babycell™

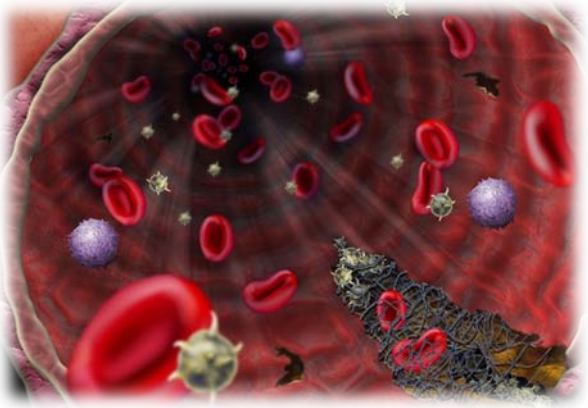
The Life Saving Cord Blood Bank

HAEMOPHILIA & UMBILICAL CORD BLOOD TRANSPLANT

babycell
The Life Saving Cord Blood Bank

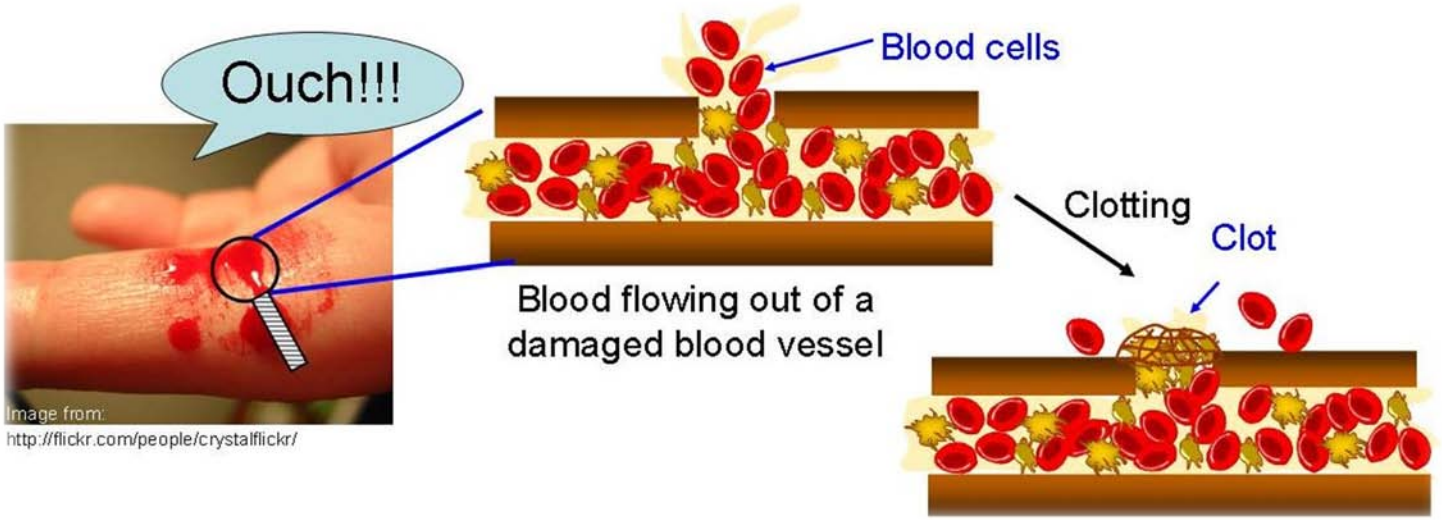
Haemostatic System in Body

- Blood vessels
- Platelets
- Plasma coagulation system
- Proteolytic or Fibrinolytic system



How Bleeding Stops

- Vasoconstriction
- Platelet plug formation
- Clotting cascade activated to form fibrin clot



What is Haemophilia?

- Haemophilia is an inherited bleeding disorder in which the ability of the blood to clot is severely reduced, causing the sufferer to bleed severely from even a slight injury.

