Introduction

Being diagnosed with aplastic anaemia (AA) can be a shock to you, particularly when you are unlikely to have ever heard of this condition. If you have any questions about AA, such as what causes it, who it can affect, how it affects your body, what the symptoms are and what the treatments are for it, this booklet will hopefully cover the basics for you.

You will also find useful advice about how to get the best from your haematology service, as well as practical advice on how to help the important people in your life understand such a rare condition. For more information, talk to your haematologist or clinical nurse specialist.

This booklet was originally compiled by Ken Campbell and the rewrite was completed by Lisa Lovelidge. It has since been updated by our Patient Information Writer, Isabelle Leach. It has been peer reviewed by Professor Judith Marsh and Dr. Shreyans Gandhi, King’s College Hospital, London. We are also grateful to patient reviewers Sarah Cheeseman, founder of the Central Line Holder, and Nicholas Topley for their valuable contribution.

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

About Leukaemia Care

Our services

Helpline
Our helpline is available 8:30am – 5:00pm Monday - Friday and 7:00pm – 10:00pm on Thursdays and Fridays. If you need someone to talk to, call 08088 010 444.

Alternatively, you can send a message via WhatsApp on 07500068065 on weekdays 9:00am – 5:00pm.

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

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.leukaemiacare.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.leukaemiacare.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@leukaemiacare.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.leukaemiacare.org.uk.

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@leukaemiacare.org.uk

Patient magazine
Our magazine includes inspirational patient and carer stories as well as informative articles by medical professionals: www.leukaemiacare.org.uk/communication-preferences/
Aplastic anaemia (AA) is a rare, non-cancerous condition in which the stem cells in the bone marrow (the cells responsible for making all mature blood cells) are attacked and destroyed by the patient's own immune system. The cause for this auto immune reaction is often unexplained. In fact, in more than half of patients who develop AA, the reason for this auto immune reaction is not known. AA can be brought on following a viral infection, or exposure to certain chemicals or radiation exposure. In addition, defects in a gene (inherited from your parents) that is responsible for making blood cells may also be an important factor as to why some people develop bone marrow failure and AA.

The stem cells in your bone marrow give rise to all the types of blood cells. But in those suffering with AA, because they become damaged, they cannot produce enough normal blood cells (red blood cells, white blood cells and platelets). When you look at the bone marrow in AA patients (in a bone marrow biopsy) it appears empty or sparsely populated, when it is normally full of cells.

Importantly, a diagnosis of AA excludes other causes for the bone marrow malfunction such as marrow fibrosis (scarring) or leukaemia.

In normal-functioning bone marrow, the blood forming stem cells divide either to replicate more stem cells, or mature into either red cells (erythrocytes that carry oxygen in the blood), white cells (leukocytes that are important in fighting infection) or platelets (that are important for blood clotting).

The production of new blood cells is very closely controlled so that it is balanced with the day-to-day loss of worn-out or damaged cells. The stem cells in the bone marrow mature and develop into either a myeloid cell (myeloblasts) or a lymphoid cell (lymphoblasts).

A myeloid cell develops into one of three types of mature blood cells:

1. Red blood cells carry and deliver oxygen to all the tissues of the body and carry back the waste products from them.
2. Platelets form blood clots to stop bleeding, and initiate healing wherever there is tissue injury.

3. White blood cells fight infection and provide immunity.

A lymphoid cell becomes one of three types of white blood cells called lymphocytes:

1. T-lymphocytes (T-cells) manage immunity and help the B-cells make the antibodies to fight infection.

2. B-lymphocytes (B-cells) make antibodies to help fight infection.

3. Natural killer cells (NK cells) attack cancer cells and viruses.

AA is an abnormality of the stem cells in the bone marrow which makes them incapable of producing sufficient levels of each type of blood cell. AA is not a type of leukaemia, where an abnormality in one of the types of blood cells, generally one of the white blood cells, makes them rapidly proliferate and take over the bone marrow preventing it from producing normal red blood cells or platelets.

Because the levels of red blood cells, white blood cells and platelets are low in patients with AA, they experience symptoms of anaemia and are at increased risk of infection, bruising or bleeding.

When AA was first discovered, the first symptom that was noticed was the low levels of red blood cells; therefore, it was given the term anaemia. However, as further research showed that the majority of patients also had low platelets and white blood cells, the term aplastic, which means ‘failure to develop or function normally’, was added.

Who is affected by AA?

AA is a very rare condition affecting around two people per million per year in the UK. In Europe generally, studies have reported an incidence of AA of two to three per million people per year; however, in Asia the incidence is higher at 5.67 per million people per year.

The average age of patients developing AA is around 60 years.
What is aplastic anaemia? (cont.)

Although AA can occur at any age, there is a small peak in its incidence in adolescence (15 to 20 years of age), with a second peak in people aged over 60 years old. The incidence of AA is similar in men and women.

What causes AA?

