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to  
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to  
hope



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Issue Date: January 2015  
Review Date: January 2016

registered charity number 1106008



PRACTICAL INFORMATION  
FOR MALES WITH  
ASYMPTOMATIC ALD



# PRACTICAL INFORMATION FOR MALES WITH ASYMPTOMATIC ALD

## Introduction

You, your son or male relative has been diagnosed with asymptomatic adrenoleukodystrophy (ALD). ALD is an extremely rare genetic illness that mainly affects boys aged four to ten, but can also affect male teenagers and adult men. 'Asymptomatic' means that symptoms of the disease have not yet developed.

Males with asymptomatic ALD have the potential to develop symptomatic ALD, but it is not possible to tell whether this will happen or not. They might also be affected later in life by a related, but less severe condition called adrenomyeloneuropathy, AMN. You will find more information about all these things in this leaflet.

Symptomatic ALD is a disabling and eventually life-threatening condition, but in many cases it can be halted by a bone marrow transplant if the onset is discovered early enough.



This booklet of practical information has been put together by people who have been in your situation and understand how difficult it is. The medical information, presented in shaded boxes, has been carefully compiled from reputable sources (listed on p.19) and has been reviewed by ALD specialist doctors.

ALD Life is Britain's leading ALD charity, run by people with personal experience of ALD and AMN. We were founded to help and support people in your position. Our members have been through the same ordeal and have experienced the problems you now face.

Our members have helped compile this leaflet, which is packed with information patients in your position wish they had been told.

We must stress there is no right or wrong way of dealing with ALD and AMN, but in researching this booklet, we have spoken to many who are in your position and have collated much of the information they wished they had been given to help them through the first stages.

We have a worldwide contact list of people who are willing to chat about all aspects of living with the disorder.

Please contact ALD Life on 020 7701 4388 or email [info@aldlife.org](mailto:info@aldlife.org). Our website is [www.aldlife.org](http://www.aldlife.org).

## PRACTICAL INFORMATION FOR MALES WITH ASYMPTOMATIC ALD

“

IT WAS A RELIEF TO FIND  
A RELIABLE & TAILORED  
INFO CENTRE LIKE  
ALD LIFE.

”

### What is ALD?

Adrenoleukodystrophy (ALD) is caused by a genetic fault that means those affected are unable to process Very Long Chain Fatty Acids (VLCFAs). These VLCFAs accumulate and destroy the myelin sheath that covers the nerves in the body and brain. The myelin acts like the coating around an electric cable, and allows messages to be transmitted efficiently along nerve cells, so if the myelin is damaged the nerve cells cannot work properly.

The gene at fault in ALD is called ABCD1. This can be changed slightly (called mutation of the gene) or a whole piece of the gene may be missing (called a deletion). However, the situation is rather complicated because changes in this gene can cause a family of related but different conditions which affect people in varying ways and at different times in their lives. One of these is adrenal insufficiency (sometimes called Addison's Disease), another is cerebral ALD and a third is AMN (adrenomyeloneuropathy). A minority of

people with changes in the ABCD1 gene may not develop any of these problems. Unfortunately, it's not possible to predict how changes in the gene will affect any one person.

Cerebral (symptomatic) ALD is the most severe form of the disease – we have a separate booklet for families living with this condition. In cerebral ALD the myelin in the brain is gradually destroyed. The nerves in the brain no longer work properly, and the person's physical and mental abilities begin to deteriorate. Functions such as reasoning, speech and mobility are lost. Eventually most people with cerebral ALD will become completely dependent on their carers although rarely the disease can stop worsening at an early stage – this is called “arrested cerebral ALD”.

In most people with ALD the adrenal glands are also affected. These glands make steroid hormones. Poor functioning of these glands is known as adrenal insufficiency. Symptoms include weakness, tiredness, vomiting and muscle pains.

This is discussed further in the Addison's Disease or adrenal insufficiency section on page 8.

The other condition caused by the faulty ABCD1 gene affects the nerves and spinal cord of adults and is called AMN. This is a slowly progressive condition. The main symptoms are stiffness, weakness and pain in the legs. Some men with AMN will go on

to develop cerebral ALD as well. Women who are carriers of an abnormal ABCD1 gene can also develop AMN. Separate booklets are available on AMN in men and women.

