# Stem cell-based therapies

**The use of hematopoietic stem cells involves transplanting them into a patient. These cells restore damaged tissues (abnormal bone marrow in the case of leukemias) and thus save lives. These procedures are also performed in Poland (approximately 1450 procedures in 2014) in children and adults.**

We encourage you to review the tables with a classification of transplantation procedures in children and in adults. Please note that umbilical cord blood contains stem cells which are not a remedy for all diseases that may affect your children in the future.

**The table below shows sample indications for umbilical cord blood transplantation in children.**

| | Autologous umbilical cord blood transplantation – own blood | Allogeneic umbilical cord blood transplantation – from a sibling | Allogeneic umbilical cord blood transplantation – from a matched unrelated donor | Allogeneic umbilical cord blood transplantation – from a mismatched unrelated donor |
|---|---|---|---|---|
| **1. Leukemias** | | | | |
| Acute lymphoblastic leukemia | no | yes | yes | under investigation |
| Acute myeloid leukemia | depending on the disease stage | yes | yes | depending on the disease stage |
| Chronic myeloid leukemia | no | yes | yes | under investigation |

| | Autologous umbilical cord blood transplantation – own blood | Allogeneic umbilical cord blood transplantation – from a sibling | Allogeneic umbilical cord blood transplantation – from a matched unrelated donor | Allogeneic umbilical cord blood transplantation – from a mismatched unrelated donor |
|---|---|---|---|---|
| **2. Proliferative disorders of the lymphatic system** | | | | |
| Malignant lymphogranulomatosis (Hodgkin lymphoma) | depending on the disease stage | under investigation | under investigation | no |
| Malignant lymphoma | depending on the disease stage | depending on the disease stage | depending on the disease stage | no |
| **3. Myelodysplastic syndromes** | | | | |
| Chronic myelomonocytic leukemia | no | yes | yes | under investigation |
| Refractory anemia | no | yes | yes | under investigation |
| Refractory anemia with ring sideroblasts | no | yes | yes | under investigation |
| Refractory anemia with excess blasts | no | yes | yes | under investigation |

| | | | | |
|---|---|---|---|---|
| Refractory anemia with excess blasts | no | yes | yes | under investigation |

| | Autologous umbilical cord blood transplantation – own blood | Allogeneic umbilical cord blood transplantation – from a sibling | Allogeneic umbilical cord blood transplantation – from a matched unrelated donor | Allogeneic umbilical cord blood transplantation – from a mismatched unrelated donor |
|---|---|---|---|---|
| in transformation | | | | |
| **4. Diseases caused by stem cell defects** | | | | |
| Severe aplastic anemia | yes | yes | yes | under investigation |
| Fanconi anemia | no | yes | yes | under investigation |
| **5. Other neoplasms** | | | | |
| Neuroblastoma | yes | under investigation | no | no |
| Wilms' tumor (nephroblastoma) | under investigation | no | no | no |
| Brain tumor | yes | no | no | no |
| Retinoblastoma | yes | no | no | no |
| Bone tumor (Ewing's sarcoma) | yes | under investigation | no | no |
| **6. Autoimmune diseases** | | | | |

| | | | | |
|---|---|---|---|---|
| Juvenile rheumatoid arthritis | under investigation | no | no | no |

| | Autologous umbilical cord blood transplantation – own blood | Allogeneic umbilical cord blood transplantation – from a sibling | Allogeneic umbilical cord blood transplantation – from a matched unrelated donor | Allogeneic umbilical cord blood transplantation – from a mismatched unrelated donor |
|---|---|---|---|---|
| Systemic lupus erythematosus | under investigation | no | no | no |
| Crohn's disease | under investigation | no | no | no |
| Evans syndrome | under investigation | no | no | no |
| **7. Mucopolysaccharidosis** | | | | |
| Hurler's syndrome – MPS I H | no | yes | yes | under investigation |
| Maroteaux-Lamy syndrome – MPS VI | no | under investigation | under investigation | under investigation |
| **8. Hereditary red blood cell disorders** | | | | |
| Beta-thalassemia | no | yes | under investigation | no |
| Diamond-Blackfan anemia | no | yes | under investigation | no |
| Sickle cell anemia | no | yes | under investigation | no |
| **9. Hereditary immune system dysfunctions** | | | | |
| Ataxia telangiectasia | no | yes | yes | yes |
| Bare lymphocyte syndrome | no | yes | yes | yes |

| | Autologous umbilical cord blood transplantation – own blood | Allogeneic umbilical cord blood transplantation – from a sibling | Allogeneic umbilical cord blood transplantation – from a matched unrelated donor | Allogeneic umbilical cord blood transplantation – from a mismatched unrelated donor |
|---|---|---|---|---|
| Common variable immunodeficiency | no | yes | yes | yes |
| DiGeorge syndrome | no | yes | yes | yes |
| Kostmann disease | no | yes | yes | yes |
| Leukocyte adhesion deficiency | no | yes | yes | yes |
| Hyper IgM syndrome | no | yes | yes | yes |
| Omenn syndrome | no | yes | yes | yes |
| Wiskott-Aldrich syndrome | no | yes | yes | yes |
| X-linked lymphoproliferative syndromes | no | yes | yes | yes |
| Severe combined immunodeficiency (SCID) | no | yes | yes | yes |
| SCID, T-cell negative, B-cell negative | no | yes | yes | yes |

