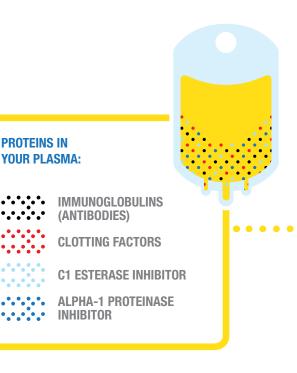


# WHAT RARE DISEASES ARE TREATED BY PLASMA PROTEIN THERAPIES?

Plasma is the straw-colored liquid portion of blood. It contains hundreds of proteins which carry out critical functions in the human body, such as antibodies to fight diseases and clotting factors to regulate bleeding. If a person has insufficient levels of any one plasma protein, his or her body cannot carry out these vital functions, causing a variety of chronic and life-threatening medical conditions. Plasma protein therapies are unique biologic medicines that treat plasma protein deficiencies by replacing a person's missing or functionally damaged proteins.



## RARE DISEASES TREATED BY PLASMA PROTEIN THERAPIES

#### PRIMARY IMMUNODEFICIENCY DISEASES (PID)

- » Caused by missing immunoglobulins (antibodies)
- » Antibodies control the immune system and prevent illness
- » Patients are chronically ill from severe, persistent, recurrent infections

#### CHRONIC INFLAMMATORY DEMYELINATING POLYNEUROPATHY (CIDP)

- » Cause not certain; immune system attacks nerve coating
- » Messages from the brain aren't delivered to the body if nerve coating is damaged
- » Patients experience progressive weakness, loss of limb function, and disability

## **BLEEDING DISORDERS (E.G., HEMOPHILIA)**

- » Caused by missing clotting factor protein
- » These disorders are characterized by recurrent bleeding episodes, primarily into the joints and muscles. Over time, repeated joint bleeding damages the cartilage and bone, which ultimately leads to crippling joint disease.

## **HEREDITARY ANGIOEDEMA**

- » Caused by missing C1 esterase inhibitor protein (C1-INH)
- » C1-INH helps regulate inflammation
- » Patients have edema (severe swelling) which can be fatal if airway is obstructed

#### **ALPHA-1 ANTITRYPSIN DEFICIENCY**

- » Caused by missing alpha-1 proteinase inhibitor
- » Alpha-1 proteinase inhibitor protects the lungs
- » Patients have chronic emphysema and liver damage