There is strong evidence that the damage to stem cells in the bone marrow is started by the patient's own immune system attacking itself and that the patient's own T-lymphocytes are responsible for this. When the immune system attacks the body's own cells, this is called an auto immune reaction. This immune-mediated destruction of the stem cells can occur following a viral infection or following exposure to certain environmental toxins or chemicals. AA also happens in people with inherited genetic mutations in the genes that control blood cell production. More often than not, however, a defined cause for these auto-immune events is never identified.

Inherited or acquired (idiopathic) AA

Inherited AA

Inherited forms of AA usually happen during the first decade of life (i.e. during infancy or childhood) but can also occur rarely in adulthood. Inherited AA results from a defect (mutation) in a gene present since birth. This could have been inherited from one or both parents.

Acquired AA

In the majority of patients with acquired AA there is no defined precipitating event that triggers the immune-mediated destruction of the stem cells. For this reason, it is sometimes called an idiopathic (a condition which arises spontaneously or for which the cause is unknown) or primary acquired AA. The incidence of acquired AA is lower in children.

In a small number of patients, acquired AA is associated with exposure to environmental toxins such as heavy-dose radiation or toxic chemicals. Other causes that have been implicated
include antibiotics, nonsteroidal anti-inflammatory drugs and penicillamine or contact with animal fertilizers or agricultural pesticides.

In rare cases viral infections have also been identified as a potential initiator of AA. In these cases a person’s immune system becomes confused and the response to the virus also results in damage to the blood-forming stem cells.

The Aplastic Anaemia Trust (AAT) is the only charity in the UK solely dedicated to research into Aplastic Anaemia and allied rare bone marrow failures. They also provide practical information and advice to patients and their families, as well as peer-to-peer support. For further information on the latest research and to access information and support, please contact the AAT via their website www.theaat.org.uk
What are the signs and symptoms of acquired AA?

The most common symptoms and signs of acquired AA are those of anaemia (low red cell count), bleeding and easy bruising due to low platelet counts and susceptibility to infections, especially if the white cell (neutrophil) count is very low.

Because acquired AA prevents the bone marrow from producing normal blood cells, patients develop symptoms caused by their lack of red blood cells, white blood cells and platelets. Symptoms can vary greatly between patients depending on the severity of the acquired AA. Most patients are diagnosed having experienced symptoms, while others experience few or no symptoms at all in the early stages of acquired AA. Acquired AA can occur suddenly, or it can develop slowly and worsen over time. It is classified as very severe, severe or non-severe depending on these symptoms and speed of onset.

Patients with acquired AA may have any of the following symptoms or signs according to which blood cells are at particularly low levels:

Low levels of red blood cells.
- Fatigue, tiredness and weakness
- Low energy levels
- Shortness of breath, light-headedness and palpitations

Low levels of white blood cells predispose to infections. Infection with low white cell counts (neutropenia) requires urgent treatment.
- Fever
- Cough, colds and chest infections
- Urinary tract infections
- Sore mouth, including ulcers

Low levels of platelets: Severe bleeding is uncommon but it requires urgent treatment.
- Bruising of the skin
- Nosebleeds
- Bleeding gums
• Vision problems due to bleeding in the retina

• Bleeding from the gastrointestinal tract (anywhere along the gut passages) and the brain can be serious
How is acquired AA diagnosed?

There is no single test that reliably diagnoses acquired AA and the diagnosis is one of exclusion. All alternative causes of bone marrow failure must be excluded as part of the diagnostic evaluation. Tests required to achieve this include:

- Full blood count - This is a routine blood test which measures the number of red cells, different types of white cells and platelets in the blood.

- A blood film examination - The blood is smeared on a microscope slide, allowing the blood cells to be examined under the microscope. In many patients with acquired AA, the number of red blood cells, neutrophils and platelets are decreased; however, the number of lymphocytes are usually normal.

- Bone marrow biopsy - The bone marrow sample can be taken from the hip bone under local anaesthetic, using special biopsy needles: liquid bone marrow (aspirate) and a tiny core of bone marrow tissue (trephine). A bone marrow biopsy is mandatory for a diagnosis and will show an empty or sparse marrow which usually contains fat cells.

- Tests for gene mutations - Blood or bone marrow tests may be performed to check for mutations to exclude inherited AA and also somatic (acquired) mutations related to the patient’s external causes. Somatic mutations are acquired, non-inherited mutations which are related to environmental factors. In acquired AA, the most common somatic mutations can be found in the following genes:
  - PIGA (phosphatidylinositol glycan class A)
  - ASXL1 (additional sex combs like 1)
  - BCOR (BCL6 corepressor)
  - DNMT3A (DNA [cytosine-5]-methyltransferase 3 alpha)

To diagnose acquired AA, at least two of the following criteria must be met in the bone marrow.
sample:
• Haemoglobin <100g/L
• Platelets <50g/L
• Neutrophils <1.5g/L
• Bone marrow with few cells (hypocellular), but no abnormal cells or fibrosis.

Hypocellular bone marrow (as in low cell number) is defined as:
• Bone marrow cells <25%

or
• Bone marrow cells between 25% and 50%, with less than 30% of stem cells

Fat cells are still present in the bone marrow and a fatty bone marrow remains crucial to the diagnosis.

