Clinical and organisational aspects of haemophilia care: the patients' view

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Dear Sir,

Over the last years, the life expectancy of patients with haemophilia has become ever closer to that of the general population and the patients' quality of life has improved dramatically[1]. These advances have been achieved thanks to a continuous improvement in factor VIII product availability in most countries, which has allowed the use of coagulation factor replacement therapy on a larger scale, particularly in western countries.

In the early 1980s, viral infections had a dramatic impact on the haemophilia community. The improvement of methods for inactivating or removing viruses from plasma products, together with a concomitant application of stringent donor selection criteria and nucleid acid amplification testing of donations have increased the safety profile of plasma-derived products. As a result, no cases of blood-borne transmission of known viruses from these products have been observed in the last two decades.

In the late 1980s, on the other hand, the advent of recombinant technology offered the possibility of an innovative change in patients' treatment which contributed to restoring patients' trust in replacement therapy and compliance, which had been severely compromised by the blood-borne virus epidemics. In fact, the manufacturing process of recombinant factor VIII products evolved quickly, with the goal of eliminating the risk of pathogen transmission. This goal has been pursued through the improvement of protein purification techniques and viral inactivation steps and progressive elimination of human and animal proteins from the various phases of production.

We, on behalf of our community of haemophiliacs, are aware that there are still old problems to be solved as well as new challenges to be dealt with, such as cardiovascular diseases, malignancies and other age-related co-morbidities. The unresolved problems include major clinical issues such as inhibitor development and how to eradicate such inhibitors. The ongoing scientific debate on these issues is lively[2,3]. As far as concerns inhibitors, unless undisputed evidence from prospective clinical trials shows a real difference in clinical practice, it cannot be claimed that plasma-derived factor VIII products are superior to recombinant factor VIII products[4]. Even their equivalence should be scientifically demonstrated by controlled clinical trials.

Continuity of care, intended as maintenance of treatment with the product that offers the best clinical benefit to each patient, is essentially the only indisputable therapeutic approach for the community of haemophiliacs. However, in times of global recession, when financial constrictions are jeopardising the current levels of health care, governments and health-care decision-makers may be tempted to turn the clock back and, in the name of cost-saving, to adopt policies that might compromise access to the best available therapies and put the continuity of care in haemophilia management at serious risk.

In such a context, small changes in clinical guidelines, for example, those concerning the choice of treatment for previously untreated patients, if supported only by low-level evidence, such as that from uncontrolled and/or retrospective studies, could have a knock-on effect putting well-established landmarks in patients' treatment at risk. Therefore, as patients' representatives, with the endorsement of the World Federation of Hemophilia, we have many expectations on the potential of the Study on Inhibitors in Plasma-Products Exposed Toddlers (SIPPET) to give the final word on inhibitor development rate in previously untreated patients managed with recombinant or plasma-derived products.

In conclusion, we strongly believe that patient empowerment and engagement, according to the endorsement given by the World Federation of Hemophilia, involves all aspects of haemophilia care, including clinical and organisational aspects and programmes. Patients' representatives, together with all the other stakeholders, should be involved in

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the discussion of clinical organisational guidelines and health care programmes, as stated by the fourth principle of the European Principles of Haemophilia Care[5]. In this context, the Italian Federation of Haemophilia Associations (FedEmo) has promoted the definition of an accreditation scheme for haemophilia treatment centres and haemophilia programme guidelines by Italian Regions based on the European Principles of Haemophilia Care.

References

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