

## CHELATION THERAPY

### Information for people with Diamond Blackfan anemia and their families.

#### Why would a person with Diamond Blackfan anemia need chelation therapy?

In Diamond Blackfan anemia (DBA) the bone marrow (the center of the bones where blood cells are made) does not make enough red blood cells. One of the treatments for DBA is blood transfusion therapy. Blood transfusions temporarily increase the number of red blood cells. Some people need blood transfusions only now and then, such as when they are sick. Other people need regular blood transfusions over a long period of time. This is called chronic transfusion therapy.

One of the risks of chronic transfusion therapy is getting too much iron in the body. Blood contains a lot of iron. Because the body has no natural way to get rid of iron, the iron in transfused blood builds up in the body, a condition called iron overload. Eventually, after dozens of transfusions, the iron will build up to toxic levels and can damage different organs in the body. The iron cannot be removed from the blood before transfusion, as it is a critical component of hemoglobin, a protein in red blood cells that carries oxygen. Fortunately, iron overload and organ damage can be prevented with chelation therapy.

#### What is chelation therapy?

Chelation therapy means using a medication to remove certain metals, such as iron, from the body. These medications bind to the iron and remove it through the urine or stool.

#### What chelation drugs are currently available?

There are currently two chelation drugs available in the United States. Each drug has benefits and risks.

#### Deferoxamine (Desferal)

Deferoxamine (Desferal) is a chelation drug that has been used in the United States for many years. It works very well but must be given as an infusion, meaning through a needle that is placed under the skin (subcutaneously). It cannot be taken by mouth. Deferoxamine is usually given over 8–12 hours, 5–7 nights per week using a battery operated pump.

#### *Noticeable side effect:*

People who take deferoxamine will notice an orange to red color to their urine. This discoloration means that the medication is working, and the iron is leaving the body. When the medication is taken properly, and iron is removed adequately from the body, this discoloration in the urine gradually decreases over time.

#### *Possible side effects:*

- The most common side effect of deferoxamine is irritation of the skin around the needle.
- Headache, upset stomach, nausea and diarrhea, and dizziness rarely occur.
- Vision and hearing loss can occur if the dose of deferoxamine given is higher than the amount of iron available. Therefore, close monitoring of the person's dose and iron status, as well as hearing and vision testing, are required while taking deferoxamine.
- Any medication may cause serious allergic reactions. These types of reactions, which include rash, trouble breathing, and swollen lips and tongue, are very rare with deferoxamine.

For more information call:

1-877-DBA-NURSE





## Deferasirox (Exjade)

Deferasirox (Exjade) is a chelation drug that became available in the United States in 2005. The advantage of deferasirox is that it can be taken by mouth. Deferasirox tablets are dissolved in water or juice and taken once a day. Deferasirox works by binding to the iron and removing it through the stool. It does not cause discoloration to the stool.

### *Possible side effects:*

- Mild skin rash.
- Fever.
- Cough.
- Headache, stomach cramps, nausea, vomiting and diarrhea; these symptoms usually go away in a few days once the body adjusts to the medication.
- Rarely, deferasirox can damage the liver or the kidneys; your doctor will do regular blood and urine tests to check the liver and kidneys. If the medicine is affecting the liver or kidneys, the dose of deferasirox may be lowered or stopped for a period of time to allow them to return to normal.

## How do doctors check the amount of iron in the body?

There are a few different ways that doctors can check the amount of iron in the body such as:

### *Ferritin*

The amount of iron in the body can be regularly checked with a blood test called a serum ferritin. This blood test will give a general idea of how well the chelation drug is working.

### *T2\* MRI (magnetic resonance imaging) and FerriScan MRI*

T2\* MRI is a special type of scan that shows the amount of iron in the heart. FerriScan MRI is a special scan that shows the amount of iron in the liver. These tests are very accurate and are non-invasive, meaning that they do not require a blood test or insertion of a needle into the organ. T2\* MRI and FerriScan are relatively new and are currently only available at a few medical centers. Small children who cannot lie still for the scan may require sedation.

### *Liver biopsy*

Liver biopsy involves inserting a very small needle into the liver and removing a tiny piece of the liver tissue. The amount of iron in the liver can then be measured. The liver tissue can also be checked under the microscope to see if the iron has caused any damage.

This test is very accurate, but it does have some risks. Most children and some adults need to be sedated for liver biopsy. There is a very small chance that there will be some bleeding from the liver where the needle was placed. There is also a small risk of infection where the needle is placed. People may have mild soreness or pain for a couple of days after the procedure.

### *SQUID (superconducting quantum interference device)*

SQUID is a special type of x-ray that uses magnets to show how much iron is in the body. This test is very accurate and is non-invasive, but is currently only available in two cities in the United States (New York City and Oakland, California).

## Important things to remember

- Ask questions. Make a list ahead of time to discuss with your doctor at your next visit.
- Be your own advocate because many doctors are not that familiar with DBA. Do your homework and be part of the decision-making process.
- While taking deferoxamine (Desferal), avoid taking Vitamin C unless prescribed by the doctor.
- While taking deferasirox (Exjade), avoid taking antacids that contain aluminum, such as Maalox and Mylanta.
- Have your vision and hearing checked every year while taking chelation drugs.
- Make sure you tell your doctor about other drugs you are taking while you are taking chelation drugs.



- If you are pregnant or planning to become pregnant, it is important that you talk with your doctor about your chelation drugs, as they may be harmful to the unborn child.
- Ask for help. Taking chelation drugs daily can be hard, so it is normal to feel discouraged sometimes. Support can come from friends, family members, doctors or nurses on your medical team, or a support group for people with DBA.
- Contact the DBA resource center if you have more questions or concerns.

