UNDERSTANDING HEMOPHILIA A

Hemophilia A is typically an X-linked, inherited bleeding disorder caused by an insufficient or nonfunctioning 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 4,000–10,000
HEMOPHILIA A PRESENT IN MALE BIRTHS

80-85% OF TOTAL HEMOPHILIA POPULATION

GLOBAL POPULATION LIVING WITH HEMOPHILIA A

NUMBERS ARE BASED ON A META-ANALYSIS OF NATIONAL REGISTRIES

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

XY Father: without hemophilia
XX Mother: carries the gene

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

XY Son: without hemophilia
XX Daughter: carries the gene
XY Son: has hemophilia
XX Daughter: does not carry the gene

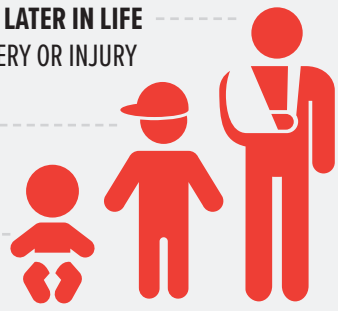
AGE OF ONSET/DIAGNOSIS

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

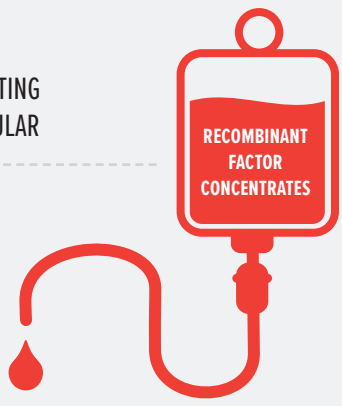
1803 A PHILADELPHIA DOCTOR IDENTIFIES AN INHERITED BLEEDING DISORDER PRIMARILY AFFECTING MEN

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**

ADVANCE YOUR KNOWLEDGE ABOUT GENE THERAPY. BE INFORMED. FEEL EMPOWERED.

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

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