Classification of acquired AA

Based on the results of the diagnostic tests, acquired AA can be classified as non-severe, severe or very severe. This is based on how low the numbers of blood cells have fallen.

Non-severe acquired AA
Meets the above diagnostic criteria for acquired AA, but does not meet the criteria for severe or very severe acquired AA.

Severe acquired AA
A hypocellular bone marrow and any two of the following criteria:
• A low platelet count (less than 20x10⁹/L)
• A reticulocyte count less than 60x10⁹/L
• A neutrophil count less than 0.5x10⁹/L

Very severe acquired AA
A hypocellular bone marrow and any two of the following criteria:
• A low platelet count (less than 20x10⁹/L)
• A reticulocyte count less than 60x10⁹/L
• A neutrophil count less than 0.2x10⁹/L

A reticulocyte is a cell made by the
bone marrow and released into the blood. Within a couple of days of its release, the reticulocyte matures into a red blood cell. The normal range for the reticulocytes in the blood is 50x10⁹/L to 100x10⁹/L.

The difference between severe and very severe acquired AA is how low the number of neutrophils falls. The number of neutrophils is used to indicate low white cell production as they represent the greatest proportion of white blood cells (60% of white blood cells). In acquired AA, the numbers of lymphocytes, normally only 25% of white blood cells, are not usually reduced below normal levels. The lower the number of neutrophils, the greater the risk of severe infection.
What is the treatment for acquired AA?

The classification of acquired AA into non-severe, severe or very severe is used as a general guide for treatment choice. Aggressive treatment is usually required for severe, or very severe acquired AA, whereas patients with non-severe acquired AA can be kept under observation at first. Non-severe AA patients can be considered for treatment if they are red cell transfusion (blood transfusion) dependent or symptomatic with bleeding or have frequent infections.

If you have non-severe acquired AA, you may not require any treatment initially and a watch and wait approach is often recommended at first. Watch and wait usually involves regular check-ups and blood tests, as well as advice on how to maintain a healthy lifestyle. Your medical team will discuss your treatment options with you. As well as the severity of your acquired AA, your age, general health and availability of a bone marrow donor are important considerations for the selection of treatment.

For all patients with acquired AA, the possible cause of the acquired AA should always be sought, particularly in the case of a drug the patient may be taking or exposure to fertilizers or pesticides. The suspected cause should be removed immediately.

Treatment of patients with acquired AA is aimed at:

- Replacing or renewing the depleted stem cell pool (such as with stem cell transplantation)
- Controlling the damage to bone marrow stem cells (immunosuppressive and anti-thymocyte therapy)
- Stimulating the remaining bone marrow stem cells
- Providing supportive treatment for anaemia, bleeding or
infections

The guidelines for acquired AA recommend allogeneic stem cell transplantation (Allo-SCT) or immunosuppressive therapy (IST) comprising of anti-thymocyte globulin (ATG) with ciclosporin (CsA) as initial treatment for acquired AA patients.

**Allogeneic stem cell transplant (Allo-SCT)**

An allogeneic stem cell transplant (Allo-SCT) is currently the only treatment that can potentially cure acquired AA. If you are being considered as a candidate for a stem cell transplant, any potential donors will be checked to see if they are a match. This does not mean that your doctors have already decided you should have a transplant, it is to save time if this turns out to be your best treatment option.

Allo-SCTs are usually only considered as an upfront treatment option for young, fit patients with a fully matched sibling donor, but may be considered for anyone who is suitable depending on the circumstances. For example, with advances in treatment and care, a matched unrelated donor (MUD) allo-SCT can be a potential treatment option for those who do not have a matched sibling donor. Alternatively, an allo-SCT also remains an important treatment option for patients with AA who fail to show response or relapse after initial rounds of immunosuppressive treatment.

Your haematology team will discuss with a specialist centre in the management of AA when reviewing these options.

All treatment protocols are approved by the European Bone Marrow Transplantation Severe Aplastic Anaemia Working Party.

If an allo-SCT is the best option for you, you will first receive chemotherapy or radiation therapy to kill all the remaining stem cells in your bone marrow before receiving the healthy donor stem cells. For young patients and those who can withstand it, they will receive the preliminary high-intensity chemotherapy and radiotherapy. In older patients the chemotherapy can be less
What is the treatment for acquired AA? (cont.)

intense (low intensity-transplant) and radiotherapy is not usually necessary.

An allo-SCT will help restore your bone marrow using the stem cells from your donor. The healthy stem cells are given to you intravenously, from where they migrate to your bone marrow, populate it and start forming new blood cells. After the transplant, you will receive drugs to help prevent rejection (immunosuppression) of the donated stem cells. The procedure requires you to stay in a hospital for four to six weeks.

An allo-SCT does have risks such as your body rejecting the transplanted cells and other life threatening complications such as bleeding or infections, and the stem cell transplant centre will have a detailed discussion with you and your friends/family about every aspect before proceeding.

If you would like more information, you can get a copy our booklet on Allo-SCT by downloading it at www.leukaemiacare.org.uk, emailing us at support@leukaemiacare.org.uk or calling 08088 010 444.

