lymphoma.

Obinutuzumab may have the following side effects;

- Hair loss
- Infections

If my lymphoma relapses I hope that I can have access to the best treatment available for me" – *Max*

- Lowered blood counts
- Mouth sores
- Nausea
- Skin problems
- Tiredness

Refer to the (obinutuzumab) Consumer Medicines Information for more side effects.

Obinutuzumab is approved by the TGA and listed on the PBS for the following indications;

- CLL as a first line treatment in combination with chlorambucil

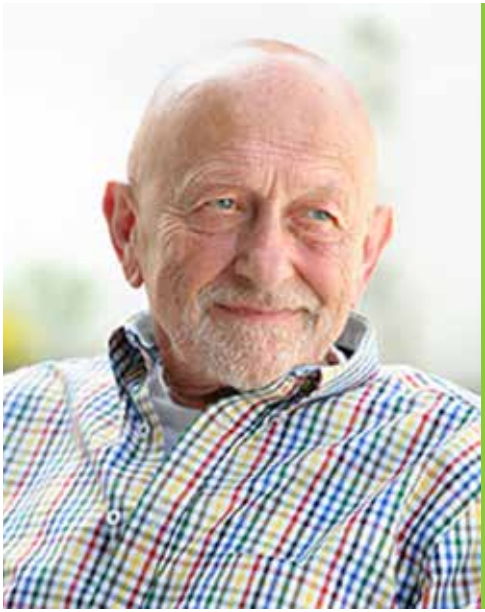
Romidepsin (Istodax ™)

Romidepsin is a targeted therapy medicine used in the treatment of Peripheral T Cell Lymphoma (PTCL) and Cutaneous T Cell Lymphoma (CTCL). Romidepsin is isolated from a bacteria called Chromobacterium violaceum and is thought to reduce the growth and division of lymphoma cells by inhibiting the the process involved in the regulation in these lymphomas.

Romidepsin is given by intravenous infusion which means it goes directly into the vein through a needle in your arm or through a central line if you have one in place. Romidepsin is given every week for three weeks then a week off the medicine to complete a four week cycle. Number of cycles given will depend on your subtype and disease status of lymphoma.

Romidepsin may have the following side effects;

- Diarrhoea
- Infections
- Low blood counts
- Nausea
- Tiredness



Now I am taking this new tablet I can stop worrying about my cancer every day and try and get on with life knowing that I am feeling better"
– Bob

Romidepsin is approved by the TGA and currently awaiting approval to be listed on the PBS however access to romidepsin can be found in clinical trials around Australia and by access programs through the pharmaceutical company.

Venetoclax (Venclexta ™)

Venetoclax is a targeted therapy medicine used in the treatment of CLL with a promising outlook in treatment of other subtypes of lymphoma in the future. Venetoclax works by targeting a specific protein in your blood called BCL-2 which is a protein that may build up in your body when you have CLL and prevent CLL cells from self-destruction naturally.

Venetoclax is an oral tablet that you take every day.

I am amazed at how much I am able to do whilst I am on this medication and how fortunate I feel to be part of this clinical trial for my lymphoma"
– Jill

Venetoclax may have the following side effects;

- Diarrhoea
- Lowered blood counts
- Nausea
- Tiredness
- Upper respiratory tract infection

Venetoclax is approved by the TGA and is being considered for listing and approval with the PBS.

Vorinostat (Zolinza ™)

Vorinostat is a targeted therapy medicine used in the treatment of Cutaneous T Cell Lymphoma (CTCL). Vorinostat is thought to work by reducing the growth and division of lymphoma cells a process in the DNA which is involved in the gene regulation in these lymphomas.

Vorinostat is an oral capsule that you take every day.

Vorinostat may have the following side effects;

- Diarrhoea
- Infections
- Low blood counts
- Nausea
- Tiredness

Vorinostat is approved by the TGA for the following indications;

- CTCL as a third line treatment

"My skin has never been as good as it is now I am on this new medicine and I also have more energy to be able to go and do things with my family" – *Dorothy*

Biological medicines and biosimilars

A biological medicine is a medicine that contains one or more active substances that are made by or extracted from living cells or organisms. They are usually made up of proteins that are naturally made in the body and are developed for the treatment of many cancers including lymphoma.

