Cancer Association of South Africa (CANSA)

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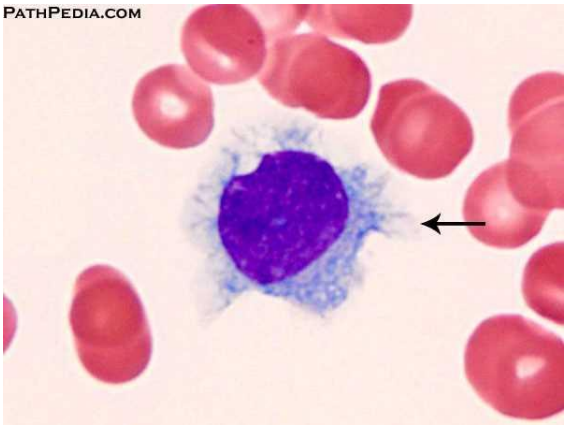
Fact Sheet on Adult Hairy Cell Leukaemia

Introduction

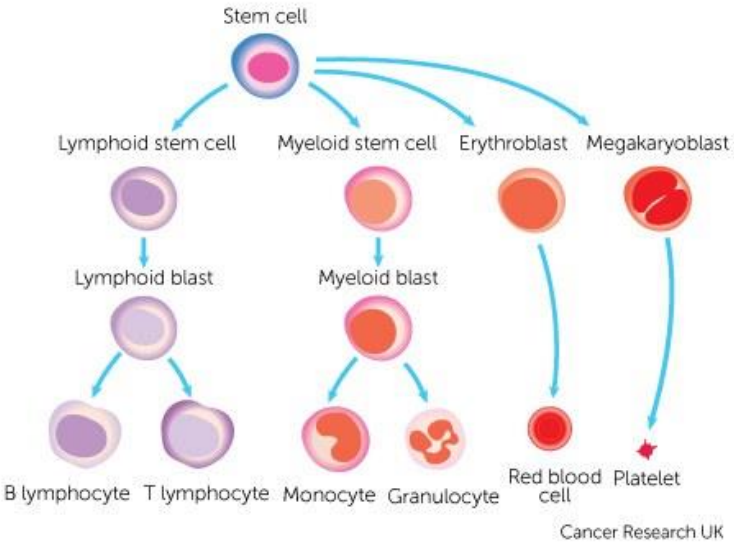
Leukaemia is a cancer of the blood forming system. Most types of leukaemia cause the bone marrow to make abnormal white blood cells. These abnormal cells can get into the bloodstream and circulate around the body.



[Picture Credit: Hairy Cell Leukaemia]

The body makes blood cells in the bone marrow. The bone marrow is the soft inner part of one's bones. The bone marrow makes blood cells in a controlled way, as the body needs them. All blood cells start as the same type of cell, called a stem cell. The stem cells then develop into:

- Myeloid stem cells, which become white blood cells called monocytes and neutrophils
- Lymphoid stem cells, which become white blood cells called lymphocytes
- Erythroblasts, which become red blood cells
- Megakaryocytes, which become platelets



White blood cells (leucocytes) - there are several different types of white cells in the blood. There are more of some types than others. They all play a part in the immune response – the response of the body to infection, or anything else the body recognises as foreign. These blood cells can be made very quickly and generally have a short life. Some only live for a few hours, others for a few days.

Red blood cells (erythrocytes) - red blood cells carry oxygen from the lungs around the body to the

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tissues. It gives the blood its red colour.

Platelets (thrombocytes) - platelets are very important in blood clotting. It clumps together to form a plug if bleeding occurs. Then they release other chemicals that help the blood to clot and the blood vessel to be repaired.

How leukaemia affects the body - white blood cells help fight infection. If the body is making abnormal white blood cells, they will not work properly. so one may be more prone to infections. Having abnormal white blood cells also makes it more difficult to get rid of infections once one has them.

If one has too many white blood cells, they can overcrowd the bone marrow so there is not enough space for normal red blood cells and platelets and one may have lower than normal numbers of these. Having too few red blood cells makes one tired and breathless (anaemic). And if one does not have enough platelets, one can have bleeding problems.

Abnormal white blood cells can also build up in parts of the lymphatic system such as the spleen and lymph nodes. They can also build up in the liver. This can make your abdomen swell and feel uncomfortable.
(Cancer Research UK).

