

## Umbilical cord blood therapies

There are now **more than 80 diseases** that can be treated with hematopoietic stem cells (HSCs) collected from umbilical cord blood. For some diseases, these treatments are the only therapy, and for others, they are only used when first-line treatments have failed or the disease is very aggressive.

- **Allogeneic:** The patient receives stem cells from a compatible donor, whether a sibling or unrelated donor.
- **Autologous:** The patient receives his or her own stem cells. By registering cord blood in a family bank, parents ensure that their child can use his or her own cells for autologous treatment or that an immediate family member can benefit from related allogeneic treatment.

## Cancers

### Leukemias (a cancer of the blood immune system)

| Diagnosis                                | Autologous | Allogeneic |
|--|------------|------------|
| Acute biphenotypic leukemia              | ✗          | ✓          |
| Acute lymphoblastic leukemia (ALL)       | ✗          | ✓          |
| Acute myeloid leukemia (AML)             | ✗          | ✓          |
| Acute undifferentiated leukemia          | ✗          | ✓          |
| Chronic lymphocytic leukemia (CLL)       | ✗          | ✓          |
| Chronic myeloid leukemia (CML)           | ✗          | ✓          |
| Juvenile chronic myeloid leukemia (JCML) | ✗          | ✓          |
| Juvenile myelomonocytic leukemia (JMML)  | ✗          | ✓          |

### Myelodysplastic syndromes (also called pre-leukemias)

| Diagnosis                                  | Autologous | Allogeneic |
|--|------------|------------|
| Refractory anemia                          | ✗          | ✓          |
| Refractory anemia with ringed sideroblasts | ✗          | ✓          |

|  |                          |                                     |
|--|--------------------------|-------------------------------------|
| Refractory anemia with excess blasts                   | <input type="checkbox"/> | <input checked="" type="checkbox"/> |
| Refractory anemia with excess blasts in transformation | <input type="checkbox"/> | <input checked="" type="checkbox"/> |
| Chronic myelomonocytic leukemia (CMML)                 | <input type="checkbox"/> | <input checked="" type="checkbox"/> |

### Lymphomas (a cancer of the blood immune system)

| Diagnosis                               | Autologous                          | Allogeneic                          |
|---|-------------------------------------|-------------------------------------|
| Hodgkin lymphoma                        | <input checked="" type="checkbox"/> | <input checked="" type="checkbox"/> |
| Non-Hodgkin lymphoma (Burkitt lymphoma) | <input checked="" type="checkbox"/> | <input checked="" type="checkbox"/> |

### Solid tumors (not originating from blood or the immune system)

| Diagnosis       | Autologous                          | Allogeneic               |
|-----------------|-------------------------------------|--------------------------|
| Neuroblastoma   | <input checked="" type="checkbox"/> | <input type="checkbox"/> |
| Retinoblastoma  | <input checked="" type="checkbox"/> | <input type="checkbox"/> |
| Medulloblastoma | <input checked="" type="checkbox"/> | <input type="checkbox"/> |

## Blood disorders

### Anemias (deficiency or malformation of red blood cells)

| Diagnosis                                 | Autologous               | Allogeneic                          |
|---|--------------------------|-------------------------------------|
| Aplastic anemia                           | <input type="checkbox"/> | <input checked="" type="checkbox"/> |
| Congenital dyserythropoietic anemia       | <input type="checkbox"/> | <input checked="" type="checkbox"/> |
| Fanconi anemia                            | <input type="checkbox"/> | <input checked="" type="checkbox"/> |
| Paroxysmal nocturnal hemoglobinuria (PNH) | <input type="checkbox"/> | <input checked="" type="checkbox"/> |

### Inherited red blood cell disorders

| Diagnosis               | Autologous               | Allogeneic                          |
|-------------------------|--------------------------|-------------------------------------|
| Beta-thalassemia major  | <input type="checkbox"/> | <input checked="" type="checkbox"/> |
| Diamond-Blackfan anemia | <input type="checkbox"/> | <input checked="" type="checkbox"/> |
| Pure red cell aplasia   | <input type="checkbox"/> | <input checked="" type="checkbox"/> |

