POLICY PRIORITY:

Advocate for a safe blood supply, and work to ensure bleeding disorder therapies are safe and effective (whether plasma-derived, recombinant, or other).

Mindful always of our community's history, HFA has pledged to remain vigilant and engaged in matters of blood and product safety.

BACKGROUND:

In the 1980s, blood borne pathogens contaminated the clotting factor products that were used by the U.S. bleeding disorders community. Approximately 90% of people with severe hemophilia were infected with HIV; an even high number contracted hepatitis C (HCV.) Many thousands died from these infections, and families were rocked by the loss of loved ones. HFA, founded in response to this human tragedy, committed itself from its first days to the cause of blood and product safety.

KEY PRINCIPLES:

1. Patient safety is paramount. Patient organizations, health care provides, government, and industry (blood and plasma organizations, pharmaceutical/biotech, specialty pharmacies) should place patient safety at the center of product development, manufacturing, distribution, and post-market surveillance.
2. Blood and product safety policy must be based on rigorous science.
3. The federal government bears responsibility for maintaining and enforcing a strong and well-resourced regulatory framework, but all stakeholders play a role in ensuring product safety.
4. The patient perspective must be included at every stage of the plasma product/drug lifecycle. Patients are entitled to clear disclosures and reliable information allowing them to assess the risk-benefit profiles of the products they use or may use.

WHAT HFA IS DOING TO CHAMPION BLOOD & PRODUCT SAFETY:

Blood safety. HFA monitors the work of the FDA's Blood Products Advisory Committee and HHS's Advisory Committee on Blood and Tissue Safety and Availability. HFA also participates in APLUS (American Plasma Users) Coalition, a group of national patient organizations representing 125,000 individuals with rare diseases who use life-saving plasma protein therapies. APLUS gives voice to the patient perspective in matters relating to plasma collection and the manufacture of plasma protein therapies.

Product safety. An unsettling spate of product recalls and safety notifications prompted HFA and NHF to organize a Product Safety Summit in January 2020. The Safety Summit brought together stakeholders from across the bleeding disorders landscape to discuss monitoring, educating, and communicating issues around bleeding disorders product safety. HFA continues to work with NHF to publish follow-up materials and otherwise disseminate the findings of the Safety Summit. Both HFA and NHF recognize the need to address: safety gaps that exist in the present; standards that will serve the community as we enter a new era of gene therapy and other novel treatments; and the community's interest in timely, accurate, and consistent education and safety communications.

WHAT CAN YOU DO?

- Educate yourself about:
  - What is an adverse event and other concerns to share with your health care provider.
  - Basics of clinical trials, informed consent, and drug safety reporting,
  - The importance of broad-based registries that collect information and safety signal monitoring for existing and novel therapies, and
  - Current products, including what you use and products in development.
- **Always report issues to your medical provider.**
- Keep medication logs.
- Register with the Patient Notification System (PNS). PNS is a **fast, free,** and **confidential** program that alerts patients with hemophilia, von Willebrand Disease, and other bleeding disorders of a withdrawal or recall of therapy products. All major plasma-derived and recombinant analog therapy manufacturers participate in this system.

Resources:

- The Patient Voice: Product Safety Issues
- Patient Notification System
- Plasma Protein Therapeutics Association (PPTA) – Programs and Standards
- Committee of Ten Thousand (COTT)
- Food & Drug Administration (FDA) – Blood and Blood Products
- Food & Drug Administration (FDA) – Drug Safety
- MedWatch: The FDA Safety Information and Adverse Event Reporting System
- Department of Health and Human Services – Advisory Committee on Blood and Tissue Safety and Availability (ACBTSA)
- CDC – Hemophilia Blood Safety
- National Library of Medicine – Drug Safety
- American Association of Blood Banks (AABB)
- Healthy People 2020 – Blood Disorders and Blood Safety
- European Medicines Authority