August 17, 2023

Medexus Study Supports Efficacy and Safety of IXINITY in Pediatric Patients with Severe or Moderately Severe Hemophilia B

Poster presentation at National Hemophilia Foundation (NHF) Bleeding Disorders Conference 2023 highlights effective prophylaxis, control of bleeding episodes, and consistent safety profile

Phase 3/4 data presentation follows FDA acceptance for review of IXINITY® supplemental Biological License Application for pediatric patients

Toronto, Ontario and Chicago, Illinois--(Newsfile Corp. - August 17, 2023) - Medexus Pharmaceuticals (TSX: MDP) (OTCQX: MEDXF) today announced the presentation of data from a Phase 3/4 study that evaluated the pharmacokinetics (PK), safety, and efficacy of IXINITY, an intravenous recombinant factor IX therapeutic, as a prophylactic treatment in previously treated pediatric patients under 12 years of age with severe or moderately severe hemophilia B, a hereditary bleeding disorder characterized by a deficiency of clotting factor IX in the blood.

In a poster presentation at the National Hemophilia Foundation (NHF) Bleeding Disorders Conference 2023 in National Harbor, Maryland, researchers reported that prophylaxis with IXINITY was associated with low annualized bleeding rates (ABRs), effective control of bleeding episodes, consistent PK, and a consistent safety profile. See "About the study" below for additional information about the study's methods, results, and conclusions. IXINITY is currently approved for use in patients 12 years of age or older with hemophilia B.

"This study demonstrated the efficacy of IXINITY in the prevention and control of bleeding episodes in pediatric subjects while providing a favorable safety profile," commented Johnny Mahlangu, BSc, MBBCh, MMed, FCPath, the study's principal investigator. "The results bolster the overall utility of this recombinant factor IX agent in the treatment of individuals with hemophilia B."

"The data presented at BDC 2023 add to the growing body of evidence of IXINITY's efficacy and safety," said Mark Fosdal, DHSc, PA-C, Director of Scientific Communications at Medexus and a coauthor of the study. "Subject to FDA approval of the pediatric indication, the accumulated data should give clinicians greater confidence in administering IXINITY to maintain reliably high factor IX levels long-term, for all patients of all ages with hemophilia B."

About the study

The Phase 3/4 study was a single-arm, open-label clinical trial evaluating the PK, safety, and efficacy of IXINITY prophylaxis in 21 previously treated patients less than 12 years of age with severe (factor IX activity <1%; n = 13) or moderately severe (factor IX activity 1% to

<2%; n = 8) hemophilia B. The prophylaxis dose was based on IXINITY recovery from PK assessments, after a single intravenous infusion dose of 75 ± 5 IU/kg, and was within the recommended prophylaxis dose of 35-75 IU/kg, twice weekly. The hemostatic efficacy of IXINITY was determined by calculating the ABR while on prophylaxis to prevent bleeding episodes, the number of infusions used to treat bleeding episodes, and overall ratings of efficacy by participants and investigators. Safety was evaluated by assessment of adverse events (AEs), physical examinations, vital signs, and laboratory assessments.

The mean ABR for the entire study was 2.34 ± 4.226 (median = 0.86, range = 0-18.7) for all bleeds and 0.63 ± 1.257 (median = 0, range = 0-4.7) for spontaneous bleeds. One-third (33.3%) of participants had no bleeds and more than sixty percent (61.9%) had zero spontaneous bleeds. Participants rated the hemostatic efficacy of IXINITY as "excellent" or "good" in 78.8% of all episodes. Overall, investigators rated IXINITY prophylaxis as "effective"; there were three ratings of "partially effective" during the study. All investigator ratings for control over bleeding episodes were "effective". Of the bleeding episodes that required treatment, 83.8% resolved after one or two infusions.

There appeared to be no clinically important differences in results for participants younger than 6 years of age compared to those between 6 and 12 years of age. The younger age group experienced lower mean incremental recovery of clotting factor (0.731 vs 0.849 IU/dL:IU/kg) accompanied by a higher mean clearance (7.3 vs 6.1 mL/[kg*hr]), consistent with the PK pattern of factor IX in children aged 12 and younger when compared to use in adolescents and adults.

Overall, 76.2% of participants had at least one AE; the most frequently reported AEs were nasopharyngitis (23.8%) and bronchitis (14.3%). Only one AE was considered possibly treatment-related: a non-serious, moderate hypersensitivity reaction that led to the participant's withdrawal from the study.

