

- **PREVENTING A JOINT BLEED**

A PARENTS' GUIDE
TO THE
**Prophylactic
Treatment**
OF HAEMOPHILIA



HAEMOPHILIA FOUNDATION AUSTRALIA

Preventing a joint bleed.

A parents' guide to the prophylactic treatment of haemophilia.

Prophylaxis means giving treatment in advance to prevent a condition from happening. For children and teenagers with haemophilia* prophylactic treatment involves giving regular infusions (injections) of the missing clotting factor (either VIII or IX) to prevent bleeds from occurring. This is different to the usual form of haemophilia treatment called 'on-demand therapy' where the missing factor is given after a bleed has started.

The aim of prophylaxis in children and teenagers with severe haemophilia is to increase their blood factor VIII or IX activity levels from 1% or less of normal levels, to at least 3-4%. This means, in effect, that the child receiving prophylaxis has moderate rather than severe haemophilia and is less likely to have spontaneous bleeds, or joint or muscle bleeds from minor injuries. Having few or no bleeds means that the chance of joint damage due to internal bleeding is minimised. However, prophylactic treatment cannot restore a damaged joint back to normal. Nor can it reverse the long-term onset of arthritis in an affected joint.

To maintain a blood factor VIII or IX level of at least 3-4%, prophylaxis usually involves infusions of clotting factor VIII every other day or three times every week, or infusions of clotting factor IX twice a week.

Currently, prophylactic treatment is available up to the age of 18. Up until now there has been no evidence to show whether or not there is benefit in continuing prophylaxis after this time. It will be a matter to be decided after consultation with your haematologist.

History of continuous prophylaxis'

While this is a relatively new form of treatment for haemophilia in Australia, it has been around for about 40 years. As long ago as 1968 a study published in USA on the use of prophylaxis acknowledged that it was effective in preventing and reducing joint damage. In 1992 prophylactic treatment again came to the forefront when a Swedish study found that prophylaxis, begun at an early age, appeared to prevent joint damage and joint deterioration.

* This information also applies to some children and teenagers with severe von Willebrand disorder.

Advantages:

On-demand therapy with clotting factor concentrate is an effective way of treating bleeds, especially if given just after the bleed has started. However, one problem with this method of treatment is that once a joint bleed has begun, the affected joint is more likely to undergo a second bleed while it is recovering. Sometimes this leads to the joint becoming what is called a "target joint" which has to cope with repeated bleeds - never recovering properly between them. Studies over a number of years of people with haemophilia show that they have many small joint bleeds. Although home therapy has been very successful in treating people with joint bleeds, it hasn't prevented the onset of arthritis. The development of arthritis may lead to permanent joint damage and disability.

The greatest advantage for children receiving prophylaxis is that they are much less likely to have long-term joint damage than if they were receiving on-demand therapy.

Furthermore, because they have fewer bleeds, children and teenagers receiving prophylaxis will miss less school or work, spend less time in hospital receiving treatment for severe bleeds, and can take part in social activities and some sports.

The psychological advantages can be as important as the physical ones. Children and teenagers on prophylaxis will have fewer restrictions caused by their condition, and will worry far less about the possibility of having a bleed.

There are advantages for the families of children and teenagers with haemophilia too. They will spend less time having to care for their child during and after bleeds, and will worry less about them injuring themselves.

Possible Disadvantages:

Prophylaxis requires repeated clotting factor infusions. While most people are able to undertake home therapy, they may still feel tied by the need for regular infusions. In the long-term though, these regular infusions should prove easier to cope with than irregular bleeds that could need hospital treatment.

Another possible disadvantage of prophylaxis is that the repeated injection of plasma-derived clotting factor may increase the possibility for a person to be infected by an agent carried in the clotting factor concentrate. All clotting factor concentrates are now treated to destroy HIV, hepatitis B and C, but it is still possible that new viruses will appear in blood concentrates and infect people who have haemophilia.

This potential danger is thought to be decreased by the use of recombinant (DNA derived) clotting factor concentrates that are now available. As these are not made from purified human blood, they are very unlikely to carry human viruses. Further advantages are that they carry few impurities and so are less likely to cause a reaction in the person receiving the infusions. Additionally, the volume of recombinant clotting factor may be smaller for each treatment than the plasma-derived product.