### What is the outlook for boys with asymptomatic ALD?

It is thought that about 65 % of males with an abnormal ABCD1 gene will eventually develop cerebral ALD, either in childhood or later. Many of the others will develop AMN at some point in their life. AMN can start to appear any time between adolescence and the forties or later. About half of men with AMN will experience adrenal insufficiency.

Once symptoms of cerebral ALD are well established, there is currently no cure. However, it may be possible to prevent boys with asymptomatic ALD from developing full cerebral ALD if the onset is detected early enough. Because of this, your son will need to be closely monitored for early signs of symptomatic ALD.

“

AT FIRST WE WERE  
ABSOLUTELY TERRIFIED  
AND CONVINCED HE  
WAS GOING TO DIE. BUT  
ONCE WE UNDERSTOOD  
THE CONDITION AND  
HOW WE COULD TRY  
TO PREVENT IT OUR  
WORRIES LESSENER  
CONSIDERABLY.

”



## PRACTICAL INFORMATION FOR MALES WITH ASYMPTOMATIC ALD

### Testing the rest of the family

A patient will usually be diagnosed with asymptomatic ALD because a brother has been diagnosed with symptomatic ALD, or there is an incidence of AMN or ALD in the family. Sometimes a patient is initially diagnosed with Addison's disease or adrenal failure, which is an indicator of ALD.

Once one member of a family has been diagnosed with the ALD gene, it is very important to get the rest of your family tested, especially if there are brothers (ALD usually only affects males, but females can be carriers of the gene, and can pass it on to their children). If the gene is discovered early, before symptoms appear, there is a chance of preventing ALD from developing in affected boys.

You should be offered genetic counselling as soon as the diagnosis of ALD has been confirmed. The genetic counsellor will help you make an informed decision on who and when to test.

When a boy is diagnosed with ALD it will most likely be inherited from his mother. All the daughters of an affected man will be carriers.

### Explaining the genetics of ALD

ALD is an X-linked disorder, which means that the genetic abnormality involves the X-chromosome.

Women have two X-chromosomes. In women who carry the ALD gene mutation on one X-chromosome, the full-blown disease does not appear because there is a normal copy of the gene (see figure 1) on the other X-chromosome.

Men have one X-chromosome and one Y-chromosome (Figure 2). In men who have ALD on their X-chromosome, there is no other X-chromosome for protection; therefore the male can develop symptomatic ALD.

For reasons we don't understand, not all males who have the gene are affected in the same way. Some develop cerebral ALD as boys, others have no symptoms for many years and then develop AMN as adults. Some males develop Addison's disease (adrenal insufficiency) rather than ALD or AMN. Most women with the gene will also develop some, usually mild, AMN symptoms later in life.

Figure 1:

If a woman is a carrier for ALD she has the following possible outcomes with each newborn: With a daughter, there is a 50 % chance (1 in 2) that the daughter is a carrier of ALD and a 50 % chance the child is unaffected. Where the child is a boy there is also a 50 % (1 in 2) chance the son will have ALD and a 50 % chance he will be unaffected.

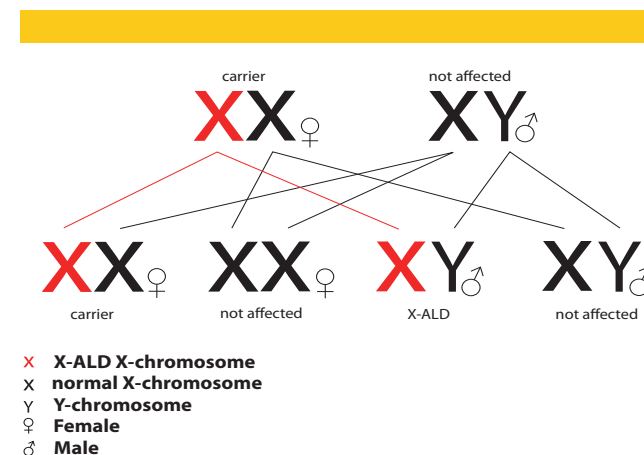
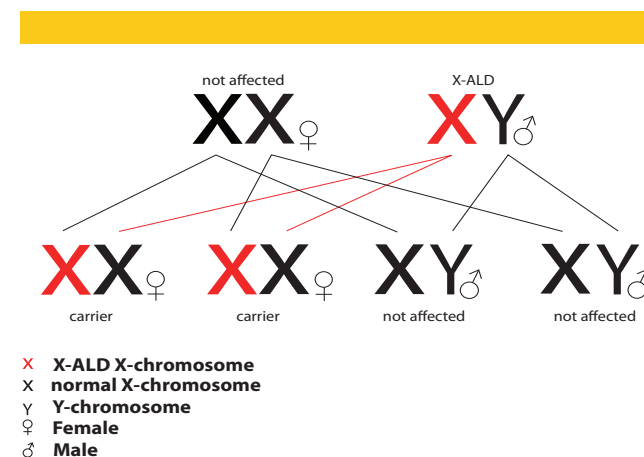


