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FOR IMMEDIATE RELEASE

BREAKING NEWS: NHS Offers Allo Haematopoietic Stem Cell Transplant (Allo-HSCT) for Adults with Transfusion Dependent Thalassaemia (TDT)

In a groundbreaking decision, NHS England has announced its agreement to provide Allo Haematopoietic Stem Cell Transplant (Allo-HSCT) for adults living with transfusion dependent thalassaemia (TDT) who meet the specific criteria. This new treatment option marks a significant step forward in improving the lives of individuals battling this challenging condition.

Currently, the mainstay of treatment for adults with TDT involves regular blood transfusions and rigorous chelation therapy, aimed at condition control. However, despite advances in care, iron overload obtained from regular blood transfusions remain a detrimental issue in thalassaemia leading to organ dysfunction / failure and even premature death if not managed appropriately with iron chelation medication.

Those living with thalassaemia can experience a reduction in their health related quality of life and life expectancy when compared to the general public.

Until today the only funded curative therapy offered by the NHS was HSCT for children under the age of 18 with a matched donor.

However, after a thorough review of the available evidence, NHS England has determined that there is sufficient supporting data to make Allo-HSCT accessible to adult TDT patients at this time. This decision signifies a significant advancement in the treatment landscape for individuals living with thalassaemia.

By expanding the availability of Allo-HSCT to adult patients with transfusion dependent thalassaemia, the NHS is demonstrating its commitment to delivering cutting-edge and life-changing treatments.

This decision will undoubtedly offer renewed hope to patients, providing them with the potential for improving quality of life and the possibility of a cure.

Romaine Maharaj, UKTS Executive Director said

"We celebrate the long-awaited approval of Allo-HSCT for adults with transfusion dependent thalassaemia! On behalf of our community, we extend our heartfelt gratitude to the team who worked on this led by Dr Banu Kaya, Consultant

Haematologist at the Royal London Hospital who dedicated their efforts in achieving this extraordinary result. This remarkable milestone offers hope to adults with donor matches who were previously excluded from accessing a curative option. Whilst it is a huge step in the right direction and a monumental win for thalassaemia, we also eagerly await the much-needed approval for gene therapies. Having both curative options available will grant more patients the chance to live transfusion-independent lives, enhancing both their quality of life and life expectancy.”

Kirthana Balachandran, a 20-year-old medical student from London, was diagnosed with thalassaemia when she was three months old. Although she previously had the potential to receive a stem cell transplant as a child and teenager, unfortunately no matched donors were found, meaning Kirthana still requires blood transfusions every three weeks and medication to manage her condition.

She said: “My condition affects me a lot,” “Sometimes I have muscle pain, back pain and I can even feel breathless or have palpitations. It just depends on your haemoglobin level. When it’s low, I feel so tired and it can affect me at the most inconvenient times, like when I have exams. But when my levels are good, I don’t really feel any symptoms at all.”

For Kirthana, the possibility of a stem cell donation in adulthood gives her an option to cure her condition, that previously wouldn’t have been available to her.

She said: “When I was younger, we looked for a donor from my family, but no one was a match and the possibility just faded away. With today’s news, if we were able to find a donor, that would be an amazing possibility, as it could really change my life. I would never need to go for a blood transfusion again for my condition and I wouldn’t have to worry about the side effects from transfusions or my health in future either.

“It’s amazing that they’ve made this treatment option available for adults, because it means we can still have a chance of finding a donor, even at an older age.”

What is Allo-HSCT

The newly introduced Allo-HSCT, also known as bone marrow transplantation (BMT), offers a curative intervention for a wide range of disorders, including TDT. This transformative procedure involves replacing the patient’s bone marrow stem cells with healthy stem cells obtained from a donor. Administered through intravenous infusion, Allo-HSCT aims to restore blood cell production in individuals whose bone marrow or immune system is damaged or malfunctioning.

For more information : <https://www.england.nhs.uk/wp-content/uploads/2023/11/2120-cc-policy-allo-hsct.pdf>

Note to editors:

- Thalassaemia is a genetic blood disorder characterised by abnormal haemoglobin production, resulting in inadequate red blood cell function.

- Allo-HSCT has been successfully utilised in the treatment of various disorders, including thalassaemia, by replacing damaged bone marrow with healthy stem cells from a compatible donor.
- This decision by NHS England reflects its commitment to evidence-based treatment options and improving patient outcomes.