Adult Hairy Cell Leukaemia (HCL)
Hairy cell leukaemia is a rare, slow-growing cancer of the blood in which the bone marrow makes too many B cells (lymphocytes), a type of white blood cell that fights infection. These excess B cells are abnormal and look "hairy" under a microscope. As the number of leukaemia cells increases, fewer healthy white blood cells, red blood cells and platelets are produced.

Hairy cell leukaemia affects more men than women, and it occurs most commonly in middle-aged or older adults. Hairy cell leukaemia is considered a chronic disease because it may never completely disappear, although treatment can lead to a remission for years.
(Mayo Clinic).

Incidence of Adult Hairy Cell Leukaemia in South Africa
In providing the incidence figures of Leukaemia in South Africa, The National Cancer Registry (2013) does not make provision for the reporting of the different types of Leukaemia – it also does not differentiate between acute and chronic Leukaemia - neither does it provide for different statistics for cases of adult and childhood Leukaemia.

According to the National Cancer Registry (2013) the following number of Leukaemia cases was histologically diagnosed in South Africa during 2013:

Group - Males 2013	Actual No of Cases	Estimated Lifetime Risk	Percentage of All Cancers
All males	351	1:645	1,98%
Asian males	8	1:907	1,00%
Black males	184	1:1 081	1,71%
Coloured males	46	1:441	1,10%
White males	114	1:282	0,56%

Group - Females 2013	Actual No of Cases	Estimated Lifetime Risk	Percentage of All Cancers
All females	279	1:1 068	0,76%
Asian females	6	1:1 625	0,60%
Black females	141	1:1 695	0,90%
Coloured females	42	1:722	1,03%
White females	91	1:394	0,57%

The frequency of histologically diagnosed cases of Leukaemia in South Africa for 2013 was as follows (National Cancer Registry, 2013):

Group - Males 2013	0 – 19 Years	20 – 29 Years	30 – 39 Years	40 – 49 Years	50 – 59 Years	60 – 69 Years	70 – 79 Years	80+ Years
All males	103	28	24	42	42	51	39	12
Asian males	3	1	1	0	1	2	0	0
Black males	65	19	15	28	18	15	10	1
Coloured males	14	3	2	4	6	8	4	1
White males	12	5	6	18	18	23	30	11

Group - Females 2013	0 – 19 Years	20 – 29 Years	30 – 39 Years	40 – 49 Years	50 – 59 Years	60 – 69 Years	70 – 79 Years	80+ Years
All females	55	34	28	30	45	35	29	16
Asian females	3	0	0	1	0	1	0	1
Black females	28	26	21	13	16	14	7	4
Coloured females	5	4	3	9	10	4	2	2
White females	14	3	4	7	18	13	20	8

N.B. In the event that the totals in any of the above tables do not tally, this may be the result of uncertainties as to the age, race or sex of the individual. The totals for 'all males' and 'all females', however, always reflect the correct totals.

Signs and Symptoms of Adult Hairy Cell Leukaemia

The causes of Hairy Cell Leukaemia (HCL) are unknown. It is not infectious and cannot be passed on to other people.

Because HCL usually develops slowly, it may not cause any symptoms for a long time. It is sometimes discovered by chance when a blood test is taken for another reason, for example as part of a routine health check.

HCL can cause symptoms such as weight loss, weakness, frequent infections and breathlessness. Some people see their doctor because they feel tired or look pale due to a lack of red blood cells (anaemia).

Some people get repeated infections, because they have a shortage of healthy white blood cells that normally fight off infections.

People who have an enlarged spleen may feel a tender lump on the left side of their abdomen.

These symptoms can be caused by other conditions but should always be checked by your doctor.

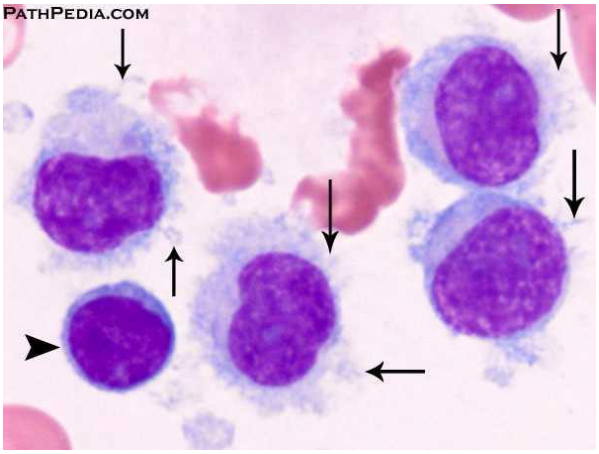
These and other signs and symptoms may be caused by adult hairy cell leukaemia or by other conditions:

- Weakness or feeling tired.
- Fever or frequent infections.
- Easy bruising or bleeding.
- Shortness of breath.
- Weight loss for no known reason.
- Pain or a feeling of fullness below the ribs.
- Painless lumps in the neck, underarm, stomach, or groin.