Figure 2:

If an affected man has children, then all of his sons will be free of ALD (because sons get the father's Y-chromosome). But all of his daughters will be carriers (because he passes his only X-chromosome to his daughter).





## PRACTICAL INFORMATION FOR MALES WITH ASYMPTOMATIC ALD

### What medical support is there?

#### Monitoring

In most cases of asymptomatic ALD the patient will display no symptoms of illness, unless he has been diagnosed with adrenal insufficiency. However, he will be monitored very closely for the slightest signs of ALD. He will be under the care of a metabolic specialist, who will be responsible for managing all monitoring. A search for a potential bone marrow donor for the patient may well be performed in order to decide whether he could have a bone marrow transplant if cerebral ALD begins. Gene therapy is also being developed and may be available for those who do not have a well matched donor. At present bone marrow transplants are usually only available for young boys.

The first test that is often performed to identify someone who may have or be at risk of developing ALD or AMN is a check of the levels of very long chain fatty acids (VLCFAs) in the blood. High levels of VLCFAs are an indicator of ALD or AMN. This test may well be used later on to monitor the success of any treatment.

The patient will also undergo a series of other tests, including neuropsychology assessment, eyesight and hearing tests and possibly nerve conduction tests. These are usually arranged through a neurologist. Dependent on where you live, you may have to go to different hospitals for different tests and procedures.

As soon as asymptomatic ALD is suspected, the patient should have an MRI scan immediately, and this should be repeated every six months during the first 10 years of life from about the age of 3-4 years. Your consultant will help make the decision as to when annual MRI scans will suffice.

#### Addison's Disease or adrenal insufficiency

Once the patient has been diagnosed with asymptomatic ALD, make sure that he is checked for adrenal insufficiency as soon as reasonably possible. In ALD the adrenal gland, which produces an essential hormone called cortisol, can stop working. The consultant who made the ALD diagnosis should refer you to an endocrinologist (a doctor specialising in hormones), who will be responsible for making sure adrenal tests are carried out.

Adrenal insufficiency can be treated easily with steroid tablets (hydrocortisone with or without fludrocortisone) to replace the hormones the adrenal gland usually produces. If untreated, adrenal insufficiency can lead to a life-threatening condition called 'adrenal crisis'.

A common sign of adrenal insufficiency is being bronzed, i.e. becoming progressively tanned even in winter, sometimes with brown creases on the palms of the hands (rather than red ones). Other signs include becoming lethargic and having difficulty recovering from infectious illnesses. For example, a minor viral infection may result

in severe repeated vomiting which can lead to dehydration and potentially dangerous changes in blood salts. This can also happen when males with adrenal insufficiency are very stressed, such as by unusually heavy exercise.

Another possible sign is 'cyclical vomiting', where the child catches a bug, starts to vomit repeatedly and quickly looks very ill and has to be admitted to hospital. Once there they are quickly 'cured' by hydration and often discharged the following day.

On very busy or stressful days boys with asymptomatic ALD may go through their steroids faster than normal, causing tiredness, vomiting, headache or fever. Some boys get hangover effects from busy days where they have to stay off school or go home early the following day simply because their system has become overstretched. Also, you will be given advice to increase the dose of hydrocortisone immediately your child becomes unwell or is prescribed antibiotics.

You will also be trained to give an emergency cortisol injection in case the patient is not retaining his hydrocortisone due to vomiting or diarrhoea.

The endocrinologist should also give you a letter stating that if your child has to go to Accident and Emergency they are to be seen immediately because they suffer from adrenal insufficiency. The endocrinologist will also inform the ambulance service that your child has adrenal insufficiency so they

carry the correct medication for him if you need to call 999. In some areas the procedure can be different so please ensure you check the correct procedure for your area.