Once a biological medicine is produced the medicine is placed under patent. A patent is a licence that gives the original developer of the medicine the legal right to be the only one on the market for a number of years. Once this patent expires other companies are allowed to produce medicines that are similar to the original biological medicine and these are called biosimilar medicines.

Biosimilar medicines are very similar to the original biological medicine and can be used to treat the same diseases in the same way as the biological medicines. These biosimilar medicines have been tested and shown to be as safe and effective as the original biological medicines.

There are currently three biosimilar medicines approved by the TGA in Australia for use in the lymphoma setting. The original biological medicine is filgrastim which was produced by a company called Amgen and patented under the trade name of Neupogen ™. Filgrastim is a man made form of granulocyte colony stimulating factor which is a substance produced by the body to stimulate the growth of neutrophils.

Neutrophils are a type of white blood cell which are important for the body's fight against infection and filgrastim can be given to people having treatment for their lymphoma to help support their neutrophil count which is lowered with the treatment they are receiving. Once this biological medicine came off of patent other companies were able to produce a biosimilar medicine and currently there are three biosimilars for this medicine with the trade names Nivestim ™ produced by Hospira, Tevagrastim ™ produced by Aspen Pharmacare and Zarzio ™ produced by Sandoz.

Biosimilars differ from generic medicines as generic medicines are exactly the same active ingredient as the original chemical medicine. An example of a generic medicine is the original chemical medicine paracetamol which was patented as Panadol and the generic versions include Panamax ™ and Herron ™ as examples.

CAR-T cells

CAR-T cells are T cells collected from a person's blood and genetically engineered to produce chimeric antigen receptor T cells (CAR-T). CAR-T cells allow the T cells to recognise antigens on lymphoma cells in the body therefore using the person's own immune system to kill their lymphoma.

The T cells are collected from a person with lymphoma by using an apheresis machine which allows the T cells to be collected from the blood stream and all the other blood cells that are not required are returned to the person on the machine. The T cells are then sent to a special laboratory to be genetically engineered for a receptor on the person's lymphoma so that when the CAR-T cells are given back to the person they will target their lymphoma.

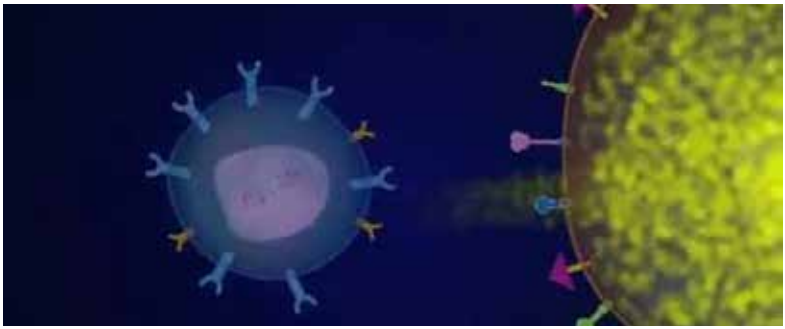
The CAR-T cells are then given back to the person with lymphoma by intravenous infusion which means they go directly in to the vein through a needle in their arm or through a central line if they have one in place. Once the CAR-T cells come into contact with the person's lymphoma in the body they do three things;

- Kill lymphoma cells directly
- Divide making more CAR-T cells at the site of the lymphoma

- Set up inflammation in the lymphoma so that the body's immune response spreads

Clinical trials for CAR-T cells commenced in the United States of America with promising results in Acute Lymphoblastic Leukaemia (ALL), Diffuse Large B Cell Lymphoma (DLBCL) and Follicular Lymphoma (FL) with evidence that may be utilised in other forms of lymphoma in the future.

Clinical trials are currently available in Australia for CAR-T cells in DLBCL and will soon be available in FL and other lymphomas in the future. The current process of these clinical trials in Australia involves people to be enrolled in a global trial where they have their T cells collected at a centre in Australia and the T cells are sent to a laboratory in the USA for genetic engineering and sent back to reinfuse to the person in Australia. Please ask your specialist doctor if CAR-T cell therapy may be appropriate for you and your lymphoma.



Clinical Trials

Clinical Trials are critical for advancing therapies for lymphoma and are a fantastic way for patients to access a certain medicine for their type of lymphoma. Clinical trials are research investigations where patients can volunteer to participate to gain access to a new medicine as a means to treat and manage their lymphoma. Clinical trials are divided into different phases and below is a table to explain the different phases and what they mean.