(MacMillan Cancer Support; National Cancer Institute).

Diagnosis of Adult Hairy Cell Leukaemia

The best approach to establishing the diagnosis of hairy cell leukaemia is to carefully examine blood and bone marrow biopsy specimens to identify cells with the morphologic features of hairy cells and to demonstrate that the neoplastic cells have an antigenic profile that is characteristic for hairy cell leukaemia.



[Picture Credit: Hairy Cell Leukaemia 2]

The cell is characterised by an eccentrically located nucleus with fine chromatin, indistinct nucleoli, and an abundant amount of grey-blue cytoplasm with shaggy margins.
(NCBI).

Other problems to be considered in the differential diagnosis of adult hairy cell leukaemia include the following:

- Primary meyelofibrosis
- Chronic lymphocytic leukaemia
- Low-grade lymphoma
- Myelosclerosis
- Pancytopenia and marrow fibrosis
- Prolymphocytic leukaemia
- Splenic marginal zone lymphoma
- Systemic mastocytosis

Differential Diagnoses:

- Anaemia
- Aplastic anaemia
- Chronic lymhocytic leukaemia
- Myelodysplastic syndrome
- Myelophthisic anaemia
- Myeloproliferative disease

- Primary myelofibrosis

(Medscape).

The following tests and procedures may be used:

Physical examination and history - an examination of the body to check general signs of health, including checking for signs of disease, such as a swollen spleen, lumps, or anything else that seems unusual. A history of the patient's health habits and past illnesses and treatments will also be taken.

Complete blood count (CBC) - a procedure in which a sample of blood is drawn and checked for the following:

- The number of red blood cells, white blood cells, and platelets.
- The amount of haemoglobin (the protein that carries oxygen) in the red blood cells.
- The portion of the sample made up of red blood cells.

Blood is collected by inserting a needle into a vein and allowing the blood to flow into a tube. The blood sample is sent to the laboratory and the red blood cells, white blood cells, and platelets are counted. The CBC is used to test for, diagnose, and monitor many different conditions.

Peripheral blood smear - a procedure in which a sample of blood is checked for cells that look "hairy", the number and kinds of white blood cells, the number of platelets, and changes in the shape of blood cells.

Blood chemistry studies - a procedure in which a blood sample is checked to measure the amounts of certain substances released into the blood by organs and tissues in the body. An unusual (higher or lower than normal) amount of a substance can be a sign of disease.

Bone marrow aspiration and biopsy - the removal of bone marrow, blood, and a small piece of bone by inserting a hollow needle into the hipbone or breastbone. A pathologist views the bone marrow, blood, and bone under a microscope to look for signs of cancer.

After a small area of skin is numbed, a bone marrow needle is inserted into the patient's hip bone. Samples of blood, bone, and bone marrow are removed for examination under a microscope.

Immunophenotyping – a laboratory test in which the antigens or markers on the surface of a blood or bone marrow cell are checked to see what type of cell it is. This test is done to diagnose the specific type of leukaemia by comparing the cancer cells to normal cells of the immune system.

Flow cytometry - a laboratory test that measures the number of cells in a sample, the percentage of live cells in a sample, and certain characteristics of cells, such as size, shape,

and the presence of tumour markers on the cell surface. The cells are stained with a light-sensitive dye, placed in a fluid, and passed in a stream before a laser or other type of light. The measurements are based on how the light-sensitive dye reacts to the light.

Cytogenetic analysis - a laboratory test in which cells in a sample of tissue are viewed under a microscope to look for certain changes in the chromosomes.

CT scan (CAT scan) - a procedure that makes a series of detailed pictures of areas inside the body, taken from different angles. The pictures are made by a computer linked to an x-ray machine. A dye may be injected into a vein or swallowed to help the organs or tissues show up more clearly. This procedure is also called computed tomography, computerized tomography, or computerized axial tomography. A CT scan of the abdomen may be done to check for swollen lymph nodes or a swollen spleen.
(National Cancer Institute).

