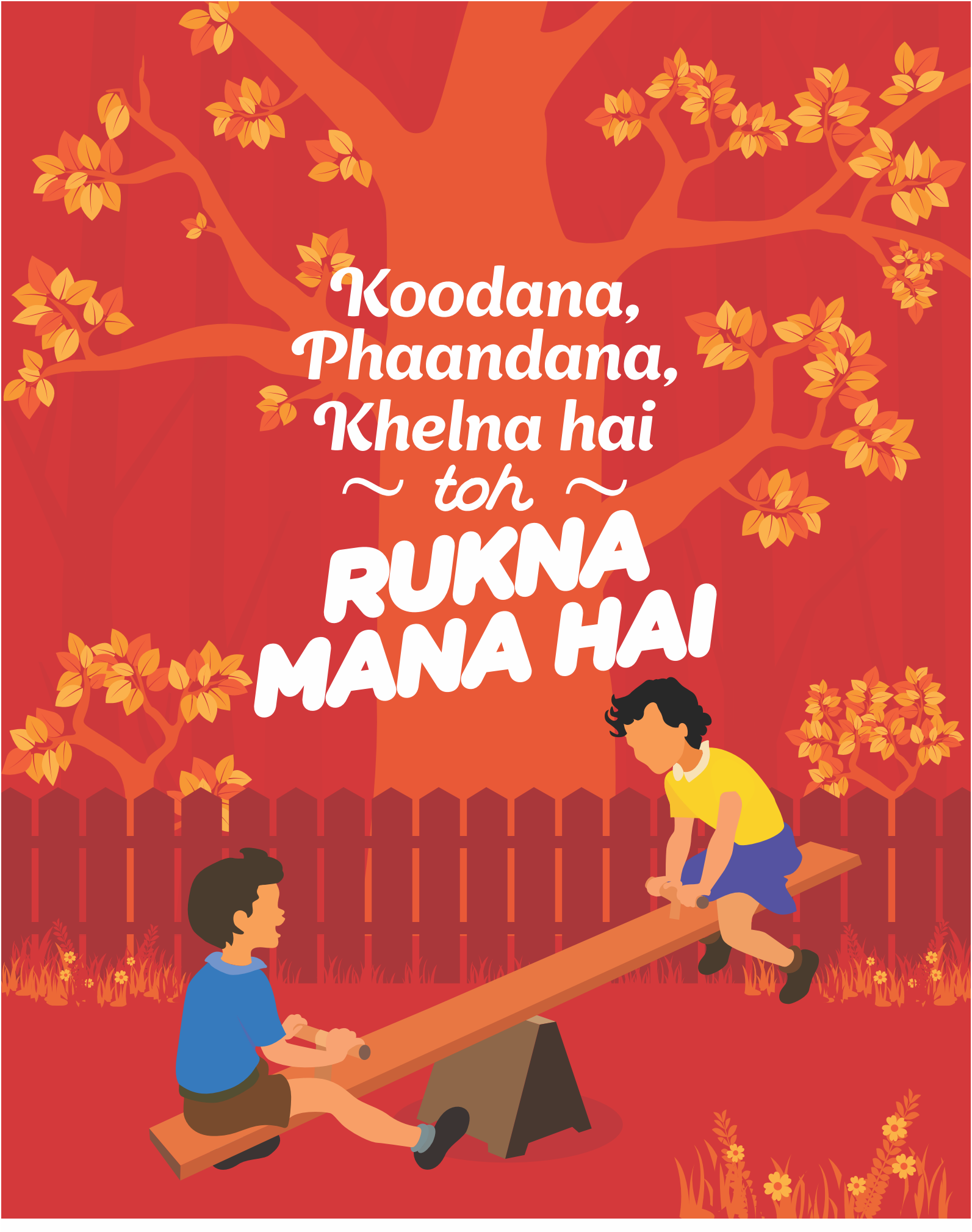
Koodana, Phaandana, Khelna hai ~ toh ~ RUKNA MANA HAI



WHAT IS **HEMOPHILIA?**[1]

Hemophilia is an inherited condition that causes **bleeding for a long time** after injury or surgery and **painful swelling of the joints** either after injury or even without injury. ("Inherited" means that the disease is passed from parents to children through their genes).

WHAT IS **THE CAUSE?**[1]

Hemophilia is caused due to a **deficiency of clotting factor**, that results in increased bleeding.

