PATIENT JOURNEY MAPPING OF THE GENE THERAPY IN HAEMOPHILIA B IN SPAIN – THE BHEMOGEN PROJECT

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BACKGROUND AND OBJECTIVES

- Haemophilia B is a rare, inherited bleeding disorder characterized by an increased bleeding tendency due to a partial or complete deficiency of clotting factor IX (FIX).
- The approval of the gene therapy for the treatment of Haemophilia B represents a paradigm shift in the management of this disruptive innovation presents challenges and opportunities for healthcare systems, professionals and patients that must be addressed in a practical and holistic way to ensure that this therapeutic option is accessible to candidate patients with Haemophilia B in Spain. Despite the considerable experience in treating Haemophilia B and the recent introduction of gene therapy in a different indication, the literature shows notable absences of patient care roadmaps for neither Haemophilia B nor treatment with gene therapy in any indication.
- The objective of this project is to develop a patient journey for the first gene therapy in Haemophilia B in Spain from a multidisciplinary point of view and to generate knowledge on practical and contextual aspects affecting its introduction and practical management by identifying challenges and proposing solutions.

METHODS

Literature review to identify current care pathway, challenges and practical experiences derived from the indications. Constitution of a multidisciplinary group of ten experts (haematologists, nurses, hospital)

pharmacists, regional specialists in advanced therapies, hospital managers, representatives of patient associations) from five Autonomous Communities with experience in the management of Haemophilia B and gene therapy, including CSUR centres and reference centres for this pathology. **Design of the patient care roadmap** based on individual interviews, group discussion and consensus, including differences and actions required versus the current care pathway for Haemophilia B. Obtaining scientific endorsements from Scientific Societies and Patient Associations.

RESULTS

PROPOSED PATIENT CARE ROADMAP - GENE THERAPY IN HAEMOPHILIA B IN SPAIN

- The patient care roadmap for gene therapy in Haemophilia B designed and agreed by the group of experts covers all different stages from diagnosis to long-term follow-up of the patient (Figure 1).
- Its design is based on the current Haemophilia B patient care roadmap, identifying the necessary organizational, structural and care changes specifically related to this new therapeutic option (Figure 1).

Roadmap for patient care process - Gene therapy in Haemophilia B in Spain



- **1. DIAGNOSIS**. Introduction of gene therapy does not impact this step since all patients are already diagnosed.
- 2. PATIENT SELECTION AND TREATMENT. Hepatology and infectology/immunology profiles are added as experts involved in this process. The clinical eligibility criteria, evaluation and **selection** of the patient candidate for gene therapy are determined by the Summary of Product Characteristics and will be individualized on a case-by-case basis.
- **INFORMED CONSENT** (IC). The IC is crucial in this therapeutic modality, and differential to the currently used or the one that it is used in clinical trials with gene therapy. Introduction of a specific IC generation process and documentation for gene therapy including detailed information, as well as commitments and obligations for the patient is required. The **psychology** and hepatology profiles are included as advisory roles. The psychological evaluation of the patient prior to signing the IC document is critical.
- 4. INITIATION OF TREATMENT: It contemplates adaptation of specific areas for the preparation and administration of gene therapy and the implementation of a detailed and specific post-

Abbreviations: FIX: factor IX; WFH: World Federation of Haemophili

Figure 1: Proposed patient care roadmap for gene therapy in Haemophilia B in Spain.

infusion follow-up protocol for this therapeutic modality. Gene therapy treatment is complex and requires **specialized and continuous training** for every healthcare professional involved.

4. SHORT- AND LONG-TERM FOLLOW-UP: The figure of hepatology is added given the need for continuous monitoring of the patient's liver health in the short and long term.

ECONOMIC ASPECTS RELATED TO CURRENT STRUCTURING AND FUNCTIONING OF THE SPANISH NATIONAL HEALTHCARE SYSTEM

Adapting Centres' structures and ensuring adequate qualification for gene therapy infusion

It will be important to find the right formula to balance key aspects such as the structural and organizational adequacy of hospital centres where patients already receive their usual therapy and are followed up by their Haematology Unit, and the training and qualification of healthcare professionals ensuring treatment availability and equity of access by patients who are candidates for such therapy in the different Spanish Autonomous Regions.

Cost compensation logistics for patients from other Autonomous Communities

Collaboration between hospitals that have already established administrative processes for the management of advanced therapies and cross-payments between Autonomous Regiones, with those centres that do not yet have this experience, in order to facilitate procedures and improve efficiency in the economic management of gene therapy will contribute to access and equity.

Operability of registry systems for the follow-up of clinical variables

Patient registries represent a basic tool both for clinical research and for short- and long-term evaluation of gene therapy effectiveness and safety. A strategy of continuous adaptation and improvement of existing patient registry systems and clinical variables is proposed, in order to effectively addressed the changing dynamics of medical care and the incorporation of disruptive therapeutic innovations such as gene therapy.

PENTALOGUE OF ACTIONS AND RECOMMENDATIONS FOR THE PRACTICAL IMPLEMENTATION OF THE PROPOSED PATIENT CARE ROADMAP



guaranteeing availability and equity in patient access across the different Spanish regions.	coordinated and multidisciplinary approach.	patient informed consent process.	obtaining robust data on clinical outcome variables.	participation in each stage of the patient journey.
CONCLUSIONS				
This project represents the first patient care roadmap for gene therapy for Haemophilia B in Spain. Its implementation in Spanish hospitals could contribute to guide effective incorporation of this disruptive therapy within the healthcare system and delivery the expected health outcomes. It does also represent the first pathway for any gene therapy in Spain. The identified challenges and the proposals to address them set a precedent for current and future gene therapies. Incorporation of the proposed steps in Haemophilia B-treating centres must be accompanied by a process flow that includes a guarantee of timelines and governance to achieve the necessary quality of care and the desired clinical objectives.				
Nith the scientific endorsement of the following Scientific Soc	cieties and Patient Associations:		De	eveloped by Sponsored by







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