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Defining the Role of Nurses in Gene Therapy for Haemophilia

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Abstract

Introduction: Haemophilia is a rare bleeding disorder now undergoing a major shift in care as gene therapy (GT), delivered through viral-vector in vivo gene transfer, has enabled adults with haemophilia A or B to stop prophylaxis with minimal bleeding. As this treatment becomes a standard option in several European countries, nurses play an essential role in education, multidisciplinary care coordination, bleed management, and long-term follow-up, requiring ongoing professional development and potentially new specialist roles.

Aim: To describe and explore the multifaceted responsibilities of nurses throughout the GT pathway, emphasising their critical role in delivering holistic, patient-centered care.

Method: This manuscript draws on discussions among experienced European haemophilia nurses to examine their expanding responsibilities throughout the GT pathways.

Results: Nurses play a central role throughout the GT pathway in haemophilia, guiding shared decision-making, preparing patients for infusion, delivering safe peri-infusion care, and coordinating extensive post-infusion monitoring. Their responsibilities include eligibility assessment, patient education, vigilant observation during infusion, early detection of adverse events, and long-term follow-up of liver function, factor levels, and treatment durability. They also support psychosocial adjustment, provide lifestyle counselling, uphold ethical communication about uncertainties, and ensure continuity of care across hub-and-spoke networks. In addition, nurses contribute to registries, quality improvement, and research, generating real-world evidence essential for evaluating long-term safety and effectiveness of GT.

Conclusion: GT in haemophilia requires well-trained and well-informed nurses to ensure safe delivery, effective follow-up, and high-quality patient support throughout the entire treatment pathway.

1 | Introduction

Haemophilia is a rare, disorder leading to bleeding after injury, surgery, or spontaneously, predominantly into joints and muscles. Treatment involves infusions of the missing clotting factor. Newer therapies, including non-factor replacement treatments and gene therapy (GT) are emerging [1]. As a single gene disorder, haemophilia is an optimal disease for implementation of GT The most common GT to date involves in vivo gene transfer, which uses a non-pathogenic viral vector to transduce the target gene into hepatocytes [2]. Multiple GT trials for adults with haemophilia A or B without inhibitors have been performed and

are clinically effective, with the majority of patients stopping prophylaxis with minimal to no bleeds [3, 4].

GT represents a transformative advancement in haemophilia treatment, offering the potential for sustained endogenous production of clotting factors through a single infusion [5]. As GT is being offered as a standard treatment option in several countries for patients with haemophilia (PWH), nurses have to be prepared to offer PWH specific guidance and care. Nurses are indispensable in haemophilia management, not just as caregivers, but as coordinators, educators, and advocates. Their role extends far beyond administering treatment.

The provision of comprehensive care requires a multidisciplinary approach in order to ensure seamless care. Nurses are able to serve as a linchpin in the process, connecting haematologists, physiotherapists, social workers, and other specialists, to teach patients and their families how to manage bleeding episodes, administer treatment, and recognise early signs of complications as well as provide emotional and practical support. Their role extends from helping with daily management, to advanced tasks, such as organising treatment and surgery plans in conjunction with haematologists and leading research and quality improvement initiatives.

As treatment advances, continuous professional development is vital to ensure nurses remain competent across diverse practices and national contexts [6, 7]. Nurses are uniquely positioned to guide patients through the complex journey of GT; from education and decision-making to long-term follow-up [8]. Nurses are involved in every step of the delivery of GT in haemophilia centres [9]. Depending on future care delivery models the haemophilia nurse and GT nurse could be one or two specialists, thus requiring care coordination [10].

This paper aims to describe and explore the multifaceted responsibilities of nurses throughout the GT pathway, emphasising their critical role in delivering holistic, patient-centered care.

2 | Methods

The concept of this manuscript arose from conversations amongst the authors about the changing role for haemophilia nurses regarding GT. The authors, who represent a range of European nurses with expertise in GT research and clinical care, met several times online between September 2025 and March 2026 to define the project and to summarise findings.

Before formulating the perspectives presented in this opinion article, a focused review of the current scientific literature was performed to ensure any recommendations are grounded and contextualised in established evidence and contemporary developments in the field. This review included peer-reviewed studies, clinical guidelines, and recent expert analyses.