| | Autologous umbilical cord blood transplantation – own blood | Allogeneic umbilical cord blood transplantation – from a sibling | Allogeneic umbilical cord blood transplantation – from a matched unrelated donor | Allogeneic umbilical cord blood transplantation – from a mismatched unrelated donor |
|---|---|---|---|---|
| SCID, T-cell negative, B-cell positive with normal B-cell count | no | yes | yes | yes |
| SCID with adenosine deaminase deficiency | no | yes | yes | yes |
| **10. Other hereditary diseases** | | | | |
| Cartilage-hair hypoplasia | no | yes | yes | yes |
| Glanzmann's thrombasthenia | no | yes | yes | yes |
| Lesch-Nyhan syndrome | no | yes | yes | yes |
| Osteopetrosis (marble bone disease) | no | yes | yes | yes |
| **11. Lysosomal storage disorders** | | | | |
| Adrenoleukodystrophy | no | yes | yes | under investigation |
| **12. Diseases treated with non-standard hematopoietic stem cell transplants** | | | | |

| | | |
|---|---|---|
| Type 1 diabetes mellitus | yes | under investigation |

| | Autologous umbilical cord blood transplantation – own blood | Allogeneic umbilical cord blood transplantation – from a sibling | Allogeneic umbilical cord blood transplantation – from a matched unrelated donor | Allogeneic umbilical cord blood transplantation – from a mismatched unrelated donor |
|---|---|---|---|---|
| Cerebral palsy | yes | under investigation | | |
| Paresis of upper and lower extremities | no | under investigation | | |
| Anoxic brain injury | yes | under investigation | | |
| Traumatic Brain Injury | yes | under investigation | | |

**The table below shows sample indications for umbilical cord blood transplantation in adults.**

| | Autologous hematopoietic stem cell transplantation – own cells | Allogeneic umbilical cord blood transplantation – from a sibling | Allogeneic umbilical cord blood transplantation – from a matched unrelated donor | Allogeneic umbilical cord blood transplantation – from a mismatched unrelated donor |
|---|---|---|---|---|
| **1. Leukemias** | | | | |
| Acute lymphoblastic leukemia | under investigation | yes | yes | under investigation |

| | Autologous hematopoietic stem cell transplantation – own cells | Allogeneic umbilical cord blood transplantation – from a sibling | Allogeneic umbilical cord blood transplantation – from a matched unrelated donor | Allogeneic umbilical cord blood transplantation – from a mismatched unrelated donor |
|---|---|---|---|---|
| Acute myeloid leukemia | depending on the disease stage | yes | under investigation | under investigation |
| Chronic lymphocytic leukemia | under investigation | yes | yes | under investigation |
| Chronic myeloid leukemia | under investigation | yes | yes | under investigation |
| **2. Proliferative disorders of the lymphatic system** | | | | |
| Malignant lymphogranulomatosis (Hodgkin lymphoma) | depending on the disease stage | under investigation | under investigation | under investigation |
| Malignant lymphoma | depending on the disease stage | under investigation | under investigation | no |
| **3. Myelodysplastic syndromes** | | | | |
| Refractory anemia - RA | under investigation | yes | yes | under investigation |
| Refractory anemia with | under investigation | yes | yes | under investigation |

| | Autologous hematopoietic stem cell transplantation – own cells | Allogeneic umbilical cord blood transplantation – from a sibling | Allogeneic umbilical cord blood transplantation – from a matched unrelated donor | Allogeneic umbilical cord blood transplantation – from a mismatched unrelated donor |
|---|---|---|---|---|
| excess blasts – RAEB | | | | |
| Refractory anemia with excess blasts in transformation – RAEB-T | under investigation | yes | under investigation | under investigation |
| **4. Plasma cell disorders** | | | | |
| Multiple myeloma | yes | under investigation | under investigation | no |
| **5. Diseases caused by stem cell defects** | | | | |
| Severe aplastic anemia | no | yes | yes | under investigation |
| Paroxysmal nocturnal hemoglobinuria | no | yes | under investigation | under investigation |
| **6. Other neoplasms** | | | | |
| Germ cell tumor | yes | no | no | no |
| Breast cancer | under investigation | under investigation | under investigation | no |

| | | | | |
|---|---|---|---|---|
| Ovarian carcinoma | under investigation | under investigation | under investigation | no |

| | Autologous hematopoietic stem cell transplantation – own cells | Allogeneic umbilical cord blood transplantation – from a sibling | Allogeneic umbilical cord blood transplantation – from a matched unrelated donor | Allogeneic umbilical cord blood transplantation – from a mismatched unrelated donor |
|---|---|---|---|---|
| Small cell lung cancer | under investigation | no | no | no |
| Kidney cancer | no | under investigation | under investigation | no |
| Glioma – tumor of the central nervous system | under investigation | no | no | no |
| Rheumatoid Arthritis | under investigation | no | no | no |
| Multiple Sclerosis | under investigation | under investigation | no | no |
| Systemic lupus erythematosus | under investigation | under investigation | no | no |
| Crohn’s disease | under investigation | no | no | no |
| **7. Other diseases** | | | | |
| Scleroderma | under investigation | under investigation | no | no |