- The condition is typically caused by a hereditary lack of a coagulation factor, most often factor VIII.

Types of Bleeding Disorders

- Haemophilia A (factor VIII deficiency)
- Haemophilia B (factor IX deficiency)
- Von Willebrand Disease (vWD)
- Other

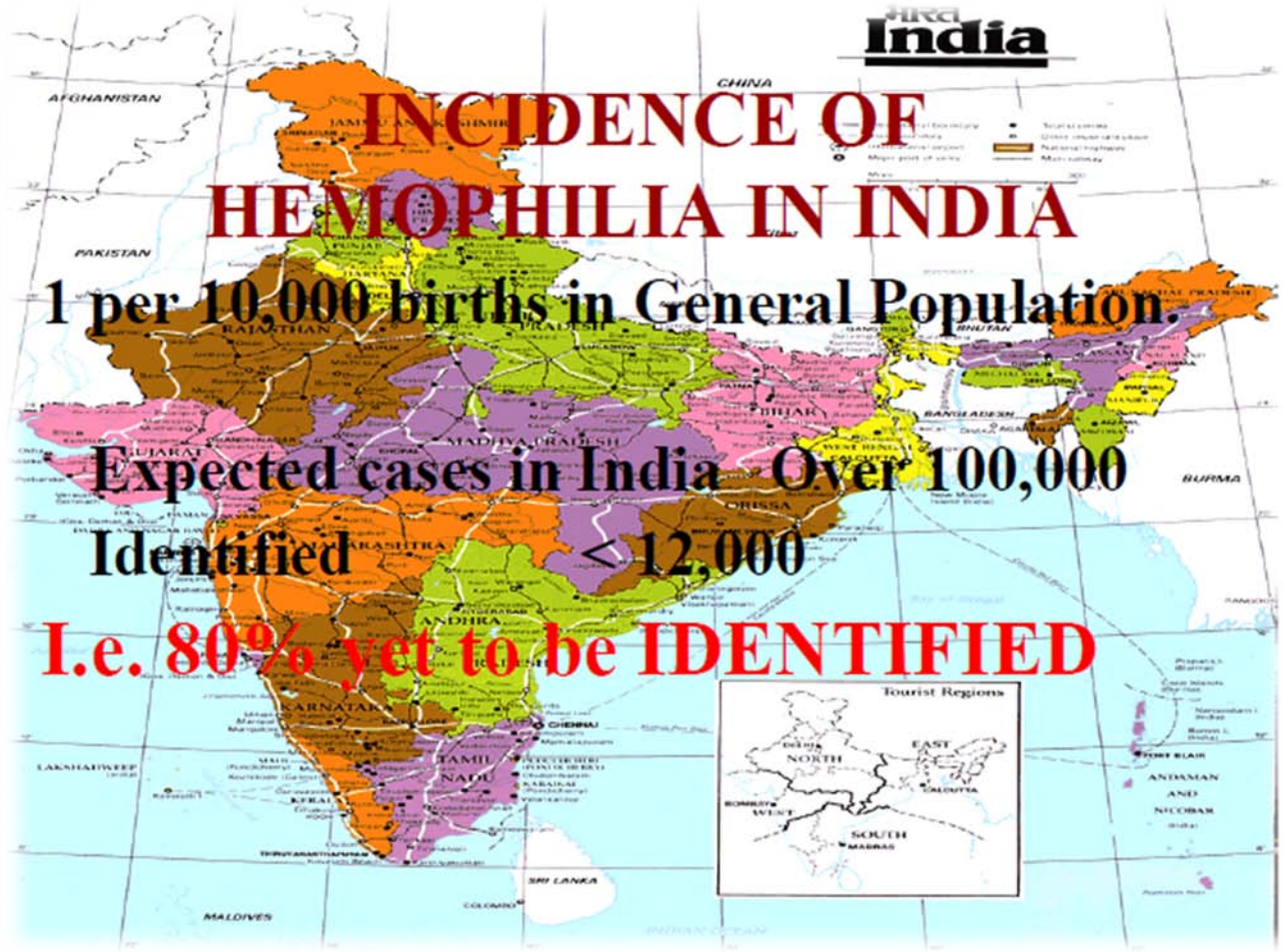
Classification of Haemophilia

Haemophilia A and B are classified as mild, moderate, or severe, depending on the amount of clotting factor VIII or IX in the blood.