3 | Results and Discussion

Although nurses are frequently mentioned across various publications on GT for haemophilia, the existing literature does not provide a dedicated or comprehensive analysis of their specific role within this evolving treatment landscape. Most articles acknowledge nursing responsibilities only indirectly—often in the context of patient education, monitoring, or multidisciplinary care—without exploring the full scope of nursing contributions or the unique competencies required for GT implementation. This gap highlights the need for focused research that systematically examines the nursing perspective, as well as the educational and clinical frameworks necessary to support safe and effective integration of GT into routine haemophilia care.

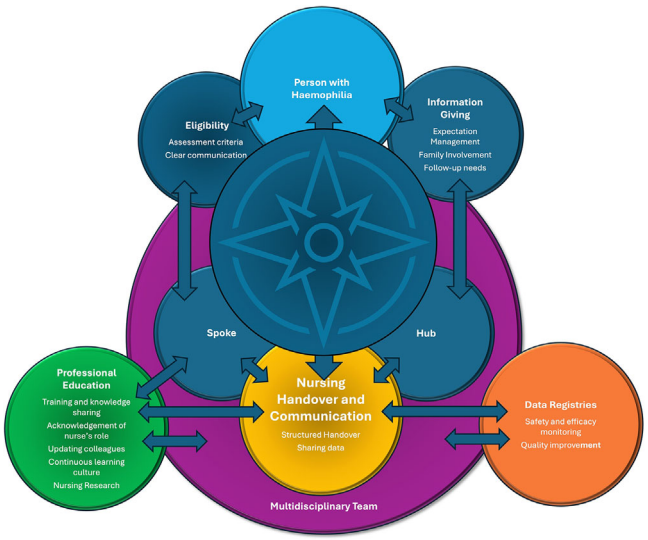

FIGURE 1 | Diagrammatic representation of the role of the nurse in gene therapy.

Key roles and responsibilities of nurses for GT in haemophilia care are summarised in Table 1 and Figure 1. Additional roles for specific GT nursing support and care, which follow the patient journey are discussed further here.

3.1 | Patient Education

Over time, GT may become as accessible as CAR-T gene therapies currently available worldwide, including in resource-limited settings. Although the initial generation of GT began with some pessimism, evolving data suggest a more optimistic future [11].

Therefore, effective education for both patients and nursing professionals is a critical component of high-quality care, particularly in complex therapeutic areas such as GT haemophilia management. Comprehensive patient education enhances understanding of how it works and what differences (or not) it is making in the everyday lives of PWH. Nurses have to empower individuals to participate actively in shared decision-making. At the same time, continuous education of nurses ensures that they remain proficient in evolving therapeutic strategies, safety monitoring, and patient counselling. Together, these educational efforts strengthen the therapeutic alliance, improve clinical outcomes, and support the safe and consistent implementation of advanced treatment modalities.

TABLE 1 | Nursing key roles and tasks in gene therapy for a person with haemophilia.