Immunosuppression

Immunosuppression involves using drugs to control the activity of the immune system and reduce the damage being done to marrow stem cells.

Immunosuppressive therapy (IST) is an effective treatment and can improve the quality of life for patients with acquired AA. Although very few show a complete response, the majority (~70%) will show a response and have an improvement in their blood counts and symptoms. This results in a symptomatic improvement reflected in freedom from blood transfusions and a reduced risk of infection or bleeding episodes.

Immunosuppressive treatment takes at least three and up to
six months and beyond before blood cell counts start to rise (i.e. you see clinical response). Your response at three to four months will be assessed and your doctors will decide whether to continue with ciclosporin or to change to other treatments.

The first-line treatment for acquired AA is an anti-thymocyte globulin (ATG) and ciclosporin (CsA) which can achieve a response in about 70% of patients with acquired AA.

- Anti-thymocyte globulins are antibodies directed against T-cells and especially those that drive the auto-immune response. ATG removes the T-lymphocytes responsible for causing acquired AA.

- Ciclosporin is one of the main immunosuppressive drugs commonly used to help prevent organ rejection in transplant patients. It stops the T-lymphocytes being activated and prevents organ rejection.

When an ATG is administered with CSA, a steroid drug called prednisolone may also be administered to control any possible side effects of ATG.

Ciclosporin is normally continued for a minimum of 12 months, and then if possible, the dose is decreased over several months. The majority of patients show a response to Immunosuppressive treatment. However, if your acquired AA does not respond, tests will be performed, including repeat bone marrow tests, to decide on a further course of action. Treatment options include other forms of immunosuppression, anabolic steroids and drugs that stimulate stem cells.

**Stem Cell Stimulation**

There have been many attempts to improve the response to ATG by adding stem cell stimulators such as androgens (hormones), granulocyte colony stimulating factor, or using alternative immunosuppressive drugs such as mycophenolate or rapamycin. They have shown very modest activity and have not shown significant changes in response rates or long-term outcomes in patients with acquired AA.

**Eltrombopag**

A synthetic mimetic compound of
What is the treatment for acquired AA? (cont.)

Thrombopoietin (a growth factor needed by the stem cell), called eltrombopag, has shown some promise, both in patients who have been refractory to previous treatment of IST, as well as new patients where eltrombopag has been added to conventional IST (ATG + CsA).

Thrombopoietin is a hormone produced by the liver and kidney which regulates the production of platelets and is known to be a regulator of blood cells production in the bone marrow. Addition of Eltrombopag to standard immunosuppression markedly increased the overall response rate in patients with acquired AA to around 90%.

In patients deemed unsuitable for intensive treatment with either allo-SCT or IST, immunosuppression treatment can be limited to a single agent, either Eltrombopag, or Ciclosporin. Eltrombopag increases platelet counts and activates the proliferation and differentiation of bone marrow cells. Eltrombopag is now approved for treatment of adult patients with acquired AA who are either unresponsive to initial treatment or to prior immunosuppressive therapy or those heavily pre-treated and are unsuitable for allo-SCT.

Eltrombopag may be a potential treatment if a repeat of standard immunosuppressive treatment or a stem cell transplant are not considered good options. It may also be preferable for patients with low kidney function.

**Alemtuzumab**

Alemtuzumab (a monoclonal antibody), also an immunosuppressive drug, has been used as a single agent in treatment-naive, relapsed (initial response to treatment, but response then stops), and refractory severe acquired AA. Alemtuzumab has shown some modest response in severe acquired AA. Slightly better responses were seen in the relapsed and refractory patients with severe acquired AA. However, as it is profoundly immunosuppressive, it significantly increases the risk of other infections, particularly viral.

Your haematology team will carefully consider this treatment option, often in consultation with a specialist centre, to see
if the merits outweigh the risks associated with Alemtuzumab.

**Supportive care**

Whether receiving active treatment or not, the majority of patients with acquired AA will require supportive treatment to address any anaemia, bleeding and to prevent or control infections. If this applies to you, your healthcare team will discuss the situation in detail and explain what is planned.

There are three areas in which patients with acquired AA require supportive care:

**Anaemia**

While they are not a cure for acquired AA, blood transfusions can control bleeding and reduce symptoms caused by lack of blood cells. Blood transfusions will help raise the red blood cell counts and help improve anaemia and symptoms associated with this – namely fatigue, tiredness, shortness of breath and low energy levels.

Red blood cell transfusions are recommended to enable patients to carry out normal daily activities. If a patient needs to have a large number of red cell transfusions over many months, they may have a build-up of iron in the body. This can be managed with drugs that help the body to get rid of excess iron (iron ‘chelation’ therapy).

**Bleeding**

Bleeding problems in acquired AA are caused by low platelet levels. Regular platelet transfusions may be needed if the platelet count falls below 10x10⁹/L, or in some instances below 20x10⁹/L if the patient has a fever and/or infection or active bleeding. A higher platelet level is achieved with regular platelet transfusions for the duration of in-patient treatment with an ATG.

If a patient with severe or very severe acquired AA needs surgery, then the surgical team will work with the haematology team to plan platelet cover and any other measures that may be needed to prevent or control bleeding.

