## First Real-world Experience Administering Etranacogene Dezaparvovec Gene Therapy for People with Hemophilia B

Disclosures

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## Table 1. Center-specific Documents Developed for the Clinical Background Site Preparedness Journey in Gene Therapy Etranacogene dezaparvovec-drlb STAKEHOLDER EDUCATION PROTOCOL DEVELOPMENT MANUFACTURER SUPPORT (CSL Behring) is the first gene CENTER-SPECIFIC DOCUMENT RELATED STEP IN CLINICAL JOURNEY therapy for hemophilia B to be The HTCs developed center-specific protocols, checklists, and Early education, both for HTC The care team received education from the manufacturer through approved by the US Food and Drug stakeholders and patients, was critical in addressing key steps in worksheets based on the key steps in the gene therapy Administration<sup>1</sup> (2022), the Europe clinical process (Table 1) Clinical administration training Training VALUATION WORKSHEET Medicines Agency<sup>2</sup> (2023), and the treatment journey (see figure These protocols provided clear guidance on the execution Operational training of every step of the process in addition to providing a Health Canada (2023)3 below) - Ordering The gene therapy treatment journey contingency plan for different scenarios (e.g., infusion The education was iterative reactions and transaminitis), ensuring the center's - Receiving is a multistep process that requires coordination between multiple involved multiple stakeholders (see key in figure below), and required PATIENT AGREEMENT FORM preparedness for administration - Neutralizing antibody testing stakeholders within a hemophilia open communication between the PRACTICAL CONSIDERATIONS HTCs, the patients, and the · A detailed handbook describing the gene therapy process as it relates to Resources treatment center (HTC) and The HTCs established the care team (including the ANDUING AND PREPARATION POLICY potentially between multiple HTCs<sup>4</sup> manufacturer (when appropriate) etranacogene dezaparvovec-drlb hematologist, pharmacist, and nursing staff, among Experience sharing is critical for HTC others), assigned roles for administration day, and Etranacogene dezaparvovec-drlb preparation and administration video teams to fully understand and rehearsed administration day in advance INFUSION DAY CHECKLIST Preparation and administration The manufacturer facilitated discussions focused on practical considerations for clinical excellence appropriately implement each step The care team ensured the supplies for the management of the gene therapy process of potential infusion reactions were available if needed · A dedicated team from the manufacturer was also present on infusion day to provide on-site support The first patients to receive ADMINISTRATION POLICY Preparation and administration etranacogene dezaparvovec-drlb outside of clinical trials were infused INFUSION REACTION PROTOCOL at the Hemophilia Outreach Center Preparation and administration **Clinical Journey in Gene Therapy in a Hemophilia Treatment Center** in Green Bay, Wisconsin, and the University of South Florida in Ionitoring and management Tampa, Elorida MONITORING POLICY post-administration **Patient education** Throughout the clinical journey CORTICOSTEROID TREATMENT IN RESPONSE Monitoring and management post-administration Objective TO ALT ELEVATIONS PROTOCOL Patient screening & **Preparation & administration of** Procurement Monitoring & management Long-term follow-up To describe the process by etranacogene dezaparvovec-drlb eliaibility post-administration which the HTCs prepared Monitoring and management POST-TREATMENT CALENDAR their centers to administer post-administration etranacogene dezanarvovec-drlb la y. , at 🗟 🖓 🚨 🍒 H LONG-TERM MONITORING POLICY Long-term follow-up References I. HEMGENIX. Prescribing Information. CSL Behring LLC. https://labeling.cslbehring.com/ Pi/US/Hemgenix/EN/Hemgenix-Prescribing-Information.pdf. Accessed September 13, 2023. ALT, alanine aminotransferase Patient screening & Delivery Monitoring and Long-term follow-up Preparation eligibility management post-Etranacogene dezaparvovec-drlb The care team practiced preparation and Open communication has been HEMGENIX. Summary of Product Characteristics. CSL Behring LLC. was delivered under cold chain administration prior to treating a patient maintained with patients Patient eligibility and suitability administration **Key Takeaways** was assessed 4-8 weeks before storage · Etranacogene dezaparvovec-drlb was prepared Monitoring and management were Patients were encouraged to https://www.ema.europa.eu/en/documents/ product-information/hemgenix-epar-product-information\_en.pdf.Accessed September 13, 2023. infusion Upon receipt, vials were using aseptic technique on infusion day in participate in the America performed by the core care team Details of the administration inspected and stored in the alignment with LISP 797 Thrombosis & Hemostasis A post-treatment calendar was original carton until use Network (ATHN) TRANSCENDS and follow-up were discussed Administration Multiple stakeholders, including hematologists, advanced 3. HEMGENIX. Product Monograph. CSL Behring shared with patients as a guide to with the patient study for long-term data LLC. https://labeling.cs/behring.ca/ PM/CA/hemgenix/EN/Hemgenix-Product- Diluted product was inspected and administered follow-up visits practitioners, nurses, pharmacists, social workers, and research collection During administration and for 3 hours Patients were provided with the coordinators, are involved in the clinical journey of gene therapy for Monograph.pdf. Accessed February 9, 2024. contact information for the clinical care team in the case of an adverse post-administration, patients were closely hemophilia B Le Quellec S, et al. Poster P129. Presented at: European Society of Gene and Cell Therapy 29th monitored for signs and symptoms of an infusion Early and iterative education, developing center-specific protocols, reaction event Congress in collaboration with British Society for Gene and Cell Therapy; October 11–14, 2022; and applying practical considerations are crucial to ensure smooth Edinburgh, Scotland. execution and optimal patient outcomes when administering 5. American Thrombosis & Hemostasis Network gene therapy at HTCs https://athn.org/what-we-do/national-projects/athn-projects-and-research-studies.html. Accessed September 20, 2023. Nurse Pharmacist Social Key: Hematologist Advanced Long-term patient follow-up and communication across HTCs are Research essential to understanding an individual patient's journey and maintaining optimal hemophilia care

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