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ABSTRACT N. PO12

CHARACTERISATION AND REAL WORD CLINICAL DAT OF HEM006, A NEWLY FORMULATED INTRANASAL SPRAY DESMOPRESSIN (DDAVP) TO ADDRESS A GLOBAL TREATMENT GAP.

G. De Giorgi[1], A. Gringeri[2], R. Franceschini[1], A. Koganti [3].

[1]Hemorare Srl; [2]Fondazione Charta, Milan; [3]Quenya LLC, USA.

Background and Aims: Desmopressin acetate (DDAVP) intranasal spray has been a cornerstone treatment for over three decades in managing bleeding episodes and surgical prophylaxis in patients with von Willebrand disease (VWD) type 1 and non-severe hemophilia A. A recent meta-analysis (>70 studies, 1,770 patients) reported a pooled response rate of 71% (95% CI: 64–78%), with higher efficacy in VWD and some variability across subtypes. Despite its inclusion in the WHO Essential Medicines List for its safety, ease of use, and clinical value, the intranasal formulation (Stimate) was discontinued in 2020, leaving intravenous administration as the only alternative and creating a critical treatment gap. Recent global estimates suggest that over 220,000 bleeding episodes could be prevented annually by DDAVP in type 1 VWD and non-severe hemophilia A patients. New chemical and functional characterization studies, along with real-world clinical data, have been collected to support the development of a newly formulated intranasal DDAVP (HEM006), aimed at addressing the global shortage and restoring access to this essential therapy.

Methods: Following advocacy from patient groups in response to the ongoing shortage, STAQ Pharma—an FDA-registered and inspected outsourcing facility—developed a new intranasal desmopressin spray under Section 503B of the U.S. Federal Food, Drug, and Cosmetic Act. Since September 2021, over 6,400 vials have been distributed in the United States. This formulation was further developed into a fully characterized nasal spray solution (HEM006, Hemorare, Italy), filled using a novel spray device designed to prevent evaporation and avoid risks of over-potency. Pump performance validation and stability studies are ongoing.

Results: HEM006 delivers 150 mcg of desmopressin acetate per spray (1.5 mg/mL, 0.1 mL per actuation), suitable for both pediatric and adult use. It currently has a 12-month shelf life and a 3-month in-use stability.In a previously reported single-center experience involving 261 patients with VWD, the rate of biological response did not significantly differ from historical series treated with Stimate (n = 623). Real-world pharmacovigilance data have confirmed a favorable safety profile. Among 169 desmopressin-related adverse event reports in the FDA database (with VWD or hemophilia A as indications), only one serious event was attributed to HEM006 — a case involving fatigue and decreased appetite in a pediatric patient receiving weekly dosing.

Conclusions: The newly formulated intranasal DDAVP (HEM006) demonstrates a favorable safety and efficacy profile in real-world use. Manufactured under cGMP standards, it offers a practical, non-invasive alternative to intravenous DDAVP and clotting factor concentrates. This formulation holds the potential to restore consistent global access to intranasal desmopressin, reduce reliance on costly and invasive therapies, and address a persistent unmet medical need in bleeding disorders.

Email: *gioacchino.degiorgi@hemorare.com*