Section	Topic	Key Points
Patient Education	**Information Session**	Compare expectations against other novel therapies (next 2–5 years)
		Explain gene therapy, risks, benefits, expectation variability or no response; duration of response; potential medications required post-therapy
		Involve family in SDM and for follow-up care
		Need for intensive follow-up and sharing outcome data
		Allow sufficient time for consideration; Debriefings may be necessary
	Patient Eligibility	Consider concomitant illnesses/medications
		Tests for AAV antibodies, liver & cardiac health
		Provide clear, patient-specific information about process
	Hub and Spoke or Hub Care?	If initial session in Spoke, arrange supplementary Hub session; Close Hub-Spoke coordination
	Follow-up Care	Regular visits (weekly initially, first 6–12 months); Hub-Spoke communication essential; Home care possible if centres are far; Monitor factor activity & transaminases; Steroid therapy if transaminases elevated; Record labs digitally for early detection
Professional Education	**Training & Knowledge Sharing**	GTH Academy (Germany): education & research; EAHAD: continuous education for European centres; Inform other specialists (hepatologists, orthopaedic, and trauma surgeons); Regular virtual MDT meetings for safety
	Role of Nurses	Acquire gene therapy knowledge; Coordinate tasks between Hub, Spoke, and patient; Support cooperation, patient discussions, therapy organization, follow-up; Adapt care approach (prophylaxis vs on-demand); Provide psychosocial support for new self-perception; Ensure smooth Hub-Spoke collaboration
	Professional Development	Nurses & doctors attend congresses and trainings; Pharma companies offer courses; Weekly clinic meetings for problem-solving
	Keeping Colleagues Updated	Document weekly meetings and share reports; Maintain accessible folders with lab forms & guidelines
	Continuous Learning Culture	Appreciation & motivation from physicians; Build trust with patients for better care
	Reflective Practice & Peer Support	Supportive team culture: Problems discussed openly, solutions found collaboratively
Multidisciplinary Team (MDT)	**Involvement & Candidate Selection**	Hub Centre organizes meetings with all parties; Nurses act as link between MDT and patient; Manage appointments, prescriptions, treatments
	Assessing Eligibility & Readiness	Careful eligibility check (e.g., liver health); Education only for motivated patients; Requirements: regular monitoring, avoid alcohol, maintain liver health, safe sexual practices, immunosuppressive meds if needed; Joint decision-making with patient, family, physician, nursing staff; Organize Hub-Spoke collaboration
Nursing Handover & Communication	**Structured Handover**	Responsibilities divided by agreement; Hub*: coordinates all aspects before & after infusion; Spoke**: care beyond infusion (visits, exams, MDT); Joint decisions during follow-up; Care division individualized per patient; Use documentation apps

(Continues)

TABLE 1 | (Continued)

Section	Topic	Key Points
	Sharing Data & Preferences	Share patient experiences during MDT meetings
	Ensuring Continuity of Care	Inform patient when to contact doctor for side effects; Guidelines for injuries/surgeries; Organize blood sampling (possibly home care); Support for elevated liver values & therapy; Use documentation apps

*Hub: Treatment centre performing the gene therapy infusion
**Spoke: Local/regional centre providing ongoing care

Nursing research in GT often focuses on understanding patient experiences, improving care processes, monitoring outcomes, and strengthening education and adherence.

3.2 | Shared Decision-Making

Shared decision-making (SDM) is a collaborative process in which patients and healthcare providers make informed treatment choices. Nurses play a key role in this process, using the globally agreed World Federation of Haemophilia (WFH) SDM tool [12], to structure discussions, present balanced evidence on GT, address uncertainties and encourage questions. The WFH tool helps PWH to understand treatment options, weigh up the risks and benefits, and express personal preferences. Nurses facilitate PWH comprehension by translating complex terminology, providing accessible information and encouraging patients to ask questions. Open communication fosters trust and enables PWH to share their values and goals. Nurses also coordinate discussions with the care team, document preferences and follow up on decisions. By using the WFH SDM tool, nurses can link clinical expertise with patient autonomy, thereby promoting empowerment and better outcomes. Nurses guide patients through three phases of GT: pre-infusion (including SDM), peri-infusion, and post-infusion [9].

3.3 | Pre-Infusion Preparation

Pre-infusion assessment ensures eligibility and safety. This includes medical history, physical and musculoskeletal examination, and laboratory tests, including screening for adeno associated virus (AAV) antibodies and hepatological evaluation. Structured guidelines including referral, dosing and follow up should be developed. These should follow national [13] or international recommendations [14, 15] adapted according to local requirements. Site readiness should be addressed before patient engagement [10].

In haemophilia A, AAV5 antibodies exclude treatment; in haemophilia B, therapy proceeds only if titers are below threshold [16]. Psychological readiness is also assessed. Nurses educate patients on expectations, monitoring, and lifestyle factors such as contraception, alcohol restriction, immunosuppression, psychosocial issues, and drug interactions [17], including discussion of efficacy, durability, and side effects to foster trust and adherence [18]. Non patient facing tasks include verification of eligibility, drug storage, organisation of dosing (liaison with pharmacy/manufacturer etc.), preparation of infusion materials, and organisation of emergency resources.