Your local haematology team will discuss and have a transfusion plan in place that best suits your clinical condition and circumstances.
Infections

Infection in patients with acquired AA can be a serious problem and cause for concern. Any fever or infective symptoms associated with such a neutrophil count defines neutropenic sepsis. This needs prompt medical attention, without any delay. Contact numbers for the Haematology team when this happens, as well as pathway for attending your nearest A&E/Haematology centre, will have been discussed and provided to you. Patients are also provided ‘a neutropenic state’ bracelet/wristband that they can wear.

When neutrophil counts are 0.5x10⁹/L prophylactic antibiotics and antifungal drugs are often given to reduce the risk of infection. When receiving immunosuppressive therapy, patients with acquired AA should also receive anti-viral drugs. You will be given detailed advice on precautions to reduce the risk of infection.

If you develop a fever or any other symptoms that might indicate infection, it is important that you contact your doctor or clinical nurse specialist immediately as early treatment is necessary.

What is the prognosis of acquired AA?

In the last 20 years, the prognosis for patients with acquired AA has been enhanced by the availability of better treatments, better understanding of the condition and the continued use of supportive measures.

Survival of patients with acquired AA is very much dependent on their age, the severity of their acquired AA at diagnosis, and how they respond to first therapy.

For patients who are suitable to undergo an allo-SCT, the five-year survival following an allo-SCT from a suitable donor is greater than 90%. Immunosuppressant treatment with ATG+CSA has shown encouraging results with overall response rates around 70% and a long-term survival of 80% to 90%.

A first course of ATG+CSA can result in a response rate of around 70% after six months. However, following relapse, response from a second course is around 55% to 60%. The risk for a
relapse in patients at 10 years is approximately 30%.

In a study of patients with mainly severe or very severe acquired AA, five-year survival rate was 96% in patients who had an allo-SCT, 68.9% in patients who had immunosuppressant treatment, and 29.6% in patients who received ciclosporin alone. Irrespective of the treatment they received, the five-year overall survival was superior in the younger patients ranging from 90.7% in patients less than 18 years to 38.1% in patients aged 60 years and over.

Patients with acquired AA who recover following IST have been shown to have a good prognosis. In addition, there is data to suggest that acquired AA patients with the mutations in PIGA, BCOR and BCORL1 genes have better prognoses than those with mutations in DNMT3A and ASXL1 genes.

In terms of long-term complications, amongst acquired AA patients receiving IST, approximately 10% will have clonal evolution to MDS and 7% will progress to AML.

MDS occurs when the bone marrow does not make enough normal functional blood cells. The blood cells that are made are immature and do not work normally (dysplasia). AML is a rapid and aggressive cancer of the myeloid cells in the bone marrow.

In addition, between 40% and 50% will show emergence of a PNH clone, of whom only 10% will have a clone large enough of clinical significance. PNH is a rare blood condition characterized by destruction of the blood cells which occurs within the blood vessels, an abnormal tendency to develop blood clots, and bone marrow failure, caused by a somatic (acquired) mutation in the PIGA gene.

Your haematology team will ensure long-term follow up and monitor you, even after you have shown response to treatment.
After a diagnosis of acquired AA, you may find that it affects you both physically and emotionally. This chapter will talk about both of these aspects.

**Emotional impact of acquired AA**

Some of the symptoms of acquired AA can be hard to cope with and, because of this, you WILL need emotional, as well as practical support. Being diagnosed with a rare disease can affect the whole of you, not just your body, and it can impact you emotionally and financially/professionally at any point of your journey. It will also impact on your family and friends. It is likely that you will experience a range of complex thoughts and emotions, some of which may feel strange, unfamiliar and scary. It is important to know that these feelings are all valid and a normal response to your illness. It is also important to know that many people have been through the same situation and that there is help and experience available to support you.

“There was a total overwhelming feeling of helplessness and being out of control of my normal everyday life. But I had to carry on regardless for everyone else.”

Finding out you have AA may be overwhelming to hear, and you may experience a wide range of emotions. There are a number of places that you can seek support from:

- **Your medical team** will be there to assist you with your treatment pathway. They can be a great source of medical and supportive information specifically relating to your AA. They will also be able to signpost you to further support services, including counselling that may be provided by the hospital, or your local council.

- **Family and Caregivers** can be there for you at home. They can also attend hospital appointments with you. Having someone else there to take in all the information can help things seem less vast.

- **Charities and organisations**, including Leukaemia Care, offer a number of services that aim to offer some comfort. You can call our helpline on 08088 010
Looking after you

Following a diagnosis of acquired AA, you may want to make changes to your lifestyle to try to stay as well as possible. Do not try to change too much at once. Adopting a healthy way of living is about making small, manageable changes to your lifestyle.

A healthy lifestyle includes having a well-balanced diet and being physically active. With some of your side effects, the idea of getting out and being active may be the last thing you want to do, but it is important to try and stay as active as possible to make you feel better and reduce some of your symptoms or side effects.