Treatment of Adult Hairy Cell Leukaemia

Not all patients will require treatment immediately after the diagnosis is made and can be monitored until it is needed. This 'watch and wait' surveillance approach can be difficult for patients and their families and generates a lot of anxiety. However, unlike other types of cancer, leukaemias do not spread or metastasise and so waiting to start treatment until there are clear signs that it is indicated is perfectly safe and has the advantage of not exposing a patient to drugs, which may have side effects, earlier than is necessary.

Other patients will clearly need treatment straight away because of symptoms and/or very low blood counts. The decision of when to start treatment depends upon the symptoms experienced by the patient and the degree of abnormality in the blood count. Often patients will remain entirely well even if they do need treatment because of a reduction in normal blood counts. This reduction is the usual reason for initiating treatment and it is preferable not to wait until the blood counts fall to very low levels even if the patient is well. Three types of cells may be reduced – red blood cells (causing anaemia), white blood cells (which help fight infection) and platelets (which prevent abnormal bleeding and bruising). Not all may be low but most commonly the ability to fight infection is reduced and treatment is needed to restore this. After treatment there is almost always a further, temporary, fall in normal counts before they recover as it takes time for the disease to clear from the bone marrow.

Recovery usually takes 3-6 weeks. Infection is the biggest risk, before, during and for a period after, treatment. It is important to treat any active infection effectively before any therapy for the HCL is started. It is rare that treatment is needed urgently for HCL. Therefore, careful clinical judgment is necessary to make the best decision for each individual patient regarding the optimal time to start treatment for the HCL.

For the past 30 years the mainstay of treatment for HCL has been with 2 drugs in the group of purine analogues called *pentostatin* and *cladribine*. *Pentostatin* was the first to be introduced in the 1980s and then *cladribine* in the early 1990s so there is now a great deal of experience and long-term follow up with these agents. Both are highly effective at inducing a remission (in almost 100% of patients) with no significant difference between the two. Most of these remissions are complete (CR) i.e. no sign of any remaining disease in the bone

marrow using standard methods. Such remissions often last for very prolonged periods of time (more than 10 years).

Pentostatin is administered as a short intravenous (IV) infusion every 2-3 weeks until a remission is achieved (usually 8-10 injections). It is excreted in the body by the kidneys and it is therefore important to check kidney function to ensure this is normal. The dose of *pentostatin* needs to be reduced if the creatinine clearance (measure of renal function) falls below a certain level. Patients can experience nausea up to 72 hours after the infusion and should have anti-sickness pills to take if needed.

Cladribine can be given in a number of ways including as a 7- day continuous IV infusion (which may require a hospital admission), daily or weekly IV infusions for 6 doses or as a subcutaneous injection on 5 consecutive days. There is no evidence that these are not all equivalent in terms of effectiveness and the choice will largely depend on the physician and patient.

Most can be given as out-patient treatment.

Both treatments are generally well tolerated but are associated with a temporary reduction in normal blood counts. This needs to be monitored closely until they recover (weekly initially). Sometimes the recovery of the blood counts can be delayed for a much longer time but eventually they do improve.

Pentostatin and *cladribine* also cause a more prolonged suppression of the immune system and advice should be given about the signs and symptoms of infection to be on the watch for. Infections should always be taken seriously as prompt treatment is important. Some doctors will also administer low doses of antibiotics/antivirals to reduce the risk of infections. In the case of *cladribine* it is better to start these after the 1-week course of treatment has been given since rashes can sometimes occur when the drugs are given concurrently. Occasionally growth factors (e.g. GCSF) may be given to speed the recovery of the white blood cells. Other supportive drugs such as allopurinol are not usually needed. Blood transfusions, if required, should be with irradiated blood.

Splenectomy is rarely undertaken now since other therapies are so effective in reducing the size of the spleen, which is often enlarged at the time of diagnosis.

Interferon is rarely used. It is not well tolerated and much less effective than the purine analogues, but occasionally may still be useful.

