Addressing Market Access Obstacles in Relation to Rare Bleeding Disorders

By Stefan Bosbach on Oct 6, 2017

Stefan Bosbach from phamax scrutinises the situation for people with rare bleeding disorders, including the challenges in recording the overall burden of the disease and the severity of the condition, which are hampering access to the right treatments.

Often, a disease is simply regarded a statistic, or set of statistics, until it affects us, or someone we care about. For a long time, this attitude of ignoring less prevalent diseases has been the bane of rare or orphan diseases, it is time to increase awareness in a structured manner and overcome market access hurdles to provide the right care to the right patients.

There are 7,000 diseases worldwide listed as rare. Rare diseases affect 6-8% of the global population, or an astounding 350 million. Bleeding disorders form the largest group among these. The following table demonstrates the reported cases of various rare bleeding disorders from 82 countries globally:

Table 1. *Reported cases of rare bleeding disorders (RBDs) from 82 countries globally. Source: World Federation of Haemophilia report on the Annual Global Survey 2015.*

Bleeding disorder	Impacted population
Haemophilia A	151,159
vWD	74,318
Haemophilia B	30,310
Factor VII	9,330

Factor XI	6,866
Factor V	2,122
Factor X	1,799
Factor I	1,777
Factor XII	1,485
Factor V + VIII	547
Factor II	278

These numbers are obviously a gross under-representation of the burden of the disease. These are self-reported numbers from countries and do not account for the vast number of patients who are either under-diagnosed or undiagnosed entirely.

Let us consider the example of congenital Factor XIII (FXIII) deficiency. FXIII deficiency occurs in approximately one in every three to five million people globally. The disease is considered to be the rarest of the bleeding disorders, with a little over 300 cases reported worldwide. Although rare, FXIII deficiency is clinically important because the incidence of intracranial haemorrhage is 20–30% - much higher than of any other bleeding disorder. The diagnosis of FXIII deficiency continues to challenge clinicians globally and is often missed, leading to potentially fatal consequences.

At phamax, when it comes to rare bleeding disorders, we believe there are barriers to providing the right care to the right individual. We have categorised these obstacles as "The 5 As":

- **Awareness** – This indicates the lack of awareness among all stakeholders involved. This is the biggest market access challenge to address in the rare bleeding disorders arena. The lack of systems to capture information on patients leads to underreporting or, worse still, no reporting of the conditions. This further leads to policy-maker ignorance on what ails the population. In turn, that creates the lack of sufficient resourcing to tackle the burden.
- **Accessibility** – This represents limited patient access to specialised diagnostic and management facilities. Rare bleeding disorders need specialised settings for

management, and adept and qualified staff to handle the complications that arise. Patients not being diagnosed, or wrongly diagnosed in terms of severity of the condition, creates a major void in disease management.

- **Availability** – This involves the inconsistent availability of relevant medications and protocols of management of these diseases, or the care paradigm to avoid complications.
- **Affordability** – The availability of the right treatment options at an affordable cost continues to be a hindrance. Researchers need to equip advocacy groups and healthcare administrators with relevant data to engage healthcare industry partners to make treatment options affordable.
- **Adherence** – This relates to the mechanisms and guidelines that are put in place to ensure that the patient receives the right medication, in the prescribed dose, and as per schedule.

These 5 As, or barriers to market access, are more prevalent in emerging economies. A look at the World Federation of Hemophilia Report on the Annual Global Survey 2014 shows a clear void in the reporting of cases. Only 82 countries reported numbers and many drew a blank for most bleeding disorders, apart from Haemophilia A, Haemophilia B and von Willebrand Disease (vWD). It should also be noted that the average usage of replacement factors to manage the diseases are lower in the emerging economies than in developed countries. Issues with misdiagnosis of the severity of the disease, which leads to suboptimal management with incorrect dosing, plays into the natural course of the disease. This leads to further complications in the management of rare bleeding disorders.

However, all is not lost. Policy-maker attention is being drawn towards these rare bleeding disorders, and the efforts of rare/orphan disease organisations, patient advocacy groups, policies and guidelines are now being put in place to manage these conditions. Clinical and research societies are now able to generate enough noise to capture the attention of decision makers. The recently concluded Congress of the International Society on Thrombosis and Haemostasis (ISTH) in Berlin was one of the largest so far, attracting close to 10,000 participants and showcasing around 3,000 abstracts and posters.

Research in the area is also moving towards more effective and less frequent interventions, such as monoclonal antibodies and gene therapy. As we close in on the end of another decade in the history of rare bleeding disorders, and going by the results from the novel therapies, we are looking at a better management of rare bleeding disorders. The other cogs in the wheel simply need to align in place to make this a success.

Dr. Stefan Bosbach has almost two decades of pharmaceutical experience in senior management roles. He is a Senior Principal Consultant at phamax and heads RBDNow. RBDNow is a scientific communication platform that brings together all stakeholders for the development of structured initiatives in rare bleeding disorders. Dr. Stefan's therapeutic knowledge ranges from Haematology, Haemophilia (RBDs), Oncology over Neuroscience to Infectious Diseases.

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