Talking Points - General

*The Hemophilia Council is the legislative advocacy arm of the four hemophilia foundations in California. We work to ensure access to care and advance the quality of life of about 400,000 Californians with hemophilia and other bleeding disorders by helping to shape state policy and educating legislators and other stakeholders about their special needs.*

Bleeding Disorders Sample Description

People with bleeding disorders like hemophilia and von Willebrand disease are missing particular clotting factors in their blood. That makes it much harder or even impossible for their blood to clot. Currently there is no cure, so people with bleeding disorders primarily rely on intravenous infusions of replacement clotting factor to stop or prevent internal or external bleeding.

Hemophilia 101 (3 Minute Speech)

Bleeding disorders, such as hemophilia, are genetic conditions, usually inherited. However, about 30% of new hemophilia cases arise from spontaneous mutations. Hemophilia is a chronic condition. It is life long and persistent. If you are born with a bleeding disorder there will never be a day in your life when you don’t have it. There is no way to prevent a bleeding disorder and there is no cure. But bleeding disorders are manageable through proper care with trained providers at hemophilia treatment centers (HTCs). Therefore, a hemophilia patient’s access to health care is essential.

Bleeding disorders are rare. At any given time, there about 20,000 people in the U.S. with hemophilia. Hemophilia is more common in men because it is an inherited sex-linked condition. However, women carriers of the defective gene also show symptoms. von Willebrand (vWD) disease is more common and affects men and women equally, but it often goes undiagnosed. vWD affects an estimated one million people.

If you have hemophilia it means your body doesn’t produce enough of one of the proteins required for your blood to clot. For hemophilia A, the most common form of the
disease, the body doesn’t produce enough clotting factor VIII. The only treatment for the disease is replacement of the missing protein, e.g. factor VIII. Regular, preventative clotting factor therapy, also known as prophylaxis, is necessary to prevent life threatening internal bleeding that may occur in muscles, joints, organs, and the head. Patients with milder forms of the disease typically treat only when needed, i.e. when they’re having a bleed.

Clotting factor is a biologic drug derived from donated human plasma or manufactured in a laboratory through genetic recombination technology. There are no generic equivalents. Factor requires specialized storage and handling and is dispensed for home infusion through an HTC 340B program or specialty pharmacies. It is essential that a patient receive the factor therapy that is prescribed by their hematologist; a patient’s response and tolerance to a specific factor therapy is unique.

People with hemophilia are some of the most expensive in the health care system. Clotting factor is prescribed according to body weight, i.e. the heavier or taller you are the more factor you need. The average annual cost of medication for a person with severe hemophilia is approximately $250,000 - $300,000. For a patient with an inhibitor, which is an immune response to the replacement factor, the annual cost is usually over $1 million. Hence, health insurance for hemophilia patients is essential.

Most hemophilia patients receive their care at an HTC. There are about 140 federally designated HTCs in the U.S. HTCs provide comprehensive, integrated, coordinated care via specially trained multi-disciplinary teams that that include hematologists, pediatricians, nurses, social workers, physical therapists, orthopedists, and dentists, among others.

**Helpful information for your reference:** This is what is in California statute about bleeding disorders.

125286.15. The Legislature hereby finds and declares all of the following:
(a) **Hemophilia** is a rare, hereditary, bleeding disorder affecting at least 4,000 persons in California and is a chronic, lifelong, and incurable, but treatable, disease.
(b) **Von Willebrand disease** is a human bleeding disorder caused by a hereditary deficiency or abnormality of the von Willebrand factor in human blood, which is a protein that helps clot blood. Von Willebrand disease is a chronic, lifelong, incurable, but treatable, disease affecting at least 360,000 Californians.