One of the most commonly reported side effects of the treatment of acquired AA is fatigue. This isn’t normal tiredness and doesn’t improve with sleep.

Some general tips on how to deal with fatigue include:

- Have a regular lifestyle - try going to bed and waking up at approximately the same time every day and try to avoid lying in.

- Take part in regular, gentle exercise to maintain your fitness levels as much as possible.

- Reserve your energy for what you find important and build rest periods around those times.

- Before going to bed, avoid stimulants such as alcohol, coffee, tea or chocolate, or using laptops, tablets or mobile phones.

- Keep your bedroom quiet and at a comfortable temperature.

- Talk about your worries with family, friends, your doctor or nurse, or in-patient support groups.
Living with acquired AA (cont.)

• Discuss your fatigue with your doctor or nurse.

"Don't get me wrong, it's hard living with a chronic condition. I get tired a lot and have to be careful about picking up infections, as my immune system is lower than others. But all in all, I will not let it take over my life. I don't intend on fighting it; it will have to fight me."

Practical support

Work and finances

Being diagnosed with AA can sometimes lead to difficulties relating to your work life. Your diagnosis may lead to temporary sick leave or a reduction in working hours but it can also mean that you have to stop work altogether. You may need to make an arrangement with your employer for times when you may need to go into hospital or for those times when you may not be well enough to go into work.

Your consultant or your GP can arrange letters to your employers to confirm your diagnosis and the effects it may have on your work life. It is often worth taking time to explain AA to your employer, as it is likely they will never have heard of the disease.

It is important for you to know that people with aplastic anaemia are covered by law by the Equality Act. This means that legally your employer cannot discriminate against you and must make reasonable arrangements and adjustments relating to your disease.

If you would like advice about some of the financial help available to you, then you can speak to our Patient Advocacy team on 08088 010 444.

You can find more information about living well with leukaemia at www.leukaemiacare.org.uk/support-and-information/information-about-bloodcancer/living-well-withleukaemia/

Alternatively, if you’re struggling to come to terms with your diagnosis and prognosis, you can speak to us on our helpline. Call us on 08088 010 444.
Alternatively, Macmillan has published a booklet about financial support following a diagnosis that might be useful to you. They can also give you personal advice over the phone via their helpline at 0808 808 0000 and you can discuss which benefits you are eligible for. Some Macmillan centres can arrange face-to-face meetings with a benefits advisor. They can also provide financial assistance in the form of grants – ask your nurse in the hospital how to apply.
Talking about acquired AA

Talking to your haematologist

Acquired AA is a rare condition. It is important for you to develop a good working relationship with your haematologist, so you are given the best treatment possible for you.

The following gives advice on working well with your haematologist:

- If it is an initial consultation, take along a list of your current medications and doses, and a list of any allergies you may have
- If you have a complicated medical history, take a list of diagnoses, previous procedures and/or complications
- Make a list of questions (on paper) to take to your appointment. This will help the discussion with your haematologist
- It can be useful to repeat back what you have heard so that you can be sure that you fully understood
- Note information down to help you remember what was said
- Be open when you discuss your symptoms and how you are coping. Good patient doctor communication tends to improve outcomes for patients
- Take a family member with you for support and understanding
- Do not be afraid to ask for a second opinion – most haematologists are happy for you to ask

You need to tell your haematologist if...

You’re having any medical treatment or taking any products such as prescribed medicines, over the counter treatments or vitamins. It is important to understand that treatments, including complementary therapies, which are perfectly safe for most people, may not be safe if you are being treated for acquired AA.

Remember, if you choose to start any form of complementary therapy outside of your medical treatment, discuss this with your haematology consultant or clinical nurse specialist, prior
to beginning it. It is important to understand the difference between complementary therapies, used alongside standard treatment, and alternative therapies, used instead of standard treatment. There is no evidence that any form of alternative therapy can treat acquired AA.

**Talking to other people**

Telling people you have a rare condition like acquired AA can be hard to explain. You might find it useful to let your close family and friends, as well as your employer know about your health condition. It might be easier to provide people with basic information and give them information leaflets about acquired AA if they want to know more in-depth details. It is nothing to be ashamed of!

"I made a conscious decision to be very open about my illness. Telling family was tough. But I encouraged people to ask questions."

It is probably best to focus conversations on the symptoms that you are experiencing, how the condition affects you and how you feel about it. Be as objective as you can during such communication. Where possible, it is advisable to let people know what you find helpful and unhelpful, in terms of what others say and do. Often people make assumptions and do what they think helps. For example, saying you look well (or saying you look pale!), recounting stories of others they know with a similar diagnosis, encouraging you to look ahead and stay positive is not always what people really want to hear. In many ways, the more you communicate with them the better.

These points may help you:

- Explain that you have a condition that means your bone marrow does not function properly, and that this affects the number of blood cells it produces
- Explain your symptoms (maybe you are tired, or have a lot of pain)
- Explain what you need (maybe more help day-to-day, or someone to talk to)

You could also consider the following when telling people
Talking about acquired AA (cont.)

- **Find out more** - Try to find out as much as you can about your condition from reliable internet sources, charitable organisations or your consultant haematologist. The more you know, the more you can share.

