Exploring serious market access roadblocks for rare bleeding disorders

Ensuring that sufferers get the treatment they require

Every single day, we are making medical advances that are taking the world by storm. New drugs are being created, procedures are being streamlined and robotics are being created to facilitate complex operations. Medically, we have taken great strides and, as each year goes by, new advances hit the headlines to demonstrate how far we have come. Unfortunately, however, there are still some areas that require much more attention, with certain diseases seemingly falling by the wayside.

One such area that requires a great deal more understanding and appreciation is that of rare bleeding disorders. Today, 7,000 diseases are listed as rare and 6-8% of the world's population is thought to be suffering from a rare disease, with rare bleeding disorders forming the largest group. In fact, as we will soon see, rare bleeding disorders pose a huge challenge to the healthcare sector and patient community. Due to certain roadblocks, it is increasingly clear that we are not providing sufferers of rare bleeding disorders with all the care and attention they need.

The key challenges for patients with rare bleeding disorders are awareness, cost of care, access to care, suboptimal treatment and, critically, a dearth of published data. A combination of these problems cause frequent problems for clinicians and result in market access concerns. Although efforts are now underway to develop and provide structured data at regional levels, the reality is that the data is not yet coordinated enough to answer unmet needs. This problem is particularly evident in emerging countries. We don't quite know the scope of the problem, but what is clear is that with conditions such as haemophilia, 75% of the patient population has little or no access to treatment. Even in some parts of Europe, like certain countries in the East and South, some individuals suffering from rare bleeding disorders are faced with a number of issues, including a lack of diagnosis and modern treatment, such as recombinant factors.

Below, we will cover the elements necessary to properly fight a disease, which obstacles are preventing patient access with regards to rare bleeding disorders, and how our biggest struggle in the healthcare sector is a lack of data and appropriate communication.

Fighting a disease

In order to fight a disease, an adequate action plan needs to be created. Healthcare policymakers and advocacy groups need to have consistent and structured patient data. On top of this, there is a need for established channels of communication between stakeholder groups. Such measures are critical when it comes to developing efficient and unified treatment guidelines.

'In emerging countries, 75% of the patient population with haemophilia has little or no access to treatment'

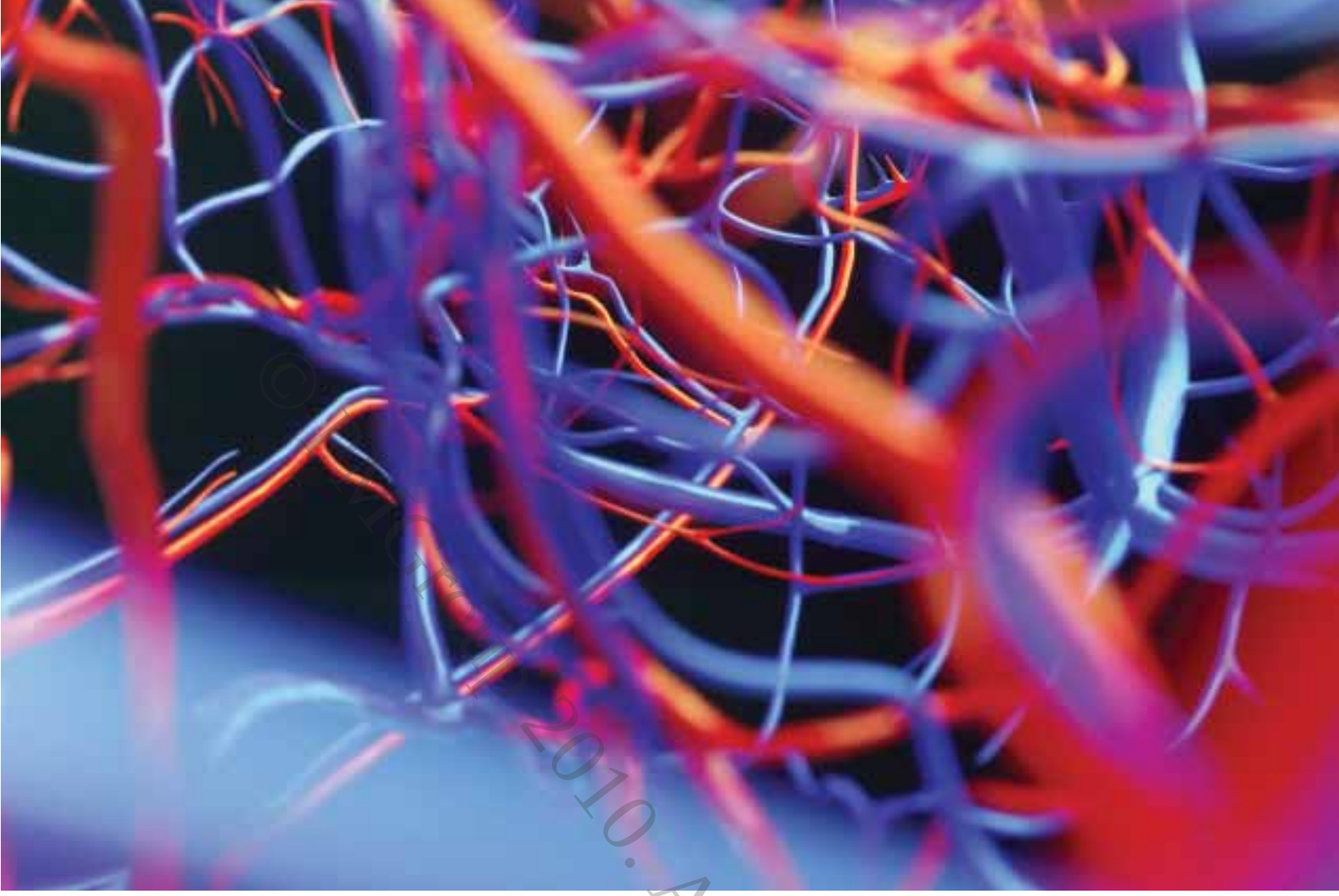
Structured data is so important to the pharmaceutical industry because if any deviation is observed, it means failures can be investigated, root causes identified and appropriate actions taken. Without such data, we're in the dark with regards to the safety, efficacy and quality of drugs, on top of the scope of a given disease.