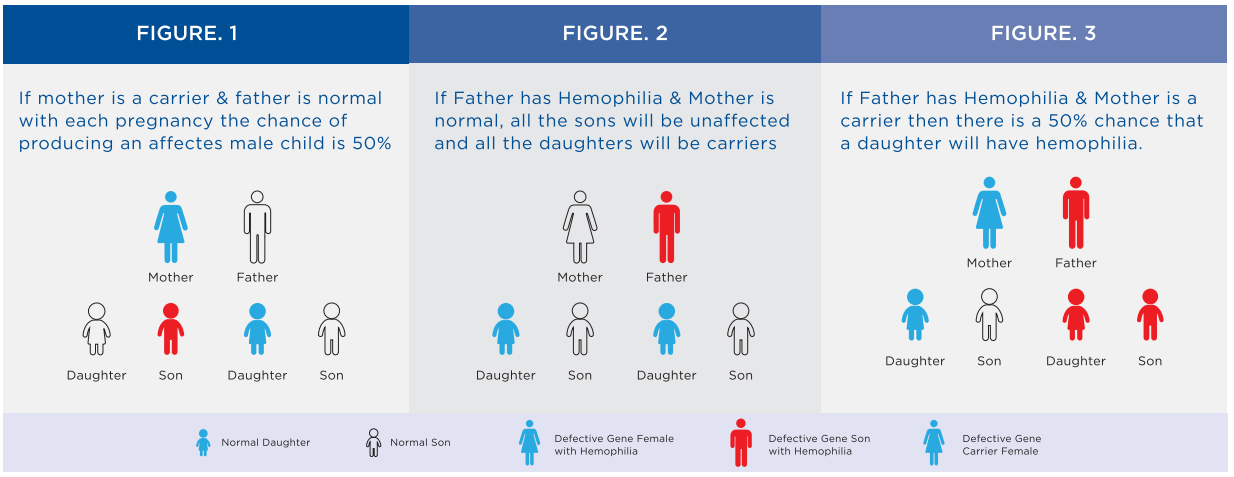
WHAT ARE THE **TYPES OF HEMOPHILIA?**[1]

THERE ARE TWO TYPES:
HEMOPHILIA A caused by clotting **factor VIII deficiency**, which is more common and occurs in about **1 in 5,000 births.**

HEMOPHILIA B caused by **factor IX deficiency** is less common and occurs in around **1 in about 20,000 births.**

HOW IS IT **INHERITED?**[1]

The disease of Hemophilia is **X linked** and inherited from the mother, though the disease is present in males. A **family history of maternal uncles or other male affected relatives on the mother's side** are often present. Though many cases are due to a **new acquired mutation** in the genes, and in these families, no family history is present.



Diagrammatic representation of inheritance of Hemophilia.

ARE THERE DIFFERENT **DEGREES OF SEVERITY IN HEMOPHILIA?**[1]

Yes, the severity is graded by measuring the baseline level of factor (without treatment) in a patient. This level is fixed for each patient; their family members may have different levels of factor if they too suffer from the disease.

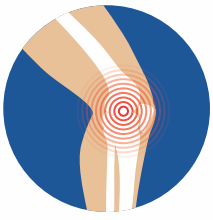
	SEVERE	MODERATE	MILD
Factor Activity (% of normal)	<1%	1% to 5%	>5% to <40%
Pattern of Bleeding Episodes	Spontaneous	Minor Trauma	Major Trauma Surgery

WHAT ARE THE COMMON **SYMPTOMS OF HEMOPHILIA?**[2]

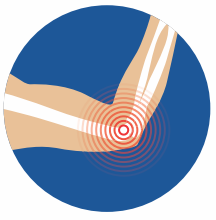
- Swelling and bruising from bleeding in ankle and knee joint
- Spontaneous bleeding in muscle and skin
- Nosebleed without a known cause
- Heavy bleeding after dental procedure
- Hematoma (blue/red patches or spots on skin)
- Unusual bleeding after vaccination

WHAT ARE THE IMPORTANT **SITES OF BLEEDING?**[1]

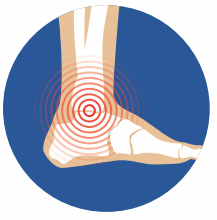
The **Ilio-psoas** (a muscle in the hip), is an important site of bleeding. If the patient has a bleed in this site, usually there is pain in the abdomen, because the muscle is deep inside no swelling is seen. Other common sites of bleeding are the main joints:



Knee Joint



Elbow Joint



Ankle Joint

Some patients may have bleeding in the brain, even after a very minor injury. Occasionally patients may have blood in the urine. It can happen with other joints like hip, shoulders, toes and etc.