All with adrenal insufficiency should have a medical identity bracelet or necklace stating their medical condition that they wear all the time. That way, if something happens and the patient is alone, medical authorities will be alerted to his condition and be able to treat him properly. There are many places now that supply these - just search online for medical ID jewellery for a list of suppliers.

#### Treating symptomatic (cerebral) ALD

The most tried and tested treatment is a bone marrow transplant which is nowadays often called a stem cell transplantation. Gene therapy is also in development but has so far only been used in a handful of patients. Trials are currently ongoing at Great Ormond Street Hospital for patients who do not have a suitable bone marrow donor. For either of these treatments to work effectively it is important that changes in the brain are picked up at an early stage, usually before any physical symptoms of ALD have appeared. This is especially important as the brain disease will often continue to deteriorate during the first year or so after treatment.

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These minute changes in the brain can only be picked up on a magnetic resonance imaging (MRI) scan, which is why it is so important to have a scan done as soon as asymptomatic ALD is diagnosed, and then regularly every six months, or annually dependent on the patient's age.

Transplantation or gene therapy are major medical procedures which carry considerable risks. Therefore, they are only used when absolutely necessary – that is, when the changes leading to symptomatic ALD have started. There is more information about bone marrow transplants on p.14.

### Lorenzo's Oil

Lorenzo's Oil, made famous by the film Lorenzo's Oil, can reduce levels of very long chain fatty acids by helping the body to metabolise them. There were great hopes that this would stop the progress of ALD, or prevent it from starting. Unfortunately, Lorenzo's Oil did not live up to this promise. The results of clinical studies have been disappointing, although the lack of large-scale trials means we cannot be certain that the oil has no benefit.

So, although Lorenzo's Oil is NOT a proven preventative, it is the only thing available that MAY possibly be beneficial, and because of this it is widely taken by boys with asymptomatic ALD.

The decision about whether to use Lorenzo's Oil is most important if the

patient is still under six years old. Taking the oil is also usually accompanied by a low fat diet and some find either the diet or taking the oil itself very difficult although these are things which dietitians can help you with. The decision as to whether to start on the oil is an individual decision, so you may want to discuss the pros and cons with your specialist before starting. Please also be aware that some children who take Lorenzo's Oil will still end up having to have a bone marrow transplant or gene therapy.

### Diet and monitoring with Lorenzo's Oil

Lorenzo's Oil is not very nice to take, and patients who do take it usually have to go on a very restrictive low fat diet for it to be effective in lowering VLCFA levels. The diet usually involves no more than 30 grams of fat per day – so no chocolate, crisps, fried food, etc. Some find this very difficult, especially if they start the diet once they have started school or later in life. Some doctors will say that the patient can have one day a month free from the diet, but some families opt not to do this, while others only relax the diet on very special occasions like a birthday.

Patients, parents and carers will need to learn how to read the fat content of food labels, and be aware there is even fat in some fruits and vegetables. Sugar is not a problem, but there is a high fat content in many sugary foods. It is best to cook everything from scratch to be certain of the fat content. Ask to be referred to a

dietitian, who can help ensure the patient is getting the best possible diet within the restrictions. The dietitian will also advise on supplements to ensure the patient gets the essential fatty acids he needs.

Lorenzo's Oil can reduce the level of platelets in the blood, which could result in the blood not clotting as it should. Levels of platelets in the blood are monitored with regular blood tests. The amount of Lorenzo's oil to be taken has to be carefully monitored as well. As patients grow, dietitians will work out how much Lorenzo's Oil they need to take.

### Where do I find Lorenzo's Oil?

Lorenzo's Oil is available on NHS prescription throughout the UK. If your funding body tells you otherwise, they are wrong. In other countries, Lorenzo's Oil is not always available and has to be bought from the manufacturer. ALD Life can advise you if you are having problems obtaining Lorenzo's Oil.

### Taking Lorenzo's Oil

Lorenzo's Oil looks like a bottle of clear liquid at the top with melted lard below. You have to mix the two to make the solution. Keep the oil in the fridge until the time comes to prepare a dose for your child. Leave the oil until it reaches room temperature and shake the bottle vigorously until the two halves have mixed together. Pour out the correct dose. The oil is not pleasant for children to take.