A significant lack of data

Given the importance of data to the pharmaceutical industry, you would hope that we would have specific, structured data with regards to rare bleeding disorders. Unfortunately, this is not the case. In fact, there is a serious scarcity of data in this field and the data that is available is not organised or shared properly with stakeholders.

This is a well-known problem, and in the past there have been efforts to improve and enhance the understanding of rare bleeding disorders with regards to prevalence, diagnosis and treatment. In 2004, the Rare Bleeding Disorders Database (RBDD) was created in order to collect epidemiological information on 3,230 patients from 66 centres around the world. In addition, epidemiological data can be found from the World Federation of Haemophilia's annual survey. Unfortunately, the data is not homogenous and doesn't give an accurate reflection of the global distribution of rare bleeding disorders, as roughly 50% of the data only concerns European patients.

In fact, the true scope of patients affected by rare bleeding disorders is very limited, and this is particularly true of developing countries. This might be due to the limited number of reliable national registries. In less economically developed countries, political, economic and social situations can sometimes lead to patients not being diagnosed, which means their conditions are never managed.



Market access issues

Due to the fact that accurate data is such an issue for rare bleeding disorders, a number of market access issues arise that limit quality of life for hundreds of people around the world. Without appropriate data, we are unable to determine rates and severity of disease complications, engage stakeholders, identify unmet needs, develop systems, disseminate evidence, assess which health issues should be considered for further study and build awareness. Essentially, by collecting and managing vast amounts of data from a number of sources, pharmaceutical companies will be able to make more informed, strategic decisions.

In order to collect this data, we need to emphasise the importance of real-world data - a process which is useful in many phases of product development. Real-world research incorporates randomised controlled trials (RCTs) and 'more pragmatic research in real clinical practice'. Randomised controlled trials were once considered the gold standard among experimental methods, but due to their limitations, we now understand that more is needed. It has been said that real-world evidence is increasingly essential in order to ensure patient access and commercial success, as it is able to offer a bigger picture of the risks and benefits of a particular product, treatment or drug.

'Transparency of individual patient data is essential to personalise treatments and improve patient health'

One source points out how transparency of individual patient data is essential to personalise treatments and improve patient health, as it allows healthcare providers to monitor utilisation and forecast future needs. This same source suggests that part of the reason for this lack of data is that patients do not always track their own bleeds in a reliable and consistent way and, as such, an intuitive, easily accessible system should be provided to patients in order to easily report this data. This tracking system would provide transparent, visible data to all stakeholders, which would in turn 'facilitate benchmarking between centres and countries'. More complete data will also help pharmaceutical companies to justify drivers of treatment costs - a key element when it comes to ensuring market access.

Overcoming roadblocks

When it comes to overcoming market access roadblocks and improving quality of care for patients with rare bleeding disorders, we need to focus on collaboration and communication, through established, structured, scientific initiatives that engage clinical stakeholders.

In order to streamline communication, we need to assemble different groups of the health ecosystem (clinical leaders, patient organisations, developmental organisations, governments and healthcare industry partners) on a scientific communication and cooperation platform. Such a platform can then go on to generate evidence and insights to design, develop and deliver structured, scientific and sustainable initiatives, on a scale specific to disease areas.

It's critical for experts to convene, identify and formulate short-term and long-term activities to oversee their progress and provide course correction advice to manage rare bleeding disorders optimally. Such a network also facilitates the generation of relevant data on the issues, which can then be published in relevant scientific conferences or publications. The experts will be well-placed to participate in activities designed to aid patient access, as well as acting as advocates for rare bleeding disorder patients in their respective countries.

In time, with enough professional dedication and enthusiasm, we can work together to break down each and every roadblock that stands in the way of rare bleeding disorder sufferers getting the attention and care they deserve.

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