About IXINITY®

IXINITY® [coagulation factor IX (recombinant)] Lyophilized Powder for Solution for Intravenous Injection is a coagulation factor IX (recombinant) indicated in adults and children ≥12 years of age with hemophilia B for on-demand treatment and control of bleeding episodes, perioperative management, and for routine prophylaxis to reduce the frequency of bleeding episodes.

The most common adverse reaction observed in >2% of patients in clinical trials was headache. For more information about IXINITY®, including important safety information, see the full prescribing information, which is available on the product's website at: www.ixinity.com.

IXINITY® is approved by the FDA for sale and use in the United States only and is not intended for export outside the United States. Medexus makes no representation that IXINITY® is appropriate for, or authorized for sale to or use by, persons who are not located in the United States.

About hemophilia B

Hemophilia B is a genetic bleeding disorder caused by a deficiency of coagulation factor IX,

a protein needed to produce blood clots to stop bleeding. The clinical spectrum may include spontaneous or trauma-induced bleeding into joints, muscles, and soft tissues, resulting in joint damage, reduction in mobility, and severe arthritis, all of which negatively impact health-related quality of life. The primary aim of care is to prevent and treat bleeding by replacing the deficient clotting factor.

About Medexus

Medexus is a leading specialty pharmaceutical company with a strong North American commercial platform and a growing portfolio of innovative and rare disease treatment solutions. Medexus's current focus is on the therapeutic areas of oncology, hematology, rheumatology, auto-immune diseases, allergy, and dermatology. For more information about Medexus and its product portfolio, please see the company's corporate website at www.medexus.com and its filings on SEDAR+ atwww.sedarplus.ca.

Contacts

Ken d'Entremont | CEO, Medexus Pharmaceuticals
Tel: 905-676-0003 | Email: ken.dentremont@medexus.com

Marcel Konrad | CFO, Medexus Pharmaceuticals
Tel: 312-548-3139 | Email: marcel.konrad@medexus.com

Forward-looking statements

Certain statements made in this news release contain forward-looking information within the meaning of applicable securities laws (forward-looking statements). The words "anticipates", "believes", "expects", "will", "plans", "potential", and similar words, phrases, or expressions are often intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words, phrases, or expressions. Specific forward-looking statements contained in this news release include, but are not limited to, statements regarding the potential benefits of IXINITY®, the occurrence, timing, and expected outcome of the FDA review process for Medexus's supplemental Biological License Application for IXINITY®, and expectations regarding the product's prospects including if approved by the FDA. These statements are based on factors or assumptions that were applied in drawing a conclusion or making a forecast or projection, including assumptions based on historical trends, current conditions, and expected future developments, and, in particular, Medexus's interpretation and assessment of the data presented by Medexus as summarized in this news release. Since forward-looking statements relate to future events and conditions, by their very nature they require making assumptions and involve inherent risks and uncertainties. Medexus cautions that although it is believed that the assumptions are reasonable in the circumstances, these risks and uncertainties give rise to the possibility that actual results may differ materially from the expectations set out in the forward-looking statements. Material risk factors include, but are not limited to, those set out in Medexus's materials filed with the Canadian securities regulatory authorities from time to time, including Medexus's most recent annual information form and management's discussion and analysis. In addition, specific risks and uncertainties relevant to the content of this news release include, among other things, the uncertainties inherent in research initiatives (including the possibility of unfavorable new data and further analyses of existing data); the risk that data are subject to differing interpretations and assessments by regulatory authorities and/or

other relevant third parties; and whether regulatory authorities and/or other relevant third parties will be satisfied with the design and methodology of and results from the relevant study, which will depend on many factors, including determinations as to whether the product's benefits outweigh its known risks. Accordingly, undue reliance should not be placed on these forward-looking statements, which are made only as of the date of this news release. Other than as specifically required by law, Medexus undertakes no obligation to update any forward-looking statements to reflect new information, subsequent or otherwise.

Additional notes

IXINITY®, as discussed in this news release, is a trademark of Medexus. Solely for convenience, trademarks may appear without the "™", "®", or similar symbols. Each such reference should be read as though it appears with the relevant symbol. Any such references are not intended to indicate, in any way, that the holder or holders of the relevant intellectual property rights will not assert those rights to the fullest extent under applicable law.

The information in this news release is provided for informational purposes to investors in Medexus securities.

Medexus sponsored the publication referenced in this news release, and Medexus employees and paid consultants participated in the authoring and editing of the publication.

Uniform resource locators, or website addresses, that appear in this news release are intended to be provided as inactive textual references only. Information contained on or accessible through these website addresses is not a part of this news release and is not incorporated by reference into this news release or any of Medexus's public filings.



To view the source version of this press release, please visit https://www.newsfilecorp.com/release/177667

SOURCE Medexus Pharmaceuticals Inc.