Sickle cell disease



### Inherited platelet disorders

#### Diagnosis

Autologous Allogeneic

Amégacaryocytose / Thrombocytopénie congénitale



Thrombasthénie de Glanzmann



### Inherited immune system disorders: severe combined immunodeficiency (SCID)

#### Diagnosis

Autologous Allogeneic

SCID with adenosine deaminase deficiency (ADA-SCID)



X-linked SCID



SCID with absence of T and B cells



SCID with absence of T cells and normal B cells



Omenn syndrome



### Inherited immune system disorders: neutropenias

#### Diagnosis

Autologous Allogeneic

Genetic infantile agranulocytosis (Kostmann syndrome)



Myelokathexis



### Inherited immune system disorders – Others

#### Diagnosis

Autologous Allogeneic

Ataxia-telangiectasia



Nude lymphocyte syndrome



Common variable immunodeficiency



DiGeorge syndrome



Leukocyte adhesion deficiency



Lymphoproliferative disorders



X-linked lymphoproliferative disorder (also called Epstein-Barr virus susceptibility)



Wiskott-Aldrich syndrome



### Myeloproliferative disorders

#### Diagnosis

Autologous Allogeneic

Acute myelofibrosis



Agnogenic myeloid metaplasia (myelofibrosis)



Polycythemia vera



Essential thrombocythemia



### Phagocyte disorders

#### Diagnosis

Autologous Allogeneic

Chediak-Higashi syndrome



Chronic granulomatous disease



Neutrophil actin deficiency



Reticular dysgenesis



### Bone marrow cancers

#### Diagnosis

Autologous Allogeneic

Multiple myeloma



Primary plasma cell leukemia (PCL)



Secondary plasma cell leukemia



Waldenström macroglobulinemia



### Immune disorders

### Inherited diseases (immune system and other organs)

#### Diagnosis

Autologous Allogeneic

Hypoplasia of cartilage and hair



|  |                          |                                     |
|--|--------------------------|-------------------------------------|
| Gunther's disease (erythropoietic porphyria) | <input type="checkbox"/> | <input checked="" type="checkbox"/> |
| Hermansky-Pudlak syndrome                    | <input type="checkbox"/> | <input checked="" type="checkbox"/> |
| Pearson syndrome                             | <input type="checkbox"/> | <input checked="" type="checkbox"/> |
| Shwachman-Diamond syndrome                   | <input type="checkbox"/> | <input checked="" type="checkbox"/> |
| Systemic mastocytosis                        | <input type="checkbox"/> | <input checked="" type="checkbox"/> |

## Metabolic disorders

### Mucopolysaccharidosis (MPS) storage diseases

| Diagnosis                        | Autologous               | Allogeneic                          |
|----------------------------------|--------------------------|-------------------------------------|
| Hunter syndrome (MPS II)         | <input type="checkbox"/> | <input checked="" type="checkbox"/> |
| Hurler syndrome (MPS IH)         | <input type="checkbox"/> | <input checked="" type="checkbox"/> |
| Maroteaux-Lamy syndrome (MPS VI) | <input type="checkbox"/> | <input checked="" type="checkbox"/> |
| Morquio syndrome (MPS IV)        | <input type="checkbox"/> | <input checked="" type="checkbox"/> |
| Sanfilippo syndrome (MPS III)    | <input type="checkbox"/> | <input checked="" type="checkbox"/> |
| Scheie syndrome (MPS IS)         | <input type="checkbox"/> | <input checked="" type="checkbox"/> |

### Sly syndrome, beta-glucuronidase deficiency (MPS VII)

| Diagnosis                           | Autologous               | Allogeneic                          |
|-------------------------------------|--------------------------|-------------------------------------|
| Mucopolipidosis II (I-cell disease) | <input type="checkbox"/> | <input checked="" type="checkbox"/> |

### Leukodystrophy disorders

| Diagnosis  | Autologous               | Allogeneic                          |
|--|--------------------------|-------------------------------------|
| Adrenoleukodystrophy (ALD) / Adrenomyeloneuropathy (AMN) | <input type="checkbox"/> | <input checked="" type="checkbox"/> |
| Krabbe disease (globoid cell leukodystrophy)             | <input type="checkbox"/> | <input checked="" type="checkbox"/> |
| Metachromatic leukodystrophy                             | <input type="checkbox"/> | <input checked="" type="checkbox"/> |
| Pelizaeus-Merzbacher disease                             | <input type="checkbox"/> | <input checked="" type="checkbox"/> |