Mild haemophilia	5–40 percent of normal clotting factor
Moderate haemophilia	1–5 percent of normal clotting factor
Severe haemophilia	Less than 1 percent of normal clotting factor

Types of Bleeds

- Joint bleeding - hemarthrosis
- Muscle haemorrhage
- Soft tissue
- Life threatening-bleeding- Head, Intracranial, Abdominal, intra GI
- Other



Reference: Kar A, Phadnis S, Dharmarajan S, Nakade J. Epidemiology and social costs of haemophilia in India. (Ind.J.Medical Res. ms.in press,)

Incidence of Haemophilia?

- 1 per 10 000 births,
- Women carriers , males sufferers
- Severity Classified as mild, moderate or severe depending on the deficiency of factor
- The number of patients reported annually from India during the last five years, is more than those being reported from developed nations.

Reference: Kar A, Phadnis S, Dharmarajan S, Nakade J. Epidemiology and social costs of haemophilia in India. (Ind.J.Medical Res. ms.in press,)

Detection of Haemophilia

- **Family history**

- **Symptoms**
  - Bruising
  - Bleeding with circumcision
  - Muscle, joint, or soft tissue bleeding

- **Laboratory testing**
  - Screening tests
  - Clotting factor tests.

Symptoms of Haemophilia

Signs of external bleeding may include:

- Bleeding in the mouth from a cut or bite or from cutting or losing a tooth
- Nosebleeds for no obvious reason
- Heavy bleeding from a minor cut
- Bleeding from a cut that resumes after stopping for a short time

Signs of internal bleeding may include:

- Blood in the urine (from bleeding in the kidneys or bladder)
- Blood in the stool (from bleeding in the intestines or stomach)
- Large bruises (from bleeding into the large muscles of the body)
- Swollen, hot to touch, and painful

Complications of Bleeding

- Flexion contractures
- Joint arthritis / arthropathy
- Chronic pain
- Muscle atrophy
- Compartment syndrome
- Neurologic impairment

Treatment of Haemophilia

- Replacement of missing clotting protein
  - On demand
  - Prophylaxis
- Antifibrinolytic Agents
  - Amicar
- Supportive measures
  - Icing
  - Immobilization
  - Rest

Stem cell Treatment in Haemophilia

Comparative Clinical Pathology
January 2014, Volume 23, Issue 1, pp 193-198

Differentiation of bone marrow hematopoietic stem cells into FVIII-producing hepatocytes: approach to hemophilia treatment

BM-HSCs from haemophilic families were cultured in liquid culture containing hepatocyte growth factor for 6 days. Differentiation into hepatocytes was evaluated by alpha-fetoprotein (AFP) expression using immunocytochemistry.

Functional evaluation of transdifferentiation into hepatic lineage was done through albumin synthesis in culture supernatant using microalbumin assay kit, factor VIII activity by one stage clotting assay and expression of FVIII mRNA by RT-PCR. BM-HSCs-derived hepatocytes showed positive AFP expression with a mean of 11 %.

Functional tests performed showed their ability to produce albumin and perform FVIII activity. Also, FVIII mRNA expression was detected. Inducing the differentiation of BM-HSCs by in vitro manipulation may become a valuable tool to provide a cell source for hepatocyte transplant procedures for treatment of haemophilia patients.

