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# Evolution in Hemophilia Therapy

BY BRENNNA RAINES, PPTA SENIOR MANAGER, HEALTH POLICY  
SARA STEFANELLI, PPTA COMMUNICATIONS ASSISTANT, EUROPE

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The following is a summary of remarks delivered by Glenn F. Pierce, M.D., Ph.D. at the 2017 Plasma Protein Forum in Washington, D.C.

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Over the past 50 years, many technological changes have occurred within the hemophilia community that had a strong impact on patients and their care.

An unequivocal sign of hemophilia is a bent leg, resulting from repeated joint bleeds. This evidence is apparent in a 100-year-old photo of Prince Alexei from Russia (Figure 1), as well as in a 50-year-old photo of Dr. Glenn F. Pierce as a child (Figure 2). Unfortunately, there are still some areas of the developing world with no access to current therapies, and patients today suffer from the same consequences of uncontrolled bleeding episodes as they did 100 years ago.

The modern evolution of hemophilia treatment began with the life-changing discovery of cryoprecipitate in 1964. A

few years later, the plasma fractionation industry developed concentrates of clotting Factors VIII and IX, which were easier to use than cryoprecipitate or fresh-frozen plasma. The smaller volumes of concentrate needed to treat hemophilia was a major change for patients in terms of ease of administration. In the 1980s, researchers successfully cloned the Factor VIII gene, allowing the revolutionary production of recombinant therapies. Recent developments in hemophilia treatment include bioengineered products with extended half-lives, and gene therapies are currently being clinically tested.

All of these treatment advances profoundly affected the care and outcome of individuals with hemophilia. Prior to the availability of treatment, many individuals spent much of

their lives hospitalized for bleeding episodes, dependent on casts, crutches, or wheelchairs. The introduction of clotting factor concentrates allowed patients to move from receiving treatment in the hospital to treating bleeding episodes on-demand at home. Patients who once experienced uncontrolled, excruciatingly painful bleeds could now instantaneously stop the episode by simply infusing a clotting factor concentrate. This shift was life-changing for patients, who could for the first time think of their future lives. Patients no longer needed to stay in hospitals and could pursue their education and careers as other healthy adults.

In the summer of 1982, the first three cases of Acquired Immunodeficiency Syndrome (AIDS) were identified in the hemophilia community. It was a time of incredible fear and unanswered questions among patients. Many patients began to advocate for a safer blood supply and worked with the predecessor of the Plasma Protein Therapeutics Association or PPTA (the International Plasma Products Industry Association or IPPIA) to develop improved donor procedures and manufacturing practices in response. These steps ushered in a new level of safety in products and would serve as the basis for PPTA's International Quality Plasma Program and Quality Standards of Excellence, Assurance and Leadership program today.

The following decade saw a number of products withdrawn from the market due to the emergence of a new pathogen (Creutzfeldt-Jakob disease and its variant version) as well as manufacturing issues. It was a hectic and chaotic time, and many patients were faced with a shortage of product.

Consequently, the National Hemophilia Foundation worked collaboratively with the IPPIA to monitor the situation. PPTA later created a data program to provide a reliable, nationwide assessment of the availability of clotting factors and other lifesaving therapies in the United States in response to drug shortage concerns.

Once recombinant factors entered the market, prophylaxis became the standard of care for hemophilia in children and eventually adults. However research has found that prophylactic treatment can still lead to joint damage. As early as the 1990s patients began to advocate for alternative treatments and ultimately, a cure. The National Hemophilia Foundation pushed for the National Institutes of Health to organize a meeting that brought together scientists and clinicians in the hemophilia community. The first meeting was held in 1992 and encouraged interaction among groups of individuals who normally didn't work together. This collaboration eventually led to the preliminary clinical trials for gene therapy in hemophilia; follow-on trials are currently ongoing as the community progresses toward a cure.

Today, the burden of therapy remains high in hemophilia patients. Despite technological advances and although a cure may be at hand, the majority of the world still does not have access to care or treatment at all. The important work of the World Federation of Hemophilia Humanitarian Aid Program is crucial to closing the gap between the United States and countries with no access to care. For more information on its work, please visit <https://www.wfh.org/en/humanitarian-aid-program>. ●



Figure 1



Figure 2