Repeated infusions can be difficult, particularly in small children. The problem is finding veins to infuse the clotting factor. This can usually be overcome by implanting a port-a-cath directly into a vein through which injections can be made. The decision as to whether to implant a port-a-cath is made by your child's haematologist in consultation with you.

Port-a-caths

A port-a-cath (often referred to as a 'port') is a type of catheter (or tube) that is inserted to allow treatment to be taken easily, and intravenously. The port-a-cath is made up of two parts: the 'port' which is a 'box' where the needle is inserted and a 'catheter' which leads from the 'box' into one of the big veins, usually in the neck. The port is placed under the skin, usually on the chest just over the ribs, so all that is visible is a 'bump' where the 'box' part of the port is positioned. It is inserted in hospital under general anaesthetic, and usually means a stay in hospital for some days.

Haematologists differ in opinion on whether the benefits of port-a-caths outweigh the risks.

Advantages:

Ports allow clotting factor to be given prophylactically even in babies and children with haemophilia. Once a port is fitted, there is no longer the need for infusions into small veins. Instead, a specially designed port needle is inserted through well-cleaned skin into the 'bump'. This allows direct access to the blood. During this procedure the child will usually only feel pressure on the chest. Commonly, parents are taught how to use the port at home. This usually means fewer visits to hospitals and allows families to lead more normal lives. Often ports are only needed for a few years, until the child's veins have developed enough to allow regular venipuncture.

Disadvantages:

Sometimes ports can become infected. These infections can be severe and may lead to a hospital admission. The infection risk means that everything must be very clean when accessing the port. Parents are informed of infection control procedures by their Haemophilia Centre when given instructions about infusing their children at home. Information is also given on what symptoms to look out for regarding a port-a-cath infection. Symptoms include a high fever or redness at the port site.

Tips:*

Infusion should never be used as a threat or punishment, or as an attempt to control behaviour. Suggesting infusion as punishment makes the child see it as a negative event to avoid or even resist. For children with haemophilia, infusion is a necessary part of life. Therefore it is better to say, "If you keep fighting you will get a bleed", "If you get a bleed, you will need an infusion", rather than, "If you don't stop fighting, I'm going to have to infuse you". Making infusion as pain and stress free as possible is the key to having it become routine.

Many people find bringing out an inexpensive toy, such as a small car or action figure, or a "special" show bag used only during infusions, is a good way to distract and comfort their child. Some parents make it a game - "How long can you lie still?" After infusion, the best form of comfort is spending time with your child, perhaps reading a story or playing a favourite game together.

When to begin prophylactic treatment

Experts differ about when prophylactic treatment should be started. Some haematologists support early infusion, before children have a joint bleed, arguing that even a few bleeds into joints can result in minor joint deterioration. Others support beginning prophylactic treatment when a child shows a bleeding pattern, arguing that until this occurs very few children have joint problems. Your haematologist will have his/her own position on this issue and will discuss with you what they believe is the most appropriate age for your child to begin prophylaxis.

Other Issues

- For many young children with severe haemophilia on prophylactic treatment, their disability may not be apparent to them as they experience few or no bleeds, and no pain. Some argue that it is important that people with haemophilia should experience bleeds to have an understanding of what their disorder is all about, and to learn why treatment is necessary. Requiring treatment three times a week for a disorder they have little personal connection with can be confusing for the child. “No one can say what self-image a child has who never bleeds but is told he/she has a disability”.ⁱⁱⁱ On the other hand, the pain and loss of self-esteem that can be associated with having chronic arthritis due to bleeds in joints is well documented.
- Because the child may be receiving more treatment product, some people have raised the concern that prophylaxis may increase the number of children developing inhibitors (antibodies to the treatment product). However, so far there is no evidence to support this concern.^{iv}
- Even when on prophylactic treatment, contact sport such as the many football codes should still be avoided as they place a great deal of stress on the body. This increases the chance of a bleed occurring. Please consult with your haematologist if your child is interested in playing any new sport or considering a strenuous activity such as rock-climbing.

