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Letter to the Editor

Memories of the past, experience for the future

It is undoubted that the World of hemophilia has changed extraordinarily if I compare the times when I joined the WFH as a volunteer in 2000 compared to today, where a landscape of advances that are happening and that will come in the next 3 years at least. Back then we did not even dare to imaging a vision like the current "Treatment for All". There were very terrible and dramatic moments due to contaminations with HIV and Hepatitis C. There were moments where the news that arrived was that colleagues and friends in the world had died. Times when distrust of the pharmaceutical industry was at its worst. Moments in which some patients, aspiring to receive some financial compensation for the damage suffered from treatment with concentrates that turned out to be contaminated, were forced to sue the doctors treating them since childhood, to support the case in court. I do not miss those moments at all, but I do recognize that it was an episode that had years where established national member organizations (NMOs) and emerging NMOs spoke the same language to society. It did not matter whether it was developed or developing. All argued facing society, facing the health authorities, facing the rulers, facing press, facing the pharmaceutical industry, facing decision makers with the same and common argument "No person with hemophilia or another bleeding disorder should be treated with cryoprecipitate or plasma". All the caregivers and leaders together with the patients monitored and demanded that the new coagulation concentrates must have undergone at least 2 viral inactivation processes, know the origin of the plasma, that they be safe and of high purity in some cases. It was a coordinated fight around the world that in many places elicited reactions from everyone involved in this issue.

What is the current scenario? We have a vision with more than 10 years of establishing "Treatment for all ..." and more recently the comment was added "one day all people with a bleeding disorder will lead a full and productive life".

On the other hand, we have realities that differ greatly from those of 20 years ago:

- The growth in age of the population
- New technologies / innovations
- What patients and the public expect.

Each of these aspects are challenges that patients and their families, health care professionals, and especially the leaders of national member organizations (NMOs) must face.

The leaders of the organizations have to support health care givers in redesigning their comprehensive treatment strategy for a person with hemophilia (PWH) considering that their life expectancy implies being able to count on the support of a multidisciplinary team with a cardiologist, urologist and even a geriatrician. In most cases and according to the initiative of the WFH, it should also include in its activities and requests to the authorities to considering the diagnosis and treatment of women and girls with bleeding disorders as well as the access to appropriate medications.

The range of therapeutic options and various drugs has an increasing role in modifying the strategy by the leaders of the NMOs together with the care providers to face health authorities. Health leaders must handle carefully the arguments between standard half-life, extended half-life, treatment with NO FACTOR concentrates, new molecules where each one has its cost benefit and cost effectiveness, and also considering we are also entering an era of the functional cure of hemophilia that is obtained with gene therapy.

It is useless still requiring governments to approve that PWH must consume at least one IU when in fact they need to be under prophylaxis, which is the Gold Standard of treatment for hemophilia in both children and adults, consisting of at least 4 IUs. The indicators at present are more suitable for the annual bleeding rate (ABR). This new indicator shows in the end what the patient needs is the possibility not to bleed or to bleed much less and therefore to avoid joint problems.

Decision makers today expect scientific evidence to be shown to support requests from patients and NMOs. The use and management of DATA is essential to convince government authorities.

Last but not least and very challenging, patients ask for what they think is best, what they hear, or think will improve their lives. Patients must not be expected to make decision, since they are receiving confusing information from different sources, such as social networks, posts, publications from different institutions and companies.

The leaders of the NMOs carried out the complicated task of listening to their followers, that is, the patients, primarily and outlining the realities and possibilities in their respective countries and later explaining those needs to the authorities, but not with the usual past requests but rather by using the opposite strategy as a proposal to the decision makers, arguing in a professional way these needs and demonstrating that the authorities can save money if they treat patients as they should.

Leaders are forced to break years of habit, break paradigms and styles and to innovate and argue basing their statements on statistics and health technology assessments.

Hopefully, the whole community, will have the same drive and make the common effort as was done some 20 years ago when everyone fought for everyone else and with a common reasoning and this is that no one should be left behind, that treatment for everyone will finally become a reality.

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