Rituximab used as a single agent in first line treatment of HCL is not as effective in inducing remissions as the purine analogues. Its use would be reserved for patients unable to tolerate purine analogues. There is early evidence that better remissions may be achieved with the combination of*rituximab* and a purine analogue (*pentostatin* or *cladribine*). Because of the very good results with purine analogues alone for most patients the addition of *rituximab* is often reserved for those patients who do not achieve a CR or who *relapse* earlier than expected after treatment.

Novel Agents: Targeted therapies such as immunoconjugates (moxetumumab pasudotox), BRAF inhibitors (e.g. vemurafenib) and B-cell receptor inhibitors (e.g. ibrutinib) all have activity in HCL. Currently these have been examined in relapsed or refractory HCL and only in a very small number of patients (in whom PAs cannot be given) as first line therapy. A number of clinical trials are being undertaken to evaluate these drugs further.

Clinical Trials: HCL is a rare disease and there are very few clinical studies being undertaken worldwide. It is important for patients to participate in clinical trials if at all possible, particularly if their disease has been hard to treat and they would particularly benefit from novel therapy.
(Hairy Cell Leukemia Foundation).

About Clinical Trials
Clinical trials are research studies that involve people. These studies test new ways to prevent, detect, diagnose, or treat diseases. People who take part in cancer clinical trials have an opportunity to contribute to scientists' knowledge about cancer and to help in the development of improved cancer treatments. They also receive state-of-the-art care from cancer experts.

Types of Clinical Trials
Cancer clinical trials differ according to their primary purpose. They include the following types:

Treatment - these trials test the effectiveness of new treatments or new ways of using current treatments in people who have cancer. The treatments tested may include new drugs or new combinations of currently used drugs, new surgery or radiation therapy techniques, and vaccines or other treatments that stimulate a person's immune system to fight cancer. Combinations of different treatment types may also be tested in these trials.

Prevention - these trials test new interventions that may lower the risk of developing certain types of cancer. Most cancer prevention trials involve healthy people who have not had cancer; however, they often only include people who have a higher than average risk of developing a specific type of cancer. Some cancer prevention trials involve people who have had cancer in the past; these trials test interventions that may help prevent the return (recurrence) of the original cancer or reduce the chance of developing a new type of cancer

Screening - these trials test new ways of finding cancer early. When cancer is found early, it may be easier to treat and there may be a better chance of long-term survival. Cancer screening trials usually involve people who do not have any signs or symptoms of cancer. However, participation in these trials is often limited to people who have a higher than average risk of developing a certain type of cancer because they have a family history of that type of cancer or they have a history of exposure to cancer-causing substances (e.g., cigarette smoke).

Diagnostic - these trials study new tests or procedures that may help identify, or diagnose, cancer more accurately. Diagnostic trials usually involve people who have some signs or symptoms of cancer.

Quality of life or supportive care - these trials focus on the comfort and quality of life of cancer patients and cancer survivors. New ways to decrease the number or severity of side effects of cancer or its treatment are often studied in these trials. How a specific type of cancer or its treatment affects a person's everyday life may also be studied.

Where Clinical Trials are Conducted

Cancer clinical trials take place in cities and towns in doctors' offices, cancer centres and other medical centres, community hospitals and clinics. A single trial may take place at one or two specialised medical centres only or at hundreds of offices, hospitals, and centres.

Each clinical trial is managed by a research team that can include doctors, nurses, research assistants, data analysts, and other specialists. The research team works closely with other health professionals, including other doctors and nurses, laboratory technicians, pharmacists, dieticians, and social workers, to provide medical and supportive care to people who take part in a clinical trial.

Research Team

The research team closely monitors the health of people taking part in the clinical trial and gives them specific instructions when necessary. To ensure the reliability of the trial's results, it is important for the participants to follow the research team's instructions. The instructions may include keeping logs or answering questionnaires. The research team may also seek to contact the participants regularly after the trial ends to get updates on their health.

Clinical Trial Protocol

Every clinical trial has a protocol, or action plan, that describes what will be done in the trial, how the trial will be conducted, and why each part of the trial is necessary. The protocol also includes guidelines for who can and cannot participate in the trial. These guidelines, called eligibility criteria, describe the characteristics that all interested people must have before they can take part in the trial. Eligibility criteria can include age, sex, medical history, and current health status. Eligibility criteria for cancer treatment trials often include the type and stage of cancer, as well as the type(s) of cancer treatment already received.

Enrolling people who have similar characteristics helps ensure that the outcome of a trial is due to the intervention being tested and not to other factors. In this way, eligibility criteria help researchers obtain the most accurate and meaningful results possible.