### Lysosomal storage diseases

| Diagnosis            | Autologous | Allogeneic |
|----------------------|------------|------------|
| Niemann-Pick disease | ✗          | ✓          |
| Sandhoff disease     | ✗          | ✓          |
| Wolman disease       | ✗          | ✓          |

### Inherited diseases - Others

| Diagnosis            | Autologous | Allogeneic |
|----------------------|------------|------------|
| Lesch-Nyhan syndrome | ✗          | ✓          |
| Osteopetrosis        | ✗          | ✓          |

## Clinical trials using stem cells

Neonatal stem cells are at the center of hundreds of clinical trials across a variety of conditions, highlighting their versatility and therapeutic potential. In some cases, they are a primary treatment option, while in others, they are explored when conventional therapies prove insufficient. These ongoing trials highlight the immense potential and promise that neonatal stem cells hold to transform the medical treatment landscape and offer hope to patients facing a variety of health challenges.

### Neurological disorders

| Diagnosis                             | Cord blood | Umbilical tissue | Placental tissue |
|---------------------------------------|------------|------------------|------------------|
| Alzheimer's disease                   | ✓          | ✓                | ✗                |
| Autism                                | ✓          | ✓                | ✓                |
| Cerebral palsy                        | ✓          | ✓                | ✓                |
| Encephalopathy                        | ✓          | ✗                | ✗                |
| Developmental delay                   | ✓          | ✗                | ✗                |
| Hearing loss (acquired sensorineural) | ✓          | ✗                | ✗                |
| Intraventricular hemorrhage           | ✓          | ✗                | ✗                |
| Parkinson's disease                   | ✓          | ✓                | ✓                |
| Spinal cord injury                    | ✓          | ✓                | ✓                |

|                        |                                     |                                     |                                     |
|------------------------|-------------------------------------|-------------------------------------|-------------------------------------|
| Stroke                 | <input checked="" type="checkbox"/> | <input checked="" type="checkbox"/> | <input type="checkbox"/>            |
| Traumatic brain injury | <input checked="" type="checkbox"/> | <input checked="" type="checkbox"/> | <input checked="" type="checkbox"/> |

### Autoimmune diseases

| Diagnosis                           | Cord blood                          | Umbilical tissue                    | Placental tissue                    |
|-------------------------------------|-------------------------------------|-------------------------------------|-------------------------------------|
| Alopecia areata                     | <input checked="" type="checkbox"/> | <input type="checkbox"/>            | <input type="checkbox"/>            |
| Amyotrophic lateral sclerosis (ALS) | <input checked="" type="checkbox"/> | <input checked="" type="checkbox"/> | <input type="checkbox"/>            |
| Crohn's disease                     | <input checked="" type="checkbox"/> | <input type="checkbox"/>            | <input type="checkbox"/>            |
| Eczema (atopic dermatitis)          | <input checked="" type="checkbox"/> | <input type="checkbox"/>            | <input type="checkbox"/>            |
| Graft-versus-host disease (GvHD)    | <input checked="" type="checkbox"/> | <input checked="" type="checkbox"/> | <input checked="" type="checkbox"/> |
| Lupus                               | <input checked="" type="checkbox"/> | <input checked="" type="checkbox"/> | <input type="checkbox"/>            |
| Multiple sclerosis                  | <input type="checkbox"/>            | <input checked="" type="checkbox"/> | <input checked="" type="checkbox"/> |
| Psoriasis                           | <input type="checkbox"/>            | <input checked="" type="checkbox"/> | <input type="checkbox"/>            |
| Rheumatoid arthritis                | <input checked="" type="checkbox"/> | <input checked="" type="checkbox"/> | <input type="checkbox"/>            |
| Scleroderma                         | <input checked="" type="checkbox"/> | <input type="checkbox"/>            | <input type="checkbox"/>            |
| Systemic sclerosis                  | <input checked="" type="checkbox"/> | <input type="checkbox"/>            | <input type="checkbox"/>            |
| Ulcerative colitis                  | <input type="checkbox"/>            | <input type="checkbox"/>            | <input type="checkbox"/>            |

