

PPTA Statement on MSM Donor Policy

FDA Blood Products Advisory Committee Meeting December 2, 2014

The Plasma Protein Therapeutics Association (PPTA) is the international trade association and standards-setting organization for the world's major producers of plasma-derived and recombinant analog therapies, collectively referred to as plasma protein therapies. Plasma protein therapies are used in the treatment of a number of rare diseases. These diseases are often genetic, chronic, life threatening conditions that require patients to receive regular infusions or injections of plasma protein therapies for the duration of their lives. These therapies include clotting therapies for individuals with bleeding disorders, immunoglobulins to treat a complex of diseases in persons with immune deficiencies, therapies for individuals who have alpha-1 anti-trypsin deficiency which typically manifests as adult onset emphysema and substantially limits life expectancy, and albumin which is used in emergency room settings to treat individuals with shock, trauma, burns, and other conditions.

PPTA supports the use of science-based decision making in determining whether there should be changes in U.S. Food and Drug and Administration (FDA) blood and plasma donor eligibility criteria for men who have had sex with men (MSM). PPTA applauds the U.S. Department of Health and Human Services (HHS) for undertaking studies that provided data for consideration. PPTA respects the recent recommendation from the HHS Advisory Committee on Blood and Tissue Safety and Availability to reduce the lifetime deferral to 12 months for MSM providing any change in policy is accompanied by a robust monitoring system to evaluate the impact of such a change.

PPTA member companies are committed to providing safe and effective therapies. Patient populations who receive the therapies made from plasma have chronic and serious conditions. Donor selection is one of the several layers of safety in the manufacturing of plasma protein therapies, and includes state of the art testing of individual plasma donations and manufacturing pools, followed by robust manufacturing processes with dedicated safety steps. Plasma protein therapies are a distinct class of therapeutic products which undergo significant viral inactivation and product purification processes that provide plasma protein therapies with significant virus safety margins. Companies have made substantial investments in all of these areas and in over two decades there have been no documented transmissions of HIV or hepatitis B or C.

PPTA's Voluntary Standards Program for collectors of Source Plasma (IQPP) and manufacturers of plasma protein therapies (QSEAL) contributes to safety of Source Plasma and plasma protein therapies. These Standards include, among others, provisions for testing donations and manufacturing pools with both serology and nucleic acid amplification testing. Donations from one-time Source Plasma donors (Qualified Donor Standard) are not used in manufacturing. Donations undergo a 60-day inventory

hold before being pooled for manufacturing. PPTA members emphasize the importance of collecting plasma from a low-risk donor population, and one of the standards is the viral marker standard (VMS), which sets limits on the number of positive qualified donations for each collection center.

PPTA member companies operate in a global environment. As such, companies must adhere to often divergent regulatory requirements. For MSM these requirements vary from continued lifetime deferral, 5-year deferral, 1-year deferral, to no specific MSM policies. As noted, PPTA supports the studies undertaken by the U.S. government as the results of these studies are valuable to help inform decision makers around the world.