Cord Blood Treatment in Haemophilia

Successful cord blood transplantation in a patient with malignant infantile osteopetrosis and haemophilia

A patient with an autosomal recessive form of MIOP successfully underwent a cord blood HCT complicated by the presence of mild haemophilia A and HCT-related complications including delayed engraftment, sinusoidal obstruction syndrome, and need for multiple invasive procedures without clinically significant bleeding. Umbilical cord blood demonstrated safety and efficacy for prevention of bleeding in haemophilia patient.

Referance: David Buchbinder et al; Successful cord blood transplantation in a patient with malignant infantile osteopetrosis and haemophilia Pediatric Transplantation Volume 17, Issue 1, (/doi/10.1111/petr.2013.17.issue-1/issuetoc) pages E20–E24, February 2013

Reasons to choose cord blood for stem cell Transplant

A doctor might choose cord blood because of some of the ways it differs from marrow or peripheral blood.

- **More tolerant matching**

  A close match between the patient and the donor or cord blood unit can improve a patient's outcome after transplant. If you have an uncommon tissue type, you may not find a closely matched adult donor for you. However, a cord blood unit may be the best option.

- **More quickly available**

  Cord blood units are stored and ready to use. A cord blood unit can be selected and delivered to the transplant center in less than two weeks whereas it can take two months or more to find an unrelated marrow or peripheral blood donor.

- **Less graft-versus-host disease**

  Graft-versus-host disease (GVHD) is a common complication after an allogeneic transplant (which uses cells unrelated donor). GVHD can range from mild to life-threatening. There is less chance of GVHD when the cord blood transplant is done using cells from a family membr.

UCB transplantation Flowchart

Step 1	• Collection, Storage and Cryopreservation of Umbilical Cord
Step 2	• Day -30 to -11: HLA typing and donor/recipient matching
Step 3	• Day -10: Patient Admission and Isolation in ICU Room
Step 4	• Day -8: Pre Transplantation conditioning Regimen for 8-10 day before transplantation
Step 5	• Day 0: Umbilical Cord Blood transplantation in Patient
Step 6	• Day 1 to Day 25: Post Transplantation follow up and Monitoring

Step 1 - Collection of UCB

- Umbilical cord Blood is collected by trained paramedic as per standard procedure and transported for storage.
- One can also plan second child and store umbilical cord blood for the treatment of their diseased first child.
- Collected cord blood sample is stored at GMP laboratory for future use.

Step 2- HLA typing

- Before implantation, HLA typing is needed to do for checking donor- recipient compatibility.
- Also attention required in the case of Blood Groups of both donor- recipient for ABO incompatibility.
- Assessment of medical history and reports.

Step 3- Hospitalization

- Patient is admitted to the hospital before 8-10 days of transplantation date.
- Patient is completely isolated from the outside and keep in ICU unit to avoid contamination.
- Access is restricted to limited personnel only

Step 4- Pre-operative Regimen

- Preparative regimen is given to the patient to prepare for implantation.

- This includes medication, antibiotics and chemotherapy to ablate the patient's immune system and avoid GVHD after transplantation.

Step 5- UCB transplantation

- Stored umbilical cord blood sample is procured from the lab before transplantation.
- Physician transplant the required quantity of umbilical cord blood cells intravenously into the patients body.
- The intravenous part of the transplant takes approximately 15 minutes.

Step 6- Post transplantation Follow up

- After transplantation, patient will be under strict monitoring for 4-5 weeks for any side effects or complications.
- Hematological engraftments is checked using blood tests and analysis.
- It can take months to recover full immune power for patient after transplantation.

Haemophilia Treatment Centers in India

- Lok Nayak Jayprakash Hospital, New Delhi
- Institute For Child Health & Hospital For Children, Chennai
- JIPMER Hospital, Chennai
- Kolkata Medical College, Kolkata
- Patna Medical College & Hospital (PMCH)
- Victoria Hospital, Bangolore

Source: Haemophilia Federation of India

http://www.hemophilia.in/index.php?option=com_content&view=article&id=37:psychosocial support&catid=19:patent-care&Itemid=137

Thank You

No Child is Born to Die

babycell™
The Life Saving Cord Blood Bank

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