3.4 | Peri-infusion Responsibilities

The infusion day requires meticulous clinical attention and coordinated teamwork, as it represents the crucial moment of GT administration. Nurses combine technical competence with relational care through vigilant monitoring, empathetic support, and effective communication within the multidisciplinary team. The infusion should take place in a dedicated setting, with immediate access to emergency equipment and resuscitation facilities. Given the complexity of procedures and required coordination, treatment centres should develop standardised infusion-day protocols to ensure safety and efficiency.

On the day of infusion, several elements must be reconfirmed, including informed consent, laboratory assessments and patient eligibility. Administration follows institutional protocols and the product label [19]. GT differs from conventional treatments as it is a one-time intravenous infusion. During the infusion, vital sign monitoring is essential to detect and manage possible adverse events. Although rare, infusion-related reactions such as hypersensitivity or anaphylaxis have been reported [20]. Therefore, predefined management strategies should be available, including supportive drugs, temporary interruption, or adjustment of infusion rate [13].

Accurate documentation of clinical observations and patient responses, along with attention to perceptions, concerns, and expectations, supports individualised care and ongoing improvement of GT pathways [21]. Before discharge, nurses should review follow-up plans covering immediate monitoring and long-term surveillance to ensure clinical safety, patient reassurance, and effective post-infusion care.

3.5 | Post-Infusion Care

The post-infusion phase is crucial for monitoring and detection of adverse events, ensuring patient safety. Nurses must be clinically vigilant, educate patients, and coordinate care networks. Follow-up spans three periods: days to weeks, weeks to months, and months to years. Each period has specific criteria [22]. During the initial phase, frequent laboratory tests are used to monitor efficacy

and safety, with a focus on factor activity and liver function. Chromogenic assays may provide more reliable measurements of transgene expression than one-stage assays in haemophilia A [23]. Nurses must recognise immune-mediated hepatotoxicity. The use of prophylactic glucocorticoids to prevent transaminitis has been explored, current guidance favors a structured, reactive approach to corticosteroid administration with defined criteria [24, 25, 26].

Patient empowerment through education is central to post-infusion care, emphasising the importance of promptly reporting unexpected symptoms. Nurses facilitate understanding, adherence, and trust, thereby enhancing early recognition and management of complications. Effective communication between hub and spoke centres ensures continuity of care. The Hub and Spoke model (if applicable) ensures a structured follow-up beyond the infusion centre, supporting long-term safety and efficacy monitoring. In this framework, specialised hubs oversee advanced clinical assessment and long-term monitoring, while regional spokes manage routine evaluations, laboratory testing, and ongoing patient follow-up [21, 27]. Nurses act as intermediaries, ensuring adherence, facilitating the flow of information, and collaborating with specialists to sustain therapy success.

3.6 | Data Registry and Quality Monitoring

Due to the novelty of GT limited long-term follow up data, registry participation is essential for tracking outcomes and detecting adverse events [23, 28]. Nurses play a pivotal role in this process and often coordinate data collection, leveraging their continuous contact with patients to ensure accuracy and completeness. Monitoring includes factor expression levels, bleeding patterns, immune responses and hepatotoxicity [18, 29]. Early identification of trends enables timely intervention. Beyond data collection, nurses contribute to quality improvement by analysing registry data to benchmark outcomes and identify care gaps. They also support research by facilitating patient participation in long-term follow up studies and post-marketing surveillance, thereby generating real-world evidence on the safety and efficacy of GT [18]. Participation in registries such as the WFH GT Registry should be encouraged in order to collect patient-reported outcomes. [30].

3.7 | Longterm Follow-up and Coordination of Care

Successful GT requires comprehensive long-term monitoring and coordinated care. Ongoing factor level assessment helps maintain therapeutic durability [28, 29]. This monitoring is essential not only for safety assessment but also to determine when (or if) to reintroduce prophylaxis if GT expression declines. Care often involves multiple specialists, requiring nurses to organise schedules that minimise patient burden while ensuring appropriate monitoring. Coordination may include primary care and referrals as needs evolve. Individualised care plans address the varying needs of PWH based on their response to therapy and comorbidities. These plans enhance engagement and retention in follow-up programmes, which are vital for long-term success through continued participation in monitoring, while engagement keeps patients informed and motivated. Although MDT meetings occur throughout the patient journey, they play a particularly important role during follow-up, where the team jointly evaluates clinical progress and manages any emerging issues. Retention strategies include flexibility, timely communication, integrating trial activities with routine care, SDM and using digital health tools [28, 29].

