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EXECUTIVE SUMMARY

National Hemophilia Foundation-McMaster University Guideline on Care Models for Hemophilia Management

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NATIONAL HEMOPHILIA FOUNDATION
for all bleeding disorders

McMaster University

The NHF-McMaster Guideline on Models of Care for Hemophilia have been endorsed by the World Federation of Hemophilia (May 20, 2016), the American Society of Hematology (May 27, 2016), the International Society on Thrombosis and Haemostasis (May 28, 2016).

WFH WORLD FEDERATION OF HEMOPHILIA

American Society *of* Hematology

ISTH International Society on Thrombosis and Haemostasis

As part of its mission to find better treatments and cures for all bleeding disorders and to preventing the complications of these disorders through education, advocacy and research support, the National Hemophilia Foundation (NHF) decided to sponsor the production and maintenance of evidence-based clinical practice guidelines choosing the topic of Care Models for Hemophilia Management as the important first step and baseline in their guideline efforts. NHF sought the expertise of McMaster University to serve as a partner in guideline development.

A number of national and international guidance documents have supported integrated care as the optimal model of care for people with hemophilia (PWH). However, the effects of integrated care on patient-important outcomes had not been systematically synthesized or compared to alternative models and questions about the ideal composition of services and providers for optimal hemophilia care remain unanswered.

- This guideline was developed to identify best practices in hemophilia care delivery to optimize outcomes for people living with hemophilia across the United States.
- It was developed foremost for persons living with hemophilia and for providers of hemophilia care.
- In addition, the guideline is meant to be a resource for hospitals and healthcare systems, Federal and State programs and policy makers, private and public insurers, and other professionals in the health sector who are responsible for developing and implementing strategies to care for individuals with hemophilia and other bleeding disorders at the national, regional and state levels.

METHODS

The methods used to develop these guidelines adhered to suggested principles for developing transparent, evidence-based guidelines promoted by the Institute of Medicine, the National Guideline Clearinghouse, and the Grading of Recommendations, Assessment, Development, and Evaluation (GRADE) Working Group.

Panel Composition: The Guideline Panel was composed of U.S. and non-U.S. health care providers (including physicians, nurses, physical therapists, and a genetic counsellor) with expertise in hemophilia care, individuals with experience in health policy, health care financing, and research related to hemophilia, PWH, parents of PWH, persons with other rare diseases, and methodologists. Conflicts of interest were disclosed and managed throughout, adhering to Institute of Medicine standards.

Patient Important Outcomes: Panel members and key stakeholders were surveyed to define guideline questions and patient-important outcomes (mortality/survival; missed days from work or school; number of emergency department visits; length of in-patient stay; quality of life; joint damage/disease; educational attainment; patient adherence; and patient knowledge).

Evidentiary Review: Systematic reviews of the literature were conducted for all factors important in decision making: benefits and harms; patient values and preferences; resource implications; acceptability; equity; and feasibility. Standardized Evidence Profiles and Evidence to Decision frameworks were developed using the GRADE approach to guide the Panel as they made their recommendations. When evidence for PWH was of low quality or not available evidence from other chronic diseases was evaluated to directly and indirectly inform the recommendations. Observations from experts were systematically pooled and a qualitative study exploring stakeholder experiences and perspectives was conducted to further inform the guideline.

The Panel made recommendations for each guideline question, and elaborated on research and implementation considerations. Final recommendations were circulated for public review.

GUIDELINE RECOMMENDATIONS

Should integrated care versus non-integrated care be used for people with hemophilia?

The Guideline Panel suggests that the integrated care model be used over non-integrated care models for PWH (conditional recommendation, moderate certainty in the evidence). For PWH with inhibitors, and those at high risk for inhibitor development, the same recommendation was graded as strong, with moderate certainty in the evidence.

For individuals with hemophilia, should a hematologist, a specialized hemophilia nurse, a physical therapist, a social worker, or round-the-clock access to a specialized coagulation laboratory be part of the integrated care team, versus an integrated care team with a lesser complement?

The Panel suggests that a hematologist, a specialized hemophilia nurse, a physical therapist, a social worker, and round-the-clock access to a specialized coagulation laboratory be part of the integrated care team, over an integrated care team that does not include all of these components (conditional recommendation, very low certainty in the evidence).

REMARKS

Using an evidence-based methodology, the NHF-McMaster Guideline on Care Models for Hemophilia Management suggests the integrated model of care, in its current structure, for optimal care of PWH based on the certainty of available evidence.

There is a need for further appropriately designed studies that address unanswered questions about specific outcomes and the optimal composition of the integrated care delivery model in hemophilia. The Panel has supplemented its recommendations with clear suggestions to guide NHF and the broader hemophilia community in setting research priorities to consolidate and expand the evidence base of the guideline's recommendations.

The Panel recommends that further studies be conducted in: geriatric populations; populations with poor access to care; and PWH who access care outside of Hemophilia Treatment Centers (HTCs). Further research is needed on which aspects of the integrated care model are a "value add," and what the impact of remote care delivery systems are on care. A wider range of outcomes must also be studied, including: cost of care; factor utilization; lost days of school and work, educational and employment attainment; and impact of patient factors on outcomes. HTCs can play an important role in generating high quality evidence by building their data collection and analysis capacity, and continuing to conduct high quality studies.

As these guidelines are implemented, training, recruitment and retention of specialized health care team members working within the integrated care model should be prioritized. Performance measures should also be developed and tracked to determine their effect on the identified patient important outcomes. Care models must continue to respond to the changing natural history of hemophilia and the changing healthcare system, and integrated care treatment centers must respond to the needs of their patient population in a dynamic way to ensure their long-term sustainability.

This work was funded by the National Hemophilia Foundation.

A detailed version of this guideline, as well as an introduction to care models in hemophilia, a methods paper, two systematic reviews, and a qualitative study have been published in the July 2016, Vol. 22 Suppl. 3 issue of the journal *Haemophilia*, available online at: http://onlinelibrary.wiley.com/doi/10.1111/hae.2016.22.issue-S3/issuetoc. Readers who wish to review this material will find that its online data supplements include evidence profiles (which present the data that informed this guideline), as well as evidence to recommendation tables (which outline how the evidence was used to inform the guideline's final recommendations).

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