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Herrn Minister Jens Spahn
Bundesminister für Gesundheit
Bundesministerium für Gesundheit
11055 Berlin
Deutschland

Brussels, 5th December 2018

GSAV: Draft law endangers the quality of haemophilia treatment in Germany

Dear Minister Spahn,

We are writing to you on behalf of the European Haemophilia Consortium (EHC), an international umbrella organisation representing 46 patient groups in Europe active in the area of haemophilia and other rare bleeding disorders. The Deutsche Hämophiliegesellschaft (DHG) is our German national member. They have informed us of their concerns with regard to a proposed legislation, GSAV, which they fear would endanger both patients' safety as well as the quality of haemophilia treatment in Germany. Respectfully, the EHC agrees with the DHG's concerns and would like to outline why:

Our concerns in brief
The EHC and the DHG fear that changing the law and allowing non-specialised clinicians to prescribe haemophilia medicines may endanger patients' lives, reduce national clinical expertise and waste precious resources. As a rare disease, haemophilia is a highly specialised field of medicine, which requires continuous training, education and communication. This is especially true at this time when novel and innovative therapies are entering the European market.

Consensus on best practices in rare diseases
As a rare disease, haemophilia requires specialised treatment and medical expertise. It is both a medical as well as European health policy consensus that rare disease patients benefit most when they receive such treatment and expertise in Centres of Expertise (such as haemophilia comprehensive care centres or CCCs). Centralizing this expertise benefits patients, clinicians and the broader health care system. Currently, the German centres are amongst the global leaders in this field.

Timing: managing the introduction of innovation
The proposed GSAV arrives at a time of great scientific innovation in haemophilia. Multiple and very different types of novel therapies are beginning to arrive. The first of these is Emicizumab, or Hemlibra, and other therapies with different modes of action will soon follow. These bring great promise of a significant and meaningful impact on the clinical care and quality of life of patients, and the EHC fully welcomes them. However, due to their completely different mechanisms of action in the body, compared to the well-known and well-understood standard factor replacement therapies, it will be critically important for both patients and clinicians to be (and to remain) well educated on how to manage these new therapies. This is because, although novel medicines comply with European regulations and will have proven their safety and efficacy in a controlled clinical trial environment, they will not yet have

demonstrated long-term effects in the real world. In fact, by using these medicines on 'real world' patients we may still discover unknown side effects and well as learn how these medicines 'behave' in case of surgery or trauma. This is why the EHC, together with European clinicians through the European Association for Haemophilia and Allied Disorders (EAHAD), recently issued a joint position statement on 'Promoting the central role of haemophilia comprehensive care centres in the treatment of haemophilia and rare bleeding disorders using novel non-replacement therapies'[1]. In particular, this position states that these novel therapies should be *exclusively* prescribed, managed and monitored by CCCs to ensure their optimal introduction and safe use while their completely different and complex modes of action, compared to traditional replacement therapies, are introduced in real-world settings.

Centralizing expertise: benefits to patients and clinicians

Centralizing treatment and care, through CCCs for example, ensures that expert health care professionals retain and grow their expertise through seeing patients regularly. This in turn ensures that patients are treated according to the latest treatment protocols and medical and scientific advances. In turn, the large number of patients seen in these centres facilitates the important task of data collection. As you know, good data collection is crucial for the continued study and improvement of treatment protocols of small patient populations, but also for safety monitoring and for the evaluation of outcomes on a national basis. Data collection is also an invaluable tool to plan and manage resources for the treatment of this patient population. By allowing non-specialists to prescribe these treatment products, there will certainly be a decrease in data reporting in the national registry and this will be ultimately to the detriment of patients' quality of treatment and quality of life, as expert clinicians will no longer be able to get a comprehensive overview of the patient population in Germany.

Centralizing expertise: benefits to the health care system

The health care system itself also benefits from centralization of treatment and care. It does so not just due to the growth of medical expertise, but also because treating patients in a CCC ensures that precious and limited resources are maximised and waste avoided. This is achieved in part by the ability of these centres to negotiate long-term and high-volumes contracts for the purchase of haemophilia treatments. Moreover, it is widely acknowledged that treating haemophilia patients in a CCC will minimise medical complications, due the aforementioned expertise, and reduce the need for costly hospitalisations. Another aspect to take into consideration is that often CCCs liaise with other hospital-based services to provide care for patients with haemophilia during surgical procedures. This often results in CCCs taking treatment from their own supplies. With the proposed change in legislation, CCCs would no longer need to be notified of any procedures occurring in people with haemophilia, which would put these patients at an unnecessary risk of bleeding and complications. Furthermore, the treating hospitals might waste resources as they would need to purchase the amount of treatment required for the procedure and undoubtedly access it with additional pharmacy charges as well as additional costs for small volumes with short-term, unplanned factor concentrates. On the other hand, if the treatment comes directly from the haemophilia Centre of Expertise, this often comes from high-volume and longer-term-agreed contracts, and no additional charges are incurred by the health care system.

Conclusion

Both the DHG and the EHC are deeply concerned that changing the law, and as a consequence the current structure and organisation of haemophilia care in Germany, would completely contravene the aforementioned best practices. Instead, and as we understand it, this proposed new law would allow non-specialist clinicians to prescribe these complex new medicines and this may impact on patients' morbidity and mortality in potentially harmful ways that could have been avoided. Furthermore, under the proposed new law, patients would visit CCCs less, leading to reduced clinical supervision, safety monitoring and maintenance of practical and hands-on clinical expertise in Centres of Expertise.

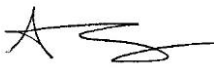
[1] https://www.ehc.eu/ehc-and-eahad-release-joint-statement-on-novel-non-replacement-therapies/

For all of the above reasons, while this proposed new law may appear practical and offer many benefits, the EHC joins its voice to that of the DHG in signalling our multiple and significant concerns. We believe that more needs to be discussed and understood about the potential ramifications and consequences of this proposed new law before proceeding with it.

To this end, we respectfully urge you to establish a working group that would include both the DHG and key haemophilia medical experts, and to proceed in your decision-making in such a manner that may guarantee that patients' lives, health care providers' critical expertise and the excellent standards-to-date of the German haemophilia system can continue to be protected and safeguarded into the future.

We thank you for your time and consideration of these concerns and requests, and remain,

Sincerely yours,



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