Most small children are given the oil in a warm drink, like milk – it will congeal in a cold drink. If the child is still on a bottle, put the oil in the baby milk. The younger a child is, the more easily they will be able to deal with taking Lorenzo's oil.

Sometimes the oil goes lumpy, which can be a problem. A way to deal with this is to force the lumps through a sieve so it mixes together properly. Some people find that warming it slightly by placing it near (but not on) a heater dissipates the lumps.



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### Telling your school

The patient's school or work place will need to know about their condition because they will need to take quite a bit of time off for tests.

For children, ask the school, and particularly the class teacher, to let you know if they witness any unusual behaviour – for example if the child can complete a jig-saw puzzle one day and the next he can't do it. This sort of incident, and perhaps irrational behaviour, sudden bursts of temper or poor attention and comprehension of what is being said, might be indicators that ALD is starting.

If the patient has adrenal failure, the school or work place will also need to be informed. Sometimes a child will need to take medication at school. This should not interfere with normal school life. Usually there are no problems, and you should find the school very helpful. In the workplace it is vital your employers are aware of your condition in case of an accident.

### Helping the patient come to terms with ALD

A patient diagnosed with asymptomatic ALD may already have a brother with symptomatic ALD, who cannot be cured. This may make it difficult for the patient with asymptomatic ALD to come to terms with their diagnosis. A lot of patients with asymptomatic ALD experience feelings of guilt, and especially with children, may also resent the attention their affected brother receives.

Play therapy can be very useful and can be arranged through your school, your local hospital, or your GP. When the patient gets older there are various organisations who can help him cope with his feelings. One of these is Young Carers, and most hospices have sibling groups. Adults should be given access to counselling if necessary.

A diagnosis of asymptomatic ALD in a family with no symptomatic children or adults can be just as difficult. As there are no real outward signs of illness, it is not easy to convey the seriousness of their condition to children or to friends, family, schools and work. You need to find the right way to deal with this according to your own instincts and situation. ALD Life can help you by putting you in touch with others in similar circumstances.

“  
IT WAS DIFFICULT TRYING TO EXPLAIN TO PEOPLE HOW SERIOUS HIS CONDITION WAS BECAUSE THERE WERE NO PHYSICAL SIGNS HE HAD ANYTHING WRONG WITH HIM.  
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“  
AFTER HE HAD THE TRANSPLANT HE HAD MASSIVE FEELINGS OF GUILT BECAUSE HIS SYMPTOMATIC BROTHER COULD NOT HAVE THE SAME TREATMENT. HE ATTENDED PLAY THERAPY ARRANGED THROUGH HIS SCHOOL WHICH SEEMED TO HELP HIM.  
”

“  
IN SCHOOL THEY WERE BRILLIANT WITH HELPING WITH TAKING MEDICATION AND WHEN HE HAD TO GO FOR HIS TRANSPLANT THE SCHOOL KEPT IN TOUCH VIA WEBCAM WHICH HELPED HIM A LOT.  
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## PRACTICAL INFORMATION FOR MALES WITH ASYMPTOMATIC ALD

### Bone marrow/stem cell transplant

There are two potential treatments which may be used to try to stop cerebral ALD worsening once early MRI signs have developed. Bone marrow transplantation is the first of these and has been in use since the 1980s. Since then two other sorts of stem cell transplants have emerged, using blood - rather than bone marrow - stem cells collected from adults or cord blood collected from the placenta of a newborn baby.

However usually it is still bone marrow that is used to treat boys with cerebral ALD because there are slightly lower risks after the transplant.

A transplant essentially involves using chemotherapy drugs to kill the patient's own bone marrow, then replacing it with marrow cells from a donor. The donor cells settle in the bone marrow and multiply, and some of them move to the brain. It is not understood exactly how they exert their effect, but after a time the healthy marrow cells can stop the process of nerve damage in the brain. However, there is a time delay, so the nerve damage and/or symptoms usually continue to progress for as long as 12-18 months after the transplant although usually at a slower rate than before.

Trying to identify a potential matched donor is one of the first things that should be done when a person is diagnosed with asymptomatic ALD. A sibling may be able to provide a match (the chance of a single sibling matching is one in four). If not, then a search will be done of the computerised bone

marrow registry within the UK and if necessary abroad. Usually a match can be found for 60-70 % of UK patients although to some extent this depends on racial origin. For instance there are comparatively few donors of African ancestry on the registries worldwide. The person who is identified as a match will be informed that they may be needed in future years. Another potential source of the required cells is umbilical cord blood, which is available from various cord blood banks and transplants can sometimes be done from cord blood donors who are more mismatched than bone marrow donors.