### Cardiovascular

| Diagnosis                                  | Cord blood                          | Umbilical tissue                    | Placental tissue         |
|--|-------------------------------------|-------------------------------------|--------------------------|
| Acute myocardial infarction (heart attack) | <input type="checkbox"/>            | <input checked="" type="checkbox"/> | <input type="checkbox"/> |
| Cardiomyopathy                             | <input type="checkbox"/>            | <input checked="" type="checkbox"/> | <input type="checkbox"/> |
| Critical limb ischemia (CLI)               | <input checked="" type="checkbox"/> | <input checked="" type="checkbox"/> | <input type="checkbox"/> |
| Heart failure                              | <input type="checkbox"/>            | <input checked="" type="checkbox"/> | <input type="checkbox"/> |
| Peripheral artery disease (PAD)            | <input checked="" type="checkbox"/> | <input checked="" type="checkbox"/> | <input type="checkbox"/> |

Surgery for congenital heart defects



## Diabetes

### Diagnosis

Cord blood    Umbilical tissue    Placental tissue

Type 1 diabetes (autoimmune)



Type 2 diabetes



Diabetic foot ulcer



Diabetic peripheral neuropathy



## Genetic and/or metabolic disorders

### Diagnosis

Cord blood    Umbilical tissue    Placental tissue

Frailty associated with aging



Duchenne muscular dystrophy



Epidermolysis bullosa



Hereditary ataxia



Lysosomal storage diseases



Metabolic syndrome



Severe combined immunodeficiency



Spinal muscular atrophy



Tay-Sachs disease



## Orthopedic

### Diagnosis

Cord blood    Umbilical tissue    Placental tissue

Ankylosing spondylitis



Cartilage lesion



Cleft palate repair





|                       |                                     |                                     |                          |
|-----------------------|-------------------------------------|-------------------------------------|--------------------------|
| Non-union fractures   | <input type="checkbox"/>            | <input checked="" type="checkbox"/> | <input type="checkbox"/> |
| Osteoarthritis        | <input checked="" type="checkbox"/> | <input checked="" type="checkbox"/> | <input type="checkbox"/> |
| Osteochondral lesion  | <input checked="" type="checkbox"/> | <input type="checkbox"/>            | <input type="checkbox"/> |
| Spinal fusion surgery | <input type="checkbox"/>            | <input checked="" type="checkbox"/> | <input type="checkbox"/> |

## Others

| Diagnosis                                  | Cord blood                          | Umbilical tissue                    | Placental tissue                    |
|--|-------------------------------------|-------------------------------------|-------------------------------------|
| Acute respiratory distress syndrome (ARDS) | <input type="checkbox"/>            | <input checked="" type="checkbox"/> | <input type="checkbox"/>            |
| Bronchopulmonary dysplasia (BPD)           | <input checked="" type="checkbox"/> | <input checked="" type="checkbox"/> | <input type="checkbox"/>            |
| Erectile dysfunction                       | <input type="checkbox"/>            | <input checked="" type="checkbox"/> | <input type="checkbox"/>            |
| Eye diseases                               | <input checked="" type="checkbox"/> | <input checked="" type="checkbox"/> | <input checked="" type="checkbox"/> |
| Fistula                                    | <input type="checkbox"/>            | <input checked="" type="checkbox"/> | <input type="checkbox"/>            |
| HIV (Human Immunodeficiency Virus)         | <input checked="" type="checkbox"/> | <input type="checkbox"/>            | <input type="checkbox"/>            |
| Liver cirrhosis                            | <input checked="" type="checkbox"/> | <input checked="" type="checkbox"/> | <input type="checkbox"/>            |
| Liver failure                              | <input checked="" type="checkbox"/> | <input checked="" type="checkbox"/> | <input type="checkbox"/>            |
| Peyronie's disease                         | <input type="checkbox"/>            | <input checked="" type="checkbox"/> | <input type="checkbox"/>            |
| Kidney failure                             | <input type="checkbox"/>            | <input checked="" type="checkbox"/> | <input type="checkbox"/>            |
| Premature ovarian insufficiency            | <input checked="" type="checkbox"/> | <input checked="" type="checkbox"/> | <input type="checkbox"/>            |
| Injuries                                   | <input type="checkbox"/>            | <input checked="" type="checkbox"/> | <input checked="" type="checkbox"/> |
| Uterine scars                              | <input type="checkbox"/>            | <input checked="" type="checkbox"/> | <input type="checkbox"/>            |