3.8 | Psychosocial Support and Lifestyle Counselling

GT for PWH represents not only a medical intervention, but also a profound life change that influences identity, body image and future expectations [31]. Nurses play a crucial role in guiding patients through this transition by addressing their emotional needs, supporting them in making lifestyle adjustments and ensuring that their decisions align with their personal values rather than solely clinical goals [32, 33]. The shift from chronic bleeding to near-normal health can evoke mixed emotions, ranging from relief and optimism to anxiety and difficulty adjusting [34]. Nurses monitor psychological distress, facilitate referrals to psychology services and provide ongoing support.

Lifestyle counselling is an important part of post-therapy care, focusing on behaviours that affect the durability of treatment, such as reducing alcohol intake and encouraging behaviour change tailored to the individual [29].

3.9 | Ethical Considerations

GT is still in its early stages, with uncertain long-term efficacy and safety [35]. This includes understanding durability, the possibility of returning to prophylaxis and the implications for life planning.

Decision-making about undergoing GT is deeply personal and is shaped by values and social context. Patients often struggle with complex terminology, highlighting the importance of clear and empathetic communication [33]. Nurses bridge this gap by using patient-centred language, tailoring explanations to health literacy levels, and providing space for reflection. Supporting patients to make value-based decisions also means recognising the broader psychosocial context, including family dynamics and personal aspirations [36]. Healthcare professionals must anticipate complications such as immune reactions and unintended effects [34]. Nurses support consent and long-term monitoring, communicate uncertainties such as the duration of treatment, delayed side effects and the need for ongoing surveillance honestly, while fostering informed consent and engagement. As the primary point of contact for PWH throughout the GT journey, nurses monitor PWH, coordinate their multidisciplinary care and reinforce adherence to follow-up [16].

There is variability in legal and regulatory frameworks of nursing roles, scope of practice, and autonomy globally, which are set by national laws and professional regulations. This will therefore influence the role of nurses in GT delivery in each individual country, but their role in the support of PWH will continue to grow as GT becomes more available. Continued professional education and development of nurses is therefore paramount.

GT is yet not available to children other than in a clinical trial setting. It is not yet known when and if GT for children will be licensed. That said, the principles outlined in this paper will be applicable with some modification to paediatric care.

4 | Conclusion

Nurses, along with the other members of the MDT play an important role in making GT a safe, understandable, and empowering experience for people with haemophilia. As a constant presence in a patient's journey, they help translate complex science into clear, practical guidance, support SDM, and recognise when psychological or clinical concerns need escalation. Their long-term involvement helps patients adapt to new expectations, overcome old habits, and navigate the challenges that persist even after treatment. In every sense, nurses are the vital bridge between innovation and compassionate, person-centred care, ensuring that GT delivers not only scientific progress but meaningful, lasting benefit for PWH and their families. Therefore, education is crucial, to equip nurses with the knowledge and confidence to guide PWH and their families through SDM and the complexities of GT.

Declaration of Generative AI and AI-assisted technologies in the writing process

Microsoft Copilot (Microsoft) was used to suggest an initial framework for the manuscript, though this framework was subsequently revised and adapted by the authors. Generative AI tools, including Grammarly and Copilot, were used to provide textual support as English was not the first language for a number of the authors. All outputs were reviewed, revised, and validated by the authors who assume full responsibility for the accuracy and integrity of the final manuscript.

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Conflicts of Interest

The authors declare no conflicts of interest.

Data Availability Statement

Data sharing not applicable to this article as no datasets were generated or analysed during the current study.

Declarations

This manuscript is an opinion piece and does not involve human participants, patient data, or interventional research; therefore, ethical approval and informed consent were not required.

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