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## Bleeding disorders: making strides towards treatment for all



June, 2020, saw the first World Federation of Hemophilia (WFH) Virtual Summit, the presentation of the latest WFH guidelines, and the publication of the 2019 WFH Annual Global Survey (AGS), which has become a key tool to understand the burden of bleeding disorders globally. The updated survey shows that over the past 20 years, the project has widened its reach, with 125 participating countries, and has documented progressive improvement in diagnosis and use of factor replacement therapy worldwide. However, the survey also reaffirms stark inequities in access to treatment.

Bleeding disorders are rare conditions, with 210 454 patients with haemophilia, 78 547 with von Willebrand disease, and 48 640 with other bleeding disorders identified worldwide in 2018. The past 50 years have seen steady progress in the management of haemophilia: the introduction of clotting factor concentrates in the 1970s achieved improvements in quality of life and life expectancy; the first recombinant factor for replacement therapy in 1992 saw safe prophylaxis become a reality; in 2017, the approval of emicizumab for patients with haemophilia A circumvented factor therapy failure; and finally, expected as early as this year, the approval of the first gene therapy might see the promise of a potential functional cure for haemophilia be realised. This progress means that many patients with haemophilia in high-income countries have access to treatments that allow them to live mostly normal lives. However, haemophilia care is not generally prioritised in resourced-constrained settings, and underdiagnosis, suboptimal treatment, and access to care are persistent problems in low-income and middle-income countries (LMICs). Other bleeding disorders have even fewer treatment options available worldwide.

Since 1963, the WFH has led a commendable effort to raise awareness and achieve treatment for all patients with bleeding disorders. In 2003, new WFH board member Alok Srivastava (Christian Medical College, Vellore, India) asked why the federation did not have guidelines to standardise care around the world. The first edition of the guidelines was published 2 years later. In 2020, the third edition aims to have a truly global focus, advocating for the use of prophylaxis over on-demand therapy in all settings. However, widespread prophylaxis use might be challenging to implement given the AGS

data on access to factor replacement therapy, the most common treatment for patients. Only 6% of the total international units (IUs) of factor VIII are accessible to 49% of patients living in LMICs in 2018 (data are similar for factor IX availability). With a substantial proportion of factor concentrates used in LMICs still being derived from blood products, shortages in blood donations during the COVID-19 pandemic might have broadened the gap in access even more in the past year. An additional concern during the pandemic is the use of fresh frozen plasma and cryoprecipitates in LMICs, discouraged in the latest WFH guidelines on the basis of safety concerns regarding inadequate viral inactivation.

In response to global inequalities, the AGS has been extended to include information on access to basic health care and treatment products, incidence of complications such as HIV and hepatitis C infections, and economic and population data. Another important initiative by WFH is the Humanitarian Aid Program, established to facilitate access to treatment in LMICs. Donations are often the only source of treatment products for many haemophilia treatment centres in LMICs. In 2018 alone, the programme distributed 191 million IUs of factor products to 62 countries, treating more than 18 000 patients. Therefore, since 2002, the AGS also collects data on humanitarian aid product use, which helps determine country eligibility for donations. This aid programme is key to achieving donation commitments for future years, but it also highlights the need to work towards implementing local sustainable care, educating health-care providers, delivering genetic counselling services, and exploring the possibility of gene therapy as part of twinning programmes for a haemophilia-free world in the future.

Data remain key to guide policy changes, and for the bleeding disorders community, the AGS has been and continues to be an instrumental tool to inform change. The achievements of the haemophilia community over the past five decades are to be celebrated, but much advocacy remains to be done to achieve treatment for all; engaging local governments, pharmaceutical companies, health-care providers, and patients is paramount to achieve investment in registries and health-care infrastructure, training and education, and facilitate access to safer treatments worldwide. ■ *The Lancet Haematology*



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For more on the **WFH Annual Global Survey** see *Hemophilia* 2020; published online June 4. <https://doi.org/10.1111/hae.14012>

For more on the **WFH Guidelines for the Management of Hemophilia** see <https://news.wfh.org/coming-soon-hemophilia-guidelines-for-all-a-new-ambition-of-the-wfh/>

For more on **blood shortages during the COVID-19 pandemic** see <https://www.healio.com/news/hematology-oncology/20200326/blood-drive-cancellations-amid-covid19-outbreak-put-big-strain-on-supply> and <https://www.paho.org/en/news/10-4-2020-paho-warns-potential-blood-shortages-during-covid-19-pandemic>

For more on the **WFH Humanitarian Aid Programme** see <https://www.wfh.org/en/wfh-humanitarian-aid-program>