

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



+



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

Father: without hemophilia

Mother: carries the gene



XY

Son: without hemophilia



XX

Daughter: carries the gene



XY

Son: has hemophilia



XX

Daughter: does not carry the gene

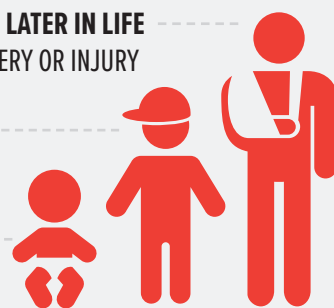
AGE OF ONSET/DIAGNOSIS

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

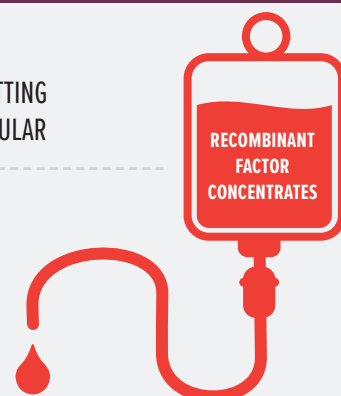
FIRST LABORATORY DISTINCTION BETWEEN HEMOPHILIA A AND B

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

HEMOPHILIA
FORWARD.