Clinical Trial	Description
Phase 1	New medicine tested in a small group of patients to evaluate safety and determine safe dose of medicine and identify any side effects
Phase 2	The medicine is then tested in a larger group of patients to determine how effective the medicine is and further evaluate its safety
Phase 3	The medicine is then tested in a larger group pf patients by comparing the intervention to other standard or experimental interventions and is used to monitor adverse side effects and to collect information that will allow the medicine to be used safely
Phase 4	Once the medicine is approved for use these trials are designed to monitor the effectiveness of the approved medicine in the general population and to collect information about any adverse side effects associated with the new medicine over longer periods of time and may be used to investigate the use of the medicine in different indications

Where To Find Clinical Trials In Australia

The Australian Clinical Trials website allows you to search for a clinical trial by disease type and you will receive a brief description of the trial, eligibility criteria for the trial and contact details for further information. You can visit the Australian Clinical Trials website by following this link; https://www.australianclinicaltrials.gov.au

There is a user friendly app that has been developed by ClinTrial Refer which allows you to search by your subtype of lymphoma for trials anywhere in Australia and it will tell you whether the trial is currently open, where it is open and who to contact for further information. You can visit the ClinTrial Refer website by following this link; http://www.clintrial.org.au/

You can download the ClinTrial Refer app for free by visiting your app store on your phone for apple or android users.

Below are some examples of the ClinTrial Refer app in use.

How Is A Medicine Approved In Australia And What You Can Do To Help?

There are two important regulatory bodies in Australia involved in the approval and use of medicines called the Therapeutic Goods Administration and the Pharmaceutical Benefits Scheme and we will take a look at how they both work to approve new medicines.

Therapeutic Goods Administration (TGA) of Australia

The TGA is Australia's regulatory authority for therapeutic goods such as medicines. The TGA carries out a range of assessment and monitoring activities to ensure medicines available in Australia are of an acceptable standard with the aim of ensuring that the Australian community has access within a reasonable time frame to these medicines and is a requirement by Australian law. The TGA must approve a medicine for a certain indication before it can be listed on the Pharmaceutical Benefits Scheme. You can go online to the TGA website to search for a medicine by name to see if it is approved for use in Australia.
https://www.tga.gov.au/

Pharmaceutical Benefits Scheme (PBS) of Australia

The PBS is part of the Australian Government's National Health Policy and they provide timely, reliable and affordable access to medicines for Australians. Under the PBS, the government subsidises the cost of a medicine for most medical conditions so that Australians do not have to pay

the entire cost of the medicine and instead make a co-payment which makes the medicine more affordable. You can go online to the PBS website listed below to search for a medicine by name to see if it is currently covered by the PBS.
http://www.pbs.gov.au/pbs/home

How do medicines get approved by the PBS?

The PBS has an advisory committee of independent expert individuals called the Pharmaceutical Benefits Advisory Committee (PBAC). PBAC follows a process to review medicines for listing on the PBS. The PBAC makes recommendations to the government about whether to subsidise new medicines or not and then the government makes the decision of whether to subsidise the new medicine on the PBS.

What is the application process for getting a new medicine approved by the PBS?

PBAC meet three times a year in March, July and November to review submissions for listing on the PBS and the key steps to submission of a medicine are as follows;

- The sponsor of the medicine (normally the pharmaceutical company) makes a submission for a medicine to the PBAC four months prior to the PBAC meeting date

- The PBAC agenda listing all submissions is published 10 weeks prior to the meeting

- Members of the public and health professionals can submit comments about the submissions to the PBAC from the date of the agenda is published up until 4 weeks prior to the meeting

- PBAC recommendations are published 6 weeks after the meeting

- If the PBAC rejects a submission it will explain why and the sponsor of the medicine can resubmit if they can address the PBAC's concerns

Under special circumstances, the government may agree to subsidise the cost of a medicine on a provisional basis. This process is known as the 'Managed Access Program' and PBAC may agree to this access program under the following circumstances;

- Where patients have an urgent clinical need for a medicine

- There is enough clinical evidence to show the benefit of the medicine but not enough to demonstrate cost-effectiveness

- Where the sponsor of the medicine will be able to provide more clinical evidence in a reasonable time period so

that the PBAC can undertake a full assessment of the medicines value

- The 'Managed Access Program' does not guarantee that a medicine will be approved for listing on the PBS indefinitely either as PBAC may recommend delisting of the medicine based on the further clinical evidence provided about the efficacy and safety of the medicine

How can patients have a say in the PBAC review process for PBS?

