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Gathering and Disseminating Standardized Gene Therapy Data – The World Federation of Hemophilia Gene Therapy Registry

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Introduction: The World Federation of Hemophilia (WFH) has launched the Gene Therapy Registry (GTR) aimed at gathering comprehensive data on all people with hemophilia (PWH) who receive gene therapy globally.

Methods: The GTR is a prospective, observational, and longitudinal registry. It was designed to standardize and centralize global data collection by establishing a single, unified repository for the gathering and dissemination of gene therapy data, ensuring mutual benefit for all stakeholders. Data entry occurs once, either directly into the GTR platform or through data transfer from National Registries.

The GTR will disseminate specific de-identified data to various stakeholders. The GTR Scientific Advisory Board accesses global data for monitoring safety and efficacy. Hemophilia treatment centres (HTC) and National Registries will receive aggregated global safety data. Regulatory agencies and Health Technology Assessment organizations can request specific data to inform their decisions, while industry partners will receive product-specific data.

Results: The WFH is engaged with a broad network of HTCs and National Registries to establish mutually beneficial collaborations with the GTR. To foster dialogue and obtain feedback from our collaborators, the GTR National Registries & HTC Consortium has been established. This group includes representatives from Ireland, Sweden, Brazil, and Saudi Arabia, and registries from Australia, Canada, France, Germany, Japan, the Netherlands, Spain, the United Kingdom, and the United States.

Based on the GTR protocol, core data set and methodology, the European Medicines Agency (EMA) Committee for Medicinal Products for Human Use (CHMP) has shown strong support for the GTR. The CHMP endorses the GTR as the worldwide registry for consolidating all international data on PWH who receive gene therapy and encourages collaboration of all HTCs and National Registries, stating that the WFH GTR is of particular value for post approval safety and efficacy studies of gene therapies and recommending its use as a planned data source for mandated Phase IV studies.

Discussion/Conclusion: The GTR simplifies the data entry process, facilitates efficient data sharing, and provides valuable information to all stakeholders, advancing our understanding of gene therapy's safety, efficacy, and long-term effects, ultimately contributing to improved patient care and treatment outcomes.

Disclosure of Interest: None Declared