National and International Regulations

National and international regulations and policies have been developed to help ensure that research involving people is conducted according to strict scientific and ethical principles. In these regulations and policies, people who participate in research are usually referred to as "human subjects."

Informed Consent

Informed consent is a process through which people learn the important facts about a clinical trial to help them decide whether or not to take part in it, and continue to learn new information about the trial that helps them decide whether or not to continue participating in it.

During the first part of the informed consent process, people are given detailed information about a trial, including information about the purpose of the trial, the tests and other procedures that will be required, and the possible benefits and harms of taking part in the trial. Besides talking with a doctor or nurse, potential trial participants are given a form, called an informed consent form, that provides information about the trial in writing. People who agree to take part in the trial are asked to sign the form. However, signing this form does not mean that a person must remain in the trial. Anyone can choose to leave a trial at

any time—either before it starts or at any time during the trial or during the follow-up period. It is important for people who decide to leave a trial to get information from the research team about how to leave the trial safely.

The informed consent process continues throughout a trial. If new benefits, risks, or side effects are discovered during the course of a trial, the researchers must inform the participants so they can decide whether or not they want to continue to take part in the trial. In some cases, participants who want to continue to take part in a trial may be asked to sign a new informed consent form.

New interventions are often studied in a stepwise fashion, with each step representing a different "phase" in the clinical research process. The following phases are used for cancer treatment trials:

Phases of a Clinical Trial

Phase 0. These trials represent the earliest step in testing new treatments in humans. In a phase 0 trial, a very small dose of a chemical or biologic agent is given to a small number of people (approximately 10-15) to gather preliminary information about how the agent is processed by the body (pharmacokinetics) and how the agent affects the body (pharmacodynamics). Because the agents are given in such small amounts, no information is obtained about their safety or effectiveness in treating cancer. Phase 0 trials are also called micro-dosing studies, exploratory Investigational New Drug (IND) trials, or early phase I trials. The people who take part in these trials usually have advanced disease, and no known, effective treatment options are available to them.

Phase I (also called phase 1). These trials are conducted mainly to evaluate the safety of chemical or biologic agents or other types of interventions (e.g., a new radiation therapy technique). They help determine the maximum dose that can be given safely (also known as the maximum tolerated dose) and whether an intervention causes harmful side effects. Phase I trials enrol small numbers of people (20 or more) who have advanced cancer that cannot be treated effectively with standard (usual) treatments or for which no standard treatment exists. Although evaluating the effectiveness of interventions is not a primary goal of these trials, doctors do look for evidence that the interventions might be useful as treatments.

Phase II (also called phase 2). These trials test the effectiveness of interventions in people who have a specific type of cancer or related cancers. They also continue to look at the safety of interventions. Phase II trials usually enrol fewer than 100 people but may include as many as 300. The people who participate in phase II trials may or may not have been treated previously with standard therapy for their type of cancer. If a person has been treated previously, their eligibility to participate in a specific trial may depend on the type and amount of prior treatment they received. Although phase II trials can give some indication of whether or not an intervention works, they are almost never designed to show whether an intervention is better than standard therapy.

Phase III (also called phase 3). These trials compare the effectiveness of a new intervention, or new use of an existing intervention, with the current standard of care (usual treatment) for a particular type of cancer. Phase III trials also examine how the side effects of the new

intervention compare with those of the usual treatment. If the new intervention is more effective than the usual treatment and/or is easier to tolerate, it may become the new standard of care.

Phase III trials usually involve large groups of people (100 to several thousand), who are randomly assigned to one of two treatment groups, or "trial arms": (1) a control group, in which everyone in the group receives usual treatment for their type of cancer, or 2) an investigational or experimental group, in which everyone in the group receives the new intervention or new use of an existing intervention. The trial participants are assigned to their individual groups by random assignment, or randomisation. Randomisation helps ensure that the groups have similar characteristics. This balance is necessary so the researchers can have confidence that any differences they observe in how the two groups respond to the treatments they receive are due to the treatments and not to other differences between the groups.

Randomisation is usually done by a computer program to ensure that human choices do not influence the assignment to groups. The trial participants cannot request to be in a particular group, and the researchers cannot influence how people are assigned to the groups. Usually, neither the participants nor their doctors know what treatment the participants are receiving.

