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Strengthening hemophilia registries globally: Strategies to improve patient outcomes

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Abstract:

Hemophilia is a genetic bleeding disorder characterized by a deficiency of clotting factor VIII or IX. Hemophilia registries are structured databases that maintain longitudinal data on people diagnosed with hemophilia. Hemophilia registries maintained at different levels serve multiple public health purposes, which can eventually improve the treatment outcomes and quality of life of the affected person. These registries can be maintained at the international level, national level, and at the regional/hospital level. Despite the immense utility of hemophilia registries in improving treatment outcomes, multiple challenges have been identified that could limit their scope and must be overcome. In conclusion, hemophilia patients can derive extensive benefits from the registries maintained at different levels. However, to optimize these benefits, we must adopt targeted, evidence-driven measures to tackle different concerns and move toward creating a robust and sustainable hemophilia registry worldwide.

Keywords:

Bleeding, emicizumab, hemophilia, low- and middle-income nations, prophylaxis, registries

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Introduction

Hemophilia is a genetic bleeding disorder characterized by deficiency of clotting factor VIII (Hemophilia A) or IX (Hemophilia B).[1] The available global estimates suggest that the condition is equally prevalent in different age groups, with hemophilia A present in 1 in 5000 live male births, while hemophilia B presents in 1 in 30,000 live male births.[1] The findings of a recent review indicated that currently, 0.84 million people are living with hemophilia, of which more than one-third are suffering from a severe form of this rare condition.[2] It is quite alarming that almost three-fourths of the diagnosed patients in low- and middle-income nations have limited access to appropriate and adequate treatment.[3] Further, it was revealed that it can significantly impair the quality of life of affected persons, especially in low-resource settings, as reported in a study done in Nigeria, wherein 40% of study participants had extremely poor health status and 50% required annual hospitalization.[4] The objectives of the current article are to explain the significance of hemophilia registries, enlist the existing challenges in hemophilia registries, and propose potential solutions to overcome these identified challenges.

Hemophilia Registries

Hemophilia registries are structured databases that maintain longitudinal data on people diagnosed with hemophilia.[5] These registries generally maintain demographics (such as age, sex, and nation), genotype (mutation status of factor VIII and factor IX), severity (mild/moderate/severe), and treatment (namely, type of treatment [like on-demand, prophylaxis, and gene therapy], dosing, and

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infusion history).[5-7] In addition, details about outcomes such as bleeding frequency, hospitalization rate, quality of life, and durability of gene therapy are also maintained.[7] Moreover, some registries also maintain records of complications, like thrombotic events, infection status (HIV, HCV, and HBV), and records of adverse reactions.[8] These registries can be maintained at the international level (like the World Bleeding Disorders Registry, and Gene Therapy Registry), national level (such as the Colombian National Registry of Hemophilia or German Hemophilia Registry), and at the regional/hospital level (namely, the Each 2 Registry maintained records of 307 hemophilia A patients).[5-10]

Significance of Hemophilia Registries

Hemophilia registries maintained at different levels serve multiple public health purposes, which can eventually improve the treatment outcomes and quality of life of the affected person.[7,11-14] To begin with, it is an excellent medium for surveillance, and aids in the quantification of real burden, like the World Bleeding Disorders Registry maintains records of more than 10,200 patients residing in 40 nations.[7] The systematic analysis of the trends provides valuable insights into the diagnostic delays in lower and middle-income nations, which justifies the need to run screening initiatives.[7] The collected data can significantly assist in the planning of services and allocation of resources in low-income nations, which carries huge importance.[11,12] For instance, the data obtained from the Expanded Humanitarian Aid program streamlined the delivery of more than 0.25 million treatments in low- and middle-income nations within 5 years of the launch of the initiative.[11] In addition, another rural hemophilia treatment center project in the United States enabled a reduction in the use of hospitals by 85% and saved millions of dollars based on the prophylactic approach that was adopted on the basis of estimates of registries.[12] These registries provide vital information about the effectiveness of different strategies employed to improve treatment outcomes.[13] In fact, the results obtained from a Dutch registry revealed that the mean annual bleeding rates of patients on Emicizumab reduced to 1.5, while after 6 months of the administration of the drug, more than 60% of patients had no bleeding episodes.[13] These registries also provide information about the safety of novel agents, like permitting the safer roll-out of Emicizumab, which brought about a significant reduction in the incidence of 6% among new severe cases of hemophilia A.[14] In continuation, the same registry provided details about the thrombotic and hypersensitivity events on a three-monthly basis to keep the regulators informed about the potential adverse effects.[14] The registries that maintain records from different nations are useful to highlight equity gaps, especially when the data from low-income nations is compared with high-income nations.[7] Another utility of these registries is their ability to streamline workflows, including promoting the uptake of prophylactic therapy.[12]