Anyone in the Australian public who has an interest in a certain medicine and the decision on whether it should be subsidised on the PBS is able to have their voice heard. This includes those people living with the disease, a loved one who has been impacted by the disease, members of the public, carers, family members, friends, health care professionals and patient consumer groups.

Consumer involvement from patients point of view allows the submission for a certain medicine to demonstrate how the availability of that particular medicine on the PBS would impact an individual's quality of life and most importantly how not being able to access the medicine would disadvantage an individual. This is a very important part of the process and we would encourage you to put in your say for a medicine.

The patient voice

Your knowledge, views and experience of living with lymphoma is important when new medicines are being considered by PBAC.

Patient organisations like Lymphoma Australia need your help when new medicines for lymphoma and CLL are being considered for reimbursement with the PBS.

Please let Lymphoma Australia know if you are on a clinical trial or are accessing a treatment that is not funded by the PBS as your experience can help others

Email enquiries@lymphoma.org.au

The PBAC meeting agenda with the list of medicines for submission can be found on the PBS website and you can make comments on a form on the following website link; http://www.pbs.gov.au/info/industry/listing/elements/pbac-meetings/agenda

You can also access the consumer comments form through the Department of Health website using the following website link; http://www.health.gov.au/internet/main/publishing.nsf/Content/PBAC_online_submission_form

What happens after the PBAC make a recommendation for medicines to be listed on the PBS?

The Health Minister (or delegate) considers the PBAC's recommendations and the Health Department's advice on the cost of the medicine to the PBS which is developed through the negotiations with the medicine's sponsor. The decision to subsidise a medicine goes to the cabinet if the cost of the medicine is expected to be in excess of 20 million dollars a year to the PBS. If the cabinet approves the subsidy the decision on the timing to add the medicine to the PBS is made by the Health Minister. The timing of these reviews by cabinet can vary but may take up to 6 months from the PBAC's recommendations.

How can a patient gain access to a medicine prior to registration or subsidy on the PBS?

Patients may be able to access a medicine prior to it being approved by the TGA on a clinical trial or by a 'Special Access Scheme'. The 'Special Access Scheme' enables doctors to apply for a medicine for their patient when they have exhausted all other approved treatment options and more information on this scheme can be found on the TGA website by following this website link; http://www.tga.gov.au/form/special-access-scheme

Some pharmaceutical companies also offer 'Patient Access Programs' for medicines approved by the TGA but awaiting PBS listing. Doctors can apply for a patient to receive a medicine on these programs and the patient eligibility is determined by the pharmaceutical company and there may be a cost of the medicine incurred by the patient. Pharmaceutical companies in Australia are guided by the Code of Conduct and are therefore limited with the information that they can provide directly to patients. If you are wondering if there is are any other medicines out there that may help you in the treatment of your lymphoma do not be afraid to ask your doctor or nurse for further information.

Where To Go For Further Information

Lymphoma Australia website
www.lymphoma.org.au

Lymphoma Australia Nurse Hotline
Phone 1800 953 081

Therapeutic Good Administration Australia website
https://www.tga.gov.au/

Pharmaceutical Benefits Scheme Australia website
http://www.pbs.gov.au/pbs/home

Australian Clinical Trials website
https://www.australianclinicaltrials.gov.au

ClinTrial Refer website
http://www.clintrial.org.au/

Australasian Leukaemia & Lymphoma Group (ALLG)
http://www.allg.org.au/

Notes

Stay connected

it's easy!

1. **Visit www.lymphoma.org.au**
to order your free resource book 'Living with Lymphoma'.

2. **Subscribe to the eNewsletter.**
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Share experiences, information and research by joining our lymphoma patients and carers Facebook group. Search Facebook groups for Lymphoma Down Under

Lymphoma Nurse Hotline

1800 953 081

We are here to help.
Please call, as fear of the unknown should not be part of the Lymphoma experience.

Lymphoma Australia would like to give a very special thank you to sponsors, hospitals, doctors, nurses, patients, families and friends that make our educational resources possible. Taking the fear of the unknown out of the Lymphoma journey can make a huge difference.

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