Case Study

My ten year old, Nicholas, went on prophylaxis when he was five years old. This was after he had a bad year at kindergarten, when Nicholas spent a lot of the time in hospital because of bleeds.

He was never offered a port and went straight onto venous access. Now he has very few bleeds. The truth is Nicholas has coped better than all of us put together. Sure he has his teary times, his hurting times, but he has adapted.

I infuse Nicholas - having been taught by the visiting nurse. Even though I have a nursing background, it is a whole different ball game when it's your own child you're injecting. When I first learnt to infuse, I missed a vein and burst into tears but Nicholas just said, "It's okay mum, try again". A big hurdle I had to cross was gaining Nicholas' trust. It is an overpowering feeling that you are doing something to help them but may also hurt them, if you don't do it correctly. We did work out a routine to encourage Nicholas to sit still for his prophylactic treatment. We bought a special sticker book, in which a new sticker goes in whenever Nicholas doesn't cry during infusion. We also got our older child, Sarah, involved by getting her to stick in pictures as well. So, it's become a family effort. My husband learnt how to infuse earlier this year, although he is still not comfortable with the procedure.

Even though Nicholas is now going through a stage of not wanting infusions and saying "Why me?", prophylactic treatment has changed our life. We feel more self-sufficient. This year for the first time we went away on a holiday and just took the treatment things away with us. Prophylaxis has made things easier on Sarah too. Before she was being shoved from pillar to post when Nicholas was in hospital. Now things have really settled down.

We don't badger Nicholas about infusing. We just remind him of the repercussions if he doesn't get his factor VIII. We have also taught him to understand that just because he is on prophylaxis he can't do everything he wants to do, for example, playing football.

Case Study

I'm 17 and I've been on prophylaxis for about 5 years. I don't get bleeds now and that's a problem because it's like I don't have haemophilia. I self-infuse - I've been doing it alone for a while. The injection is not a hassle but I hate the time it takes while I mix the factor VIII and get things ready. All up, it takes about twenty minutes out of my life everytime I infuse.

I've got hepatitis C so I have to be careful about making sure the syringes go into the sharps container, and I wipe up any blood spills. Sometimes I miss an infusion because I forget and sometimes I don't want to infuse.

They say prophylaxis will probably stop when you're 18 but in my case it shouldn't stop because I used to have an inhibitor. If I stop prophylaxis, the inhibitor may come back.

Haemophilia is out of my mind now apart from when I infuse. Before prophylaxis, haemophilia used to be my life. It was my whole family's life, especially my mother's. I used to have a lot of bleeds.

I'm careful but I do take risks. I do what I want to do, like skateboarding. Sometimes, one of the things I find hard is telling new friends that I have haemophilia, especially now that I don't have bleeds. But, all up I manage haemophilia fine. I have problems in my life but haemophilia isn't really one of them.

Case Study

As Robert is only 20 months old we kept putting off the port-a-cath operation. He just seemed too little. Because he is borderline 2% we were waiting to see whether he would display as more of a severe. When he started to have a lot of joint bleeds and continuing pain, we realised the port was necessary.

First though, we talked it through with our haematologist and haemophilia nurse, and got information from HFA. The visiting nurse was also very helpful. We also met a child with a port and then made our decision. The truth is that waiting for the operation was worse than having it.

The visiting nurse comes in three times a week to infuse Robert and we are keen to take it on ourselves. My husband is particularly keen.

Already it has made a big difference to our lives. I am more relaxed about him playing; I don't have to drop everything. I know that with the clotting factor cover he will be okay.

Information in this booklet is subject to change.

Endnotes:

- ¹ Kelley, L. A. "Prophylaxis: Panacea or Pipe Dream", *The Parent Exchange Newsletter*, April 1995, Vol. 5, Issue 1.
- ² Evans, S. "As Babies Grow", *Hemalog*, July 1997, Vol. 8, No.2.
- ³ Evans, S. Op. Cit.
- ⁴ Hann, C. "Factor VIII and Prophylaxis", *The Bulletin*, December, 1994.

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