HOW IS **HEMOPHILIA DIAGNOSED?**[1]

To diagnose Hemophilia the doctor will ask the patient about history of bleeding and any family history related to bleeding problems. This will reveal whether you or your family members, have bleeding problems. However, some families may not have family history of the disease. The doctor will perform a physical exam to see the status of the joints.

Then special blood tests to diagnose Hemophilia are performed, these blood tests may not be available everywhere. These include a baseline screening test, which measures the coagulation time of blood called PT (Prothrombin Time), APTT (Activated Partial Thromboplastin Time). Then if the APTT is prolonged, as is seen in Hemophilia, a more specialized test is performed to see the deficiency of Factor VIII (8) or IX(9) and the level of factor present. These test results will confirm if Hemophilia is present, what type of Hemophilia (A or B), and its severity. Once the doctors have all this information they will be able to advise you on the treatment and precautions.

Symptom Assessment & Family History

Screening Tests: PT, APTT

Confirmatory Laboratory Tests for Hemophilia A or B

WHEN IS **HEMOPHILIA DIAGNOSED?**[1]

Severe Hemophilia can result in serious bleeding problems even in babies. Children who have severe Hemophilia usually are diagnosed during the first year of life. People who have milder forms of Hemophilia may not be diagnosed until they are older or have a major injury or surgery. The bleeding problems of Hemophilia A and Hemophilia B are the same. Only the special blood tests can tell the type of Hemophilia, knowing which type is important because the treatments (injections) are different.

HOW IS **HEMOPHILIA TREATED?**[1]

The main treatment for Hemophilia is called factor replacement therapy. The deficient factor is replaced by concentrates of clotting factor VIII (for Hemophilia A) or clotting factor IX (for Hemophilia B). These factors can be collected from blood plasma donors and purified (plasma derived) or they can be produced artificially in a laboratory (recombinant). They are given to the patient by an injection through a vein (intravenous) directly into the blood.

WHAT TYPE OF **CLOTTING FACTORS ARE AVAILABLE?**[1]

There are 2 types of factors available:

1. The **purified plasma derived** clotting factor concentrates are made from human blood. The blood is treated to prevent the spread of diseases, such as Hepatitis, HIV etc. With the current methods of screening and treating donated blood, the risk of getting an infectious disease from human clotting factors is very small.

2. The latest products are called **recombinant clotting factors;** they are not made from human blood, but produced in a laboratory and have no risk of Hepatitis or HIV.

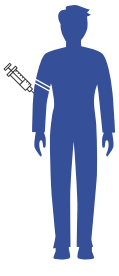
HOW IS **THE FACTOR GIVEN?**[1]

The clotting factors are supplied in vials which need reconstitution, after mixing they are given by intravenous injection over 15 minutes. For very small children a semi permanent venous access device may be needed to give the injections, in older children/people it is given directly into a vein.

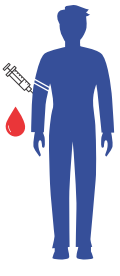
HOW IS **THE FACTOR DOSE CALCULATED?**[3]

As per World Federation of Hemophilia-
Factor VIII Dosage IUs = Body wt. (Kg) x desired factor VIII rise (%) x 0.5
Factor IX Dosage IUs = Body wt. (Kg) x desired factor IX rise (%) x 1

WHAT ARE THE **TREATMENT CHOICES?**[1]



Prophylaxis
clotting factors are injected
on a regular basis
(two or three times a week)
to prevent bleeds before they start



On Demand
involves use of clotting
factors in order to
stop the ongoing bleeding

Purna Samparq -Patient Support Program is run,
managed and implemented by Indegene Limited and sponsored by
Takeda Biopharmaceuticals India Pvt. Ltd. (formerly known as Baxalta Bioscience India Pvt. Ltd)

Purna Samparq is a non promotional program and no patient identifiable data is shared with Takeda at any stage.

18001034260 purna.samparq@indegene.com

References:

1. https://www.nhp.gov.in/haemophilia_pg
2. https://www.cdc.gov/ncbddd/hemophilia/facts.html
3. Srivastava A, Santagostino E, Dougali A et al. WFH guidelines for management of Haemophilia, 3rd edition. Haemophilia. 2020;26 (Suppl6):1-58.

CONTACT US: Takeda Biopharmaceuticals India Pvt. Ltd.
(formerly known as Baxalta Bioscience India Pvt. Ltd.)
6th Floor, Tower C, Building No. 8, DLF Cyber City, DLF Phase II, Gurgaon, Haryana- 122001, India.
Tel: +91-124-455 9100 | www.takeda.com

VV-MEDMAT-83665