People who participate in phase III trials may or may not have been treated previously. If they have been treated previously, their eligibility to participate in a specific trial may depend on the type and the amount of prior treatment they received.

In most cases, an intervention will move into phase III testing only after it has shown promise in phase I and phase II trials.

Phase IV (also called phase 4). These trials further evaluate the effectiveness and long-term safety of drugs or other interventions. They usually take place after a drug or intervention has been approved by the medicine regulatory office for standard use. Several hundred to several thousand people may take part in a phase IV trial. These trials are also known as post-marketing surveillance trials. They are generally sponsored by drug companies.

Sometimes clinical trial phases may be combined (e.g., phase I/II or phase II/III trials) to minimize the risks to participants and/or to allow faster development of a new intervention.

Although treatment trials are always assigned a phase, other clinical trials (e.g., screening, prevention, diagnostic, and quality-of-life trials) may not be labelled this way.

<u>Use of Placebos</u>

The use of placebos as comparison or "control" interventions in cancer treatment trials is rare. If a placebo is used by itself, it is because no standard treatment exists. In this case, a trial would compare the effects of a new treatment with the effects of a placebo. More often, however, placebos are given along with a standard treatment. For example, a trial might compare the effects of a standard treatment plus a new treatment with the effects of the same standard treatment plus a placebo.

<u>Possible benefits of taking part in a clinical trial</u>

The benefits of participating in a clinical trial include the following:

- Trial participants have access to promising new interventions that are generally not available outside of a clinical trial.
- The intervention being studied may be more effective than standard therapy. If it is more effective, trial participants may be the first to benefit from it.
- Trial participants receive regular and careful medical attention from a research team that includes doctors, nurses, and other health professionals.
- The results of the trial may help other people who need cancer treatment in the future.
- Trial participants are helping scientists learn more about cancer (e.g., how it grows, how it acts, and what influences its growth and spread).

Potential harms associated with taking part in a clinical trial

The potential harms of participating in a clinical trial include the following:

- The new intervention being studied may not be better than standard therapy, or it may have harmful side effects that doctors do not expect or that are worse than those associated with standard therapy.
- Trial participants may be required to make more visits to the doctor than they would if they were not in a clinical trial and/or may need to travel farther for those visits.

Correlative research studies, and how they are related to clinical trials

In addition to answering questions about the effectiveness of new interventions, clinical trials provide the opportunity for additional research. These additional research studies, called correlative or ancillary studies, may use blood, tumour, or other tissue specimens (also known as 'biospecimens') obtained from trial participants before, during, or after treatment. For example, the molecular characteristics of tumour specimens collected during a trial might be analysed to see if there is a relationship between the presence of a certain gene mutation or the amount of a specific protein and how trial participants responded to the treatment they received. Information obtained from these types of studies could lead to more accurate predictions about how individual patients will respond to certain cancer treatments, improved ways of finding cancer earlier, new methods of identifying people who have an increased risk of cancer, and new approaches to try to prevent cancer.

Clinical trial participants must give their permission before biospecimens obtained from them can be used for research purposes.

When a clinical trial is over

After a clinical trial is completed, the researchers look carefully at the data collected during the trial to understand the meaning of the findings and to plan further research. After a phase I or phase II trial, the researchers decide whether or not to move on to the next phase or stop testing the intervention because it was not safe or effective. When a phase III trial is completed, the researchers analyse the data to determine whether the results have medical importance and, if so, whether the tested intervention could become the new standard of care.

The results of clinical trials are often published in peer-reviewed scientific journals. Peer review is a process by which cancer research experts not associated with a trial review the study report before it is published to make sure that the data are sound, the data analysis was performed correctly, and the conclusions are appropriate. If the results are particularly

important, they may be reported by the media and discussed at a scientific meeting and by patient advocacy groups before they are published in a journal. Once a new intervention has proven safe and effective in a clinical trial, it may become a new standard of care.
(National Cancer Institute).

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This Fact Sheet is intended to provide general information only and, as such, should not be considered as a substitute for advice, medically or otherwise, covering any specific situation. Users should seek appropriate advice before taking or refraining from taking any action in reliance on any information contained in this Fact Sheet. So far as permissible by law, the Cancer Association of South Africa (CANSA) does not accept any liability to any person (or his/her dependants/estate/heirs) relating to the use of any information contained in this Fact Sheet.

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