

UNDERSTANDING HEMOPHILIA A

Hemophilia A is typically an X-linked, inherited bleeding disorder caused by a deficient coagulation protein, factor VIII. It is caused by mutations in the *F8* clotting factor gene.

HEMOPHILIA ARISES SPONTANEOUSLY
 – WITHOUT DIRECT FAMILY HISTORY –
IN 1 IN 3 CASES.



INCIDENCE/PREVALENCE:

Hemophilia A is the most common type of hemophilia disorder.

**~1 IN
5,000**

**HEMOPHILIA A PRESENT
IN MALE BIRTHS**



**80-85% OF TOTAL
HEMOPHILIA POPULATION**



**GLOBAL POPULATION
LIVING WITH HEMOPHILIA A***

NUMBERS ARE ESTIMATED BASED ON REPORTED CASES.
*Source: World Federation of Hemophilia

SYMPTOMS AND PRIMARY TISSUES/ORGANS AFFECTED

Depending on severity, Hemophilia A can cause prolonged or spontaneous bleeding especially into the muscles and joints or internal organs.

Although any bleed can be serious, the severity and frequency depends on the baseline level of factor VIII protein present. In patients with severe disease, excessive bleeding can occur without a triggering injury. Untreated serious bleeds can lead to long-term irreversible damage or even death.

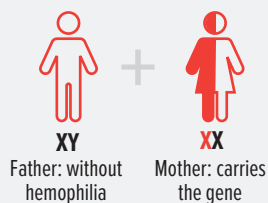


JOINTS

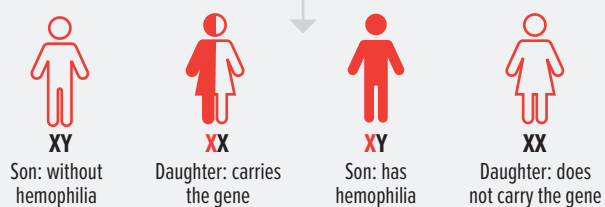


MUSCLES

GENES/GENDER



THE *F8* GENE IS AN X-LINKED RECESSIVE GENE SO THE SYMPTOMS OF HEMOPHILIA A OCCUR MOSTLY IN MALES.



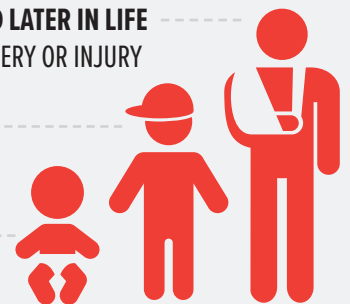
AGE OF ONSET/DIAGNOSIS

Hemophilia A severity has traditionally been defined based on the residual amount of FVIII in the blood.
 > 5-40% = MILD | 1-5% = MODERATE | < 1% = SEVERE

MILD DISEASE DIAGNOSED LATER IN LIFE
 OFTEN AFTER TRAUMA, SURGERY OR INJURY

MODERATE DISEASE
 DIAGNOSED BY AGE 5

SEVERE DISEASE
 DIAGNOSED EARLY IN LIFE

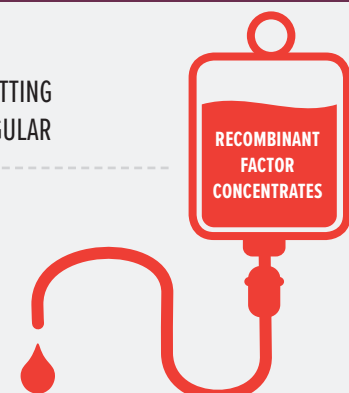


HEMOPHILIA A IN HISTORY

- 1803** A PHILADELPHIA DOCTOR IDENTIFIES AN INHERITED BLEEDING DISORDER PRIMARILY AFFECTING MEN
- 1947** FIRST LABORATORY DISTINCTION BETWEEN HEMOPHILIA A AND B
- 1984** THE *F8* GENE WAS FIRST CLONED

CURRENT STANDARD OF CARE

REPLACEMENT OF CLOTTING FACTOR THROUGH REGULAR INFUSIONS.



INHIBITORS: COMPLICATIONS WITH CURRENT STANDARD OF CARE

Some people with Hemophilia A develop inhibitors, proteins in the blood that inactivate infused clotting factor, so bleeding episodes continue even with treatment.

~33% OF PEOPLE WITH SEVERE HEMOPHILIA A DEVELOP INHIBITORS

STAY INFORMED. STAY CONNECTED.

FOR INFORMATION ABOUT GENE THERAPY RESEARCH FOR HEMOPHILIA
 Check out www.hemophiliaforward.com

**HEMOPHILIA
FORWARD.**