Identified Challenges and Potential Solutions

Despite the immense utility of hemophilia registries in improving treatment outcomes, multiple challenges have been identified that could limit their scope and must be overcome [Figure 1].[5-7,15-25] The primary concern is that almost 75% of people residing in low-income areas are not diagnosed, which results in skewed and incomplete estimates, as was reported in the World Bleeding Disorder Registry.[7] This calls for the need to organize community-level awareness activities and train healthcare professionals at the primary level to recognize bleeding symptoms at the earliest.[15] This must be supported by subjecting all newborns and children to coagulation screening tests, and reporting all

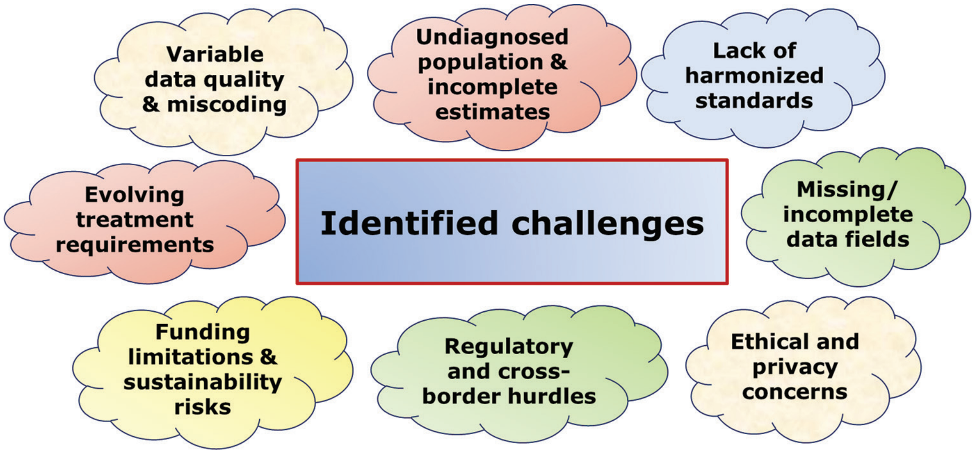

Figure 1: Identified challenges

positive cases to the existing registries, and the benefits of the same have been highlighted in a multicentric study.[16] Moreover, the option of compulsory reporting of hemophilia cases by patients and healthcare providers can also be explored, as it brought remarkable value to Colombia's registry program.[5]

The next concern is about the variable quality of data and their miscoding, especially when it is being taken from electronic health records, as it was obtained in a study in which only 56% of males met the laboratory criteria for congenital hemophilia, and there were multiple incidents of coding errors and copy-paste mistakes.[17] This problem can be minimized by introducing real-time checks into electronic forms, as it was successfully done in an international hemophilia registry, and almost 50% of missing fields were completed.[18] The approaches to conduct periodic audits to validate key outcomes and automate the process of data pulling from electronic health records, as it was done in the Centers for Disease Control and Prevention's hemophilia surveillance program, can effectively minimize the variability in data.[19,20] The next challenge is the absence of harmonized data standards, especially considering that the treatment approach might change during the course, like the inclusion of gene therapy.[6] This would essentially require mapping national core datasets to common data elements and joining the harmonization consortium to arrive at a consensus over variables and minimize the risk of duplication.[21,22]

The next concern is of long-term sustainability risk of existing hemophilia registries due to limited funding, as it was reported in a multinational registry that closed after only 2 years because of a combination of factors.[18] There arises the need to create multistakeholder (namely, Governments, insurers, and academia) funding, as it was done in Columbia's consensus-based registry, to minimize the financial load on a single agency.[5] Another practical solution to this problem was to go for a value-based care contract, which ensured the delivery of documented outcomes.[23] The final concern is regarding the complex ethics, privacy concerns, and cross-border regulatory hurdles, with many registries lacking clear plans for data sharing across jurisdictions.[18] The impact of this can be minimized by implementing privacy-preserving record linkage or tokenization, which will merge data without re-identification.[24] The approach to set up transparent governance boards with published data-access rules can ensure meeting legal and stakeholder expectations.[25]

Conclusion

Hemophilia patients can derive extensive benefits from the registries maintained at different levels. However, to optimize these benefits, we must adopt targeted, evidence-driven measures to tackle different concerns and move toward creating a robust and sustainable hemophilia registry worldwide.

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Conflicts of interest

There are no conflicts of interest.

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