In the future, gene therapy may provide an alternative treatment option for boys with X-ALD. Gene therapy involves taking the child's own bone marrow cells and introducing into the cells a working copy of the ALD gene. The gene corrected cells are then introduced back into the child like a blood transfusion. The bone marrow cells now have the correct genetic information to produce the necessary protein. The advantages of gene therapy are that it uses the child's own cells and so there is no risk of Graft-versus-Host Disease (GvHD), where the transplanted donor cells attack the transplant recipient's body. Gene therapy does present its own risks however. The method of introducing the gene into patient cells involves using a modified virus or vector which splices itself into the chromosome. This can affect the function of neighbouring genes or of the gene into which the vector places itself. In early gene therapy trials some children developed cancer because the vector turned on cancer causing genes. However, with

newer vector designs, this risk seems to have been removed although patients remain under careful follow up for this and other problems.

In children, when an MRI scan or other tests show that the changes leading to symptomatic ALD have started, a transplant will usually be carried out within a few months. A transplant is a major process; the child will be in hospital for a minimum of six to eight weeks, and very possibly longer. After that they will be largely confined to home for a further few months until their immune system has recovered.

Transplant does come with risks, but we have many boys on our charity's books who have had a transplant and are living perfectly normal lives. The success rate for bone marrow transplant is increasing all the time, with a majority now succeeding. However, there is still a risk that your son could develop AMN in adulthood.

Transplanting adults who are showing signs of cerebral ALD is not common, but practices are changing and all asymptomatic adults should be able to access yearly MRI scans.

As the risks, procedure and follow-up treatments for transplants are changing all the time, it would not be appropriate to discuss them in depth in this leaflet. If there comes a time when the patient needs a transplant, your hospital will give you all the information you need. You can, of course, seek support and advice from ALD Life and from other families who have been through

the process. You can also contact the Anthony Nolan Trust for more information about transplants [www.anthonynolan.org](http://www.anthonynolan.org).

### Financial aspects

Transplants often result in financial problems because one or more parents may have to stop working. The child will need someone in hospital with them all the time, as well as during their extended recovery period at home. There is help available – the next section describes financial and other help you may be entitled to, and there is a list of organisations that can help at the end of this booklet.

### What financial help can I get?

Children with asymptomatic ALD who are taking Lorenzo's Oil and/or have adrenal insufficiency are entitled to the lowest care rate of DLA (Disability Living Allowance). Once they start going through the transplant process, their DLA will be increased to the highest component for both mobility and care, for the period they are incapacitated until they return to school. They will still be eligible for the lower care rate of DLA due to ongoing adrenal failure. Adults with adrenal insufficiency do not qualify.

Children up to the age of 18 will also be entitled to receive grants from charities like the Family Fund Trust for special treats. If they have a bone marrow transplant they will also be eligible for help from Make A Wish and similar foundations. These not only provide holidays and special treats but can



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also provide gadgets, like laptops, which can prove invaluable during the recovery period after bone marrow transplant.

ALD Life also provides grants for those going through the bone marrow transplant process.

For adults with adrenal insufficiency, free prescriptions are available even if you are working. Medical Exemption (MedEx) certificates are issued on application to people who have a form of hypoadrenalism (for example Addison's disease) for which 'specific substitution therapy' is needed. To apply ask for an application form FP92A, available from your doctor's surgery. You need to fill in parts 1 and 2 and your doctor (or an authorised member of the practice staff) will sign to confirm the information you've given is correct.

### How can I get more information?

You can get in touch with others in a similar situation through ALD Life, who can also provide practical information, support and advice. ALD Life also runs an annual event which brings together families and individuals suffering from all aspects of carrying the ALD gene, to learn about innovations in treatment and research, and share experiences.

Listed below are just some of the useful organisations that are there to help you:

### INFORMATION, SUPPORT AND ADVICE

#### ALD Life

ALD Life was founded by Sara Hunt after both her sons were diagnosed with ALD. She has first hand experience of most aspects of dealing with the disorder: her elder son, Alex, was diagnosed with symptomatic ALD at age 7 in 2001, and passed away in 2012. Her younger son, Ayden, had a successful bone marrow transplant in 2008. The charity provides practical, emotional and financial support for all those affected by ALD and AMN.

