

Bone Marrow Transplant From 2 Years Of Age

Bone marrow transplant are reserved for those who are not tolerating standard treatment options.

This requires further discussion with your doctor. Not everyone will be able to have a transplant.

Bone Marrow transplant replaces the bone marrow with friendly marrow as with blood transfusions.

- Bone marrow biopsy if cytopenia yearly
- Bone marrow transplant

Health Management & Future Treatment

- 5 years bone marrow biopsy – yearly FerriScan
- Regular orthopedic review and endocrine reviews from approx 10 years old
- Endocrinology review from 10 years of age until end of pubertal development
- Additional screening also includes cardiac review to assess presence of heart defects and an abdominal ultrasound to check for any kidney abnormalities.

2 Years Of Age:

- FerriScan under sedation
- Liver biopsy
- Bone marrow biopsy every five years
- DEXA scan
- MRI T2*
- Monitor film vitamin D

This literature has been designed by **DBAS UK** with kind support and guidance from the **Aplastic Anaemia Trust**. Check out www.theaat.org.uk/marrowkidz for further info

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The Person Journey Map

DBA UK Registered Charity 1083179

Symptoms

Common symptoms of DBA include: Low haemoglobin, poor feeding, poor sleeping, not growing, fatigue (tiredness), shortness of breath, and headaches

Blood Tests

It is likely that the first type of test that a GP or consultant will request is a **full blood count**. This may show a low red blood cell count and the MCV will be raised.

Bone Marrow Biopsy

In DBA, the bone marrow contains **fewer red blood cells than normal**. The bone marrow sample is examined to rule out other blood-related diseases

Genetic Testing

It will take some time to receive the results from genetic tests, and treatment will most likely begin before the results are available. Still not all genes are known, so some families may still not get a genetic cause. Parents can be negative for a gene then pass on DBA via sperm/eggs.

Diagnosis

When a person is diagnosed with DBA, a team of expert doctors, nurses and other healthcare professionals are established to oversee the person's treatment and care. DBA is a rare blood disease, so careful explanation about its nature and prognosis is required. A person diagnosed with DBA should be given the opportunity to be referred to a hospital that specialises in this rare blood disease. The mental wellbeing of the person diagnosed, and that of their family and close friends, is also of great importance. Treatments may take a long time and may mean reduced contact with family and friends for a while.

Investigation of Immune System

It is likely that you will have to have further blood tests to investigate how your immune system is working. It is advised to have the Chickenpox Vaccine and the Hepatitis B Vaccine.

Blood Transfusions

Blood transfusions for a minimum of 12 months of age before we start a steroid trial as this will aid as much natural growth and development as possible.

Treatment Options

Blood transfusions then a steroid trial, whichever treatment option works best for you will be a decision between you and your doctor. What should happen:

- Referral to specialist central
- Regular blood transfusions with chelation
- A steroids trial

Transfusions

- According to exercise tolerance and growth
- <250 ml/kg/year
- Transfusion intervals are in most cases 3-4 weekly

Steroid Treatment

- Trial of prednisolone 2 mg/kg for four weeks. It may be of benefit to begin the trial in the spring or summer months as there are generally less virus's around.
- Wean alternate day over 8 weeks 2 mg/kg alternate days typical 1 mg every 6 weeks slow reduction over >6 months prednisolone ≤0.5 mg/kg alternate days FerriScan under sedation 5 to 10 years of age: MRI T2*
- Return to transfusion if no response
- Will also need medication to protect against opportunistic infection and to protect the gut while on doses >0.5mg/kg