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| **NHP MDT Referral Form for Casgevy in Transfusion-dependent Beta Thalassaemia** |
| **Please return completed form to gstt.haemoglobinpanel@nhs.net**  **REFERRER DATA** |
| |  |  |  |  | | --- | --- | --- | --- | | **Date of Referral** | Select date via arrow | **National MDT Date Aimed for** | Select date via arrow | | **Referring Clinician** | Click here to enter text. | **Clinician’s SHT/Trust** | Click here to enter text. | | **Who will present case at NHP MDT?** | | Click here to enter text. | | | **Region:** Choose your region from list  **Other (Region):** Click here to enter text. | | **HCC  SHT  LHT** | | | **Patient Hospital MRN:** *(****NOT NHS number, Patient Names*** *or other clear identifiers. Adding Initials is accepted)*  Click here to enter text. | | **NHP Unique Identifier:** Click here to enter text.  **(***for NHP admin only***)** | |   **PATIENT & CASE DETAILS**   |  |  |  |  |  |  |  | | --- | --- | --- | --- | --- | --- | --- | | **Age at referral:** | Click here to enter text. | **Sex at Birth:** | | Click here to enter text. | **Diagnosis (DBA, HbSS, TDT etc.):** | Click here to enter text. | | **Clinical Background** | | | Click here to enter text. | | | | | **Presenting Issues** | | | Click here to enter text. | | | | |
| **Do you want to refer this patient for casgevy gene therapy 🞏 Yes, 🞏 No**  ***If ‘Yes’ continue competing this form below if ‘No’ stop here.*** |

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| **Please fill in the below if the patient is being referred for Casgevy gene therapy** | |
| **Question/Patient has the following:** | **Answer** |
| Documented homozygous β-thalassaemia (including β0/β0, β0/β0-like, or non–β0/β0-like genotype) or compound heterozygous β-thalassaemia including Haemoglobin E/ β-thalassaemia and a history of at least 100 mL/kg/year or 10 units/year of packed RBC transfusions in the prior 2 years. | Yes,  No |
| Karnofsky performance status of ≥80% for patients ≥16 years of age OR Lansky performance status of ≥80% for patients <16 years of age. | Yes,  No |
| Known and available fully matched HLA related donor | Yes,  No |
| Prior allogeneic HSCT | Yes,  No |
| Patients with associated α-thalassaemia with >1 alpha deletion or alpha multiplications. Patients with sickle cell β-thalassaemia variant | Yes,  No |
| Clinically significant and active bacterial, viral, fungal, or parasitic infection as determined by the attending physician | Yes,  No |
| White blood cell count <3×109/L or platelet count <50×109/L not related to hypersplenism | Yes,  No |
| History of a significant bleeding disorder | Yes,  No |
| Any prior or current malignancy or myeloproliferative disorder or a significant immunodeficiency disorder | Yes,  No |
| Advanced liver disease defined as:  Aspartate transaminase (AST), alanine transaminase (ALT) >3 × the upper limit of normal (ULN), or conjugated bilirubin value >2.5 × ULN, or:   1. Baseline prothrombin time (International Normalized Ratio; INR) >1.5 × ULN, or 2. History of cirrhosis or any evidence of bridging fibrosis on a prior liver biopsy, if available 3. Patients with active hepatitis infection 4. Patients with history of chronic hepatitis infection are also excluded unless liver biopsy within 3 months prior to or at screening shows no evidence of bridging fibrosis or cirrhosis 5. Liver iron content (LIC) ≥15 mg Fe/g dry weight on R2 or T2\* MRI of liver unless liver biopsy within three months prior to or at screening shows no evidence of bridging fibrosis or cirrhosis | Yes,  No   1. Yes,  No 2. Yes,  No 3. Yes No 4. Yes,  No 5. Yes,  No |
| A cardiac T2\* <10ms by MRI or left ventricular ejection fraction (LVEF) <45% by echocardiogram | Yes,  No |
| Baseline estimated glomerular filtration rate <60 mL/min/1.73 m2 | Yes,  No,  Pending |
| Diffusion capacity of the lungs for carbon monoxide (TLCO) <50% of predicted (corrected for haemoglobin and/or alveolar volume) | Yes,  No,  Pending |

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