- **Have a print-out to hand** - It may help to have some information to hand to share with family and friends. This will take the pressure off you having to remember everything they may want to know.

- **Explain your needs** - Try and be clear about what your needs may be. Perhaps you need help with the weekly food shop, help with cooking dinner, or someone to drive you to and from appointments. You may find that friends and family are pleased that they can do something to help you.

- **Be open about how you feel** - Do not be afraid of opening up about how you feel, as people who care will want to help you as best they can. Talk as and when you feel comfortable, so those around you will know when you need them most. Talk to other patients and families in clinic/day unit, they are a key source of support, an important part of surviving the treatment. Talk to the nurses in the day unit, they have seen it all and provide reassurance when it gets hard but you can get through it.

If you’re struggling to come to terms with your diagnosis and prognosis, you can speak to us on our helpline. Call us on 08088 010 444.
Glossary

Acute Leukaemia
Leukaemia which progresses rapidly and is generally aggressive. There are two types: acute lymphoblastic leukaemia and acute myeloid leukaemia.

Acute Lymphoblastic Leukaemia (ALL)
Leukaemia in which lymphocytes start multiplying uncontrollably in the bone marrow, resulting in high numbers of abnormal, immature lymphocytes. Lymphocytes are a type of white blood cell involved in the immune response.

Acute Myeloid Leukaemia (AML)
Rapid and aggressive cancer of the myeloid cells in the bone marrow.

Allogeneic Stem Cell Transplant (allo-SCT)
Transplant of stem cells from a matching donor.

Amino Acids
Organic molecules which are the building blocks for making proteins.

Anaemia
Condition where the number of red blood cells are reduced. Red blood cells contain haemoglobin and transport oxygen to body cells. This may be due to a lack of iron, leukaemia, or sickle cell disease.

Antibiotic
Drug used to treat or prevent bacterial infections.

Antibody
Large Y-shaped protein produced by B-cell lymphocytes in response to a specific antigen, such as a bacteria, virus, or a foreign substance in the blood. The antibodies neutralise the bacteria and viruses.

Antigen
Toxin or other foreign substance which induces an immune response in the body, especially the production of antibodies.
Autologous Stem Cell Transplant (ASCT)
Transplant of stem cells derived from part of the same individual.

Blood Cancer
Cancer of blood cells from the bone marrow or lymphatic system. There are three main types of blood cancer:

- Leukaemia begins in the bone marrow and is classified according to the type of blood cell it affects (either myeloid or lymphoid) and whether it grows quickly (acute) or slowly (chronic).

- Lymphoma starts in the lymphocyte white blood cells within the lymphatic system.

- Myeloma is a cancer of the plasma cells and starts in the bone marrow. Plasma cells are a type of white blood cell that makes antibodies.

Blood Cells
Cells present in the blood and bone marrow which include red blood cells, white blood cells and platelets. These three types of blood cell make up 45% of the blood volume, with the remaining 55% being plasma, the liquid component of blood.

Bone Marrow
Soft blood-forming tissue that fills the cavities of bones and contains fat, immature and mature blood cells, including white blood cells, red blood cells and platelets.

Bone Marrow Aspirate
Bone marrow aspirates consist of taking a sample of the liquid part of the soft tissue bone marrow inside your bones using a syringe. They are crucial to establish a diagnosis of leukaemia and may be performed at stages during treatment to monitor progress.

Bone Marrow Biopsy
Bone marrow biopsy involves the collection of a sample of bone marrow from the hip bone, generally under local anaesthesia. A bone marrow surgical instrument with a cylindrical blade, called trephine, is used to
remove a 1 or 2 cm core of bone marrow in one piece.

**Bone Marrow Failure**
Term used when the bone marrow is unable keep up with the body's need for white and red blood cells and platelets.

**Bone Marrow Relapse**
Bone marrow relapse is defined as the presence of greater than 25% of leukaemia cells in a bone marrow aspirate following the first complete remission.

**Chemotherapy**
Drugs that work in different ways to stop the growth of cancer cells, either by killing the cells or by stopping them from dividing.

**Chromosomes**
Thread-like structures which carry the genes, and are located in the nuclei of every cell in the body. There are 46 chromosomes (23 pairs) in humans.

**Clinical Trial**
Trial designed and planned to determine a specific answer or aim; for example, whether treatment A is better than treatment B. The study will be conducted in patients who meet particular inclusion criteria, and the results are collected and analysed to provide an answer.

**ClinicalTrials.gov**
ClinicalTrials.gov is a database of trials and includes details of approximately 276,190 research studies in 205 countries.

**Clonal**
Refers to an organism descended from, and genetically identical, to a single common ancestor.

**Corticosteroids (Steroids)**
Hormones normally produced by the adrenal glands which are two small glands found above the kidneys. Corticosteroids reduce inflammation (redness and swelling) and the activity of the immune system. They are used for inflammatory conditions such as asthma and eczema and autoimmune diseases such as rheumatoid arthritis.
DNA (Deoxyribonucleic Acid)
Thread-like chain of amino acids found in the nucleus of each cell in the body which carries genetic instructions used in the growth, development and functioning of the individual's cells.