[www.aldlife.org](http://www.aldlife.org)  
Tel: 020 7701 4388  
Email: [info@aldlife.org](mailto:info@aldlife.org)

#### Carers UK

Carers UK campaigns to make sure carers receive the practical, financial and emotional support they need.

[www.carersuk.org](http://www.carersuk.org)  
Tel: 0808 808 7777  
Email: [info@carersuk.org](mailto:info@carersuk.org)

#### Contact a Family

National charity Contact a Family provide advice and support for any family with a child fighting life-threatening illnesses. They run a free helpline to answer all queries on everything from medical advice, to benefits, grants and schooling. They will even send someone to your home to assess what you will need.

[www.cafamily.org.uk](http://www.cafamily.org.uk)  
Tel: 0808 808 3555  
Email: [info@cafamily.org](mailto:info@cafamily.org)

#### Citizens Advice Bureau

Citizens Advice Bureau have free information and advice on legal and money problems and can help you if you experience problems with housing or benefits. Your local branch can be found on the national website.

[www.adviceguide.org.uk](http://www.adviceguide.org.uk)

#### Directgov

Government website for information about benefits and entitlements.

[www.direct.gov.uk](http://www.direct.gov.uk)

#### Young Carers

Young Carers is a network of support groups run by The Princess Royal Trust for Carers, the largest provider of comprehensive carer support services in the UK. Runs a network of 144 independently managed Carers' Centres and provides quality information, advice and support services to over 400,000 carers, including young carers.

[www.youngcarers.net](http://www.youngcarers.net)

### EQUIPMENT AND FINANCIAL SUPPORT

Directory of Grants for Individuals in Need available from the reference section of your local library or online at

[www.grantsforindividuals.org.uk](http://www.grantsforindividuals.org.uk)

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### Family Fund

The Family Fund is a charity that helps families with children fighting life-threatening illnesses and gives grants for things such as washing machines, driving lessons, costs of hospital visits, computers and holidays.

[www.familyfund.org.uk](http://www.familyfund.org.uk)

Tel: 08449 744 099

Email: [info@familyfund.org.uk](mailto:info@familyfund.org.uk)

### HELP FOR THOSE GOING THROUGH BONE MARROW TRANSPLANT

#### Anthony Nolan Trust

Anthony Nolan provide match donors for people who need a blood stem cell, bone marrow or cord blood transplant.

[www.anthonynolan.org](http://www.anthonynolan.org)

Tel: 0303 303 0303

### HOLIDAYS & ONCE IN A LIFETIME EXPERIENCES

#### Dreams Come True

A national charity that makes dreams a reality for children with life threatening conditions.

[www.dreamscometrue.uk.com](http://www.dreamscometrue.uk.com)

Tel: 0800 018 6013

Email: [info@dreamscometrue.uk.com](mailto:info@dreamscometrue.uk.com)

### Family Holiday Association

The Family Holiday Association provide grants towards a one-week holiday of the family's choice. Referral needed from a health visitor or social worker.

[www.familyholidayassociation.org.uk/](http://www.familyholidayassociation.org.uk/)

Tel: 020 3117 0650

Email: [grantofficer@familyholidayassociation.org.uk](mailto:grantofficer@familyholidayassociation.org.uk)

### Make-A-Wish Foundation UK

The world famous Make-A-Wish foundation grants magical dream wishes to children aged 3 –17 who are fighting life-threatening illnesses.

[www.make-a-wish.org.uk](http://www.make-a-wish.org.uk)

Tel: 01276 405 060

Email: [info@make-a-wish.org.uk](mailto:info@make-a-wish.org.uk)

### Starlight Foundation

Another famous charity that makes dreams come true for terminally and seriously ill children.

[www.starlight.org.uk](http://www.starlight.org.uk)

Tel: 020 7262 2881

Email: [info@starlight.org.uk](mailto:info@starlight.org.uk)



### INFORMATION SOURCES

The medical information in this leaflet, shaded in green, has been compiled from the following references, and reviewed by an expert doctor.

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All medical and benefits information is correct at time of going to press January 2015.