Eosinophil
Type of white blood cell which has a protective immunity role against parasites and allergens.

Erythropoiesis Stimulating Agents
Drugs which stimulate the bone marrow to make red blood cells.

Fatigue
Tiredness and weakness rendering the patient unable to work or perform usual activities.

First-line Treatment
First treatment given for a disease. It is generally the treatment accepted by the medical profession as the best initial treatment for a given type and stage of cancer.

Genes
Genes are made up of DNA which stores the genetic information required to make human proteins.

Granulocyte-Macrophage Colony-Stimulating Factor (GM-CSF)
Growth factor required to stimulate the growth of living cells.

Granulocytes
Group of white blood cells, which have granular bodies in their cytoplasm. They include the neutrophils, eosinophils and basophils white blood cells, all of which protect the body from bacteria, allergens and inflammation.

Haematology
Branch of medicine which studies the cause, prognosis, treatment, and prevention of diseases related to blood.

Haemoglobin
Red protein contained within the red blood cells and responsible for
transporting oxygen to the tissues of the body.

Incidence
Number of new cases of disease which can be reported as an incidence rate or a risk.

Leukaemia
A group of cancers that usually begin in the bone marrow and result in high numbers of abnormal blood cells. These cells are not fully developed and are called blasts or leukaemia cells. Depending on the type of blood cell involved, there are different types of leukaemia with varying characteristics, such as acute (develop quickly) or chronic (develop slowly).

Lymphocyte
Type of white blood cell that is vitally important to the immune response. There are three types of lymphocytes: B-cells, T-cells and natural killer (NK)-cells. B-cells produce antibodies that seek out invading organisms. T-cells destroy the organisms that have been labelled by the B-cells, as well as internal cells that have become cancerous. NK-cells attack cancer cells and viruses.

Lymphoid
Relates to lymphocyte white blood cells.

Macrophage
Type of white blood cell that submerges and digests cellular debris, foreign substances, microbes, cancer cells, and anything else that does not have the type of proteins specific to healthy body cells on its surface.

Megakaryocyte
Large cell in the bone marrow which produces the platelets in the blood to prevent bleeding.

Monocyte
White blood cell that attacks invading organisms and helps combat infections.

Mutation (gene)
Permanent alteration in the DNA sequence of a gene, so that it
differs from what is found in most people.

**Myeloid**
Relates to bone marrow.

**Neutrophils**
White blood cells involved in fighting inflammation and infection specifically bacterial infections.

**Palliative Care**
Also known as supportive care, this is a type of care that focusses on improving the quality of life for a patient with a life-threatening illness and their loved ones.

**Plasma Cell**
Type of white blood cell that produces antibodies and is derived from B-cells. It is an ovoid (egg-shaped) cell with an off-centre nucleus.

**Prognosis**
Indication of how well a patient is expected to respond to treatment based on their individual characteristics at the time of diagnosis or other timepoint in the disease.

**Proliferation**
Rapid increase, for example in the number of cells.

**Radiation**
Release of energy in the form of particles or waves.

**Radiation Treatment**
Cancer treatment that uses high doses of radiation to kill cancer cells and shrink tumours.

**Red Blood Cells**
Small blood cells that contain haemoglobin and carry oxygen and other substances to all tissues of the body.

**Refractory**
A condition for which treatment does not result in a remission. However, the condition may be stable.

**Relapse**
Relapse occurs when a patient initially responds to treatment, but after six months or more, the response stops. This is also
sometimes called a recurrence.

**Second-line Treatment**

Treatment other than the type used the first time (first-line treatment).

**Stem Cell**

Most basic cell in the body that has the ability to develop into any of the body’s specialised cell types, from muscle cells to brain cells. However, what makes these stem cells reproduce uncontrollably, as in cancer, is thought to be linked to chromosome abnormalities.

**Stem Cell Transplant**

Transplant of stem cells derived from part of the same individual or a donor.

**Watch and Wait**

Management approach for slow growing blood cancers. Also called active monitoring, the Watch and Wait approach is the current standard of care for patients with slow growing blood cancers who do not have any symptoms. Treatment is usually started either once symptoms appear or when test results suggest the blood cancer is progressing.

**White Blood Cells**

White blood cells are one of the types of cells found in the blood and bone marrow, along with red blood cells and platelets. White blood cells create an immune response against both infectious disease and foreign invaders. Granulocyte white blood cells include the neutrophils (protect against bacterial infections and inflammation), eosinophils (protect against parasites and allergens) and basophils (create the inflammatory reactions during an immune response). Other white blood cells include the lymphocytes (recognise bacteria, viruses and toxins, to which they produce antibodies) and monocytes (clear infection products from the body).
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.leukaemiacare.org.uk
support@leukaemiacare.org.uk

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

0808 2080 888
www.bloodcancer.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

Leukaemia Care,  
One Birch Court,  
Blackpole East,  
Worcester,  
WR3 8SG

Leukaemia Care is registered as a charity in England and Wales (no.1183890) and Scotland (no. SCO49802).  
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