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Treosulfan NDA Resubmitted to FDA

FDA decision expected within six months of acceptance
Pivotal phase 3 clinical trial of treosulfan met primary endpoint and key secondary endpoints

TORONTO and CHICAGO, July 25, 2022 (GLOBE NEWSWIRE) -- Medexus Pharmaceuticals (**Medexus**) (TSX: MDP) (OTCQX: MEDXF) and medac, a strategic partner of Medexus, are pleased to announce that medac has resubmitted its New Drug Application for treosulfan (**NDA**) with the U.S. Food and Drug Administration (**FDA**). The current submission was a response to the FDA request to submit information to complete medac's April 2022 NDA resubmission and initiate FDA review.

The NDA requests approval of treosulfan in combination with fludarabine as a preparative regimen for allogeneic hematopoietic stem cell transplantation (**allo-HSCT**). The current submission includes updates to data files and supporting information in response to the FDA's information request received with the FDA's acknowledgment of receipt of the NDA resubmission in May 2022. If the response is considered complete by the FDA, the review clock for the NDA resubmission will then start as of the date of submission of a complete response.

"We remain excited about the prospect of a treosulfan approval in the United States and about treosulfan's significant potential in the U.S. market," commented Ken d'Entremont, Medexus's Chief Executive Officer. "We are encouraged by the recent publication of the final study results and analysis of the pivotal phase 3 clinical trial of treosulfan conducted by medac, which met its primary endpoint and key secondary endpoints. An FDA approval within a two- to six-month period from the acceptance date would then pave the way for a commercial launch of treosulfan in the United States in the first half of calendar year 2023."

Mr d'Entremont continued: "If approved by the FDA, we expect that treosulfan would have a meaningful impact on Medexus's total revenue. We estimate that the current market-leading product in the United States generated approximately $126 million in peak annual revenue before genericization."

"The experience we've had [with treosulfan] has been outstanding so far," commented Dr Filippo Milano, a physician-scientist, in a June 6, 2022 interview hosted by Medexus. "I would really like to have this drug available, not just for me, but for all my colleagues." The full interview is available on the News & Media – Media section of Medexus's corporate website.

About treosulfan

Treosulfan is part of a preparative regimen for allo-HSCT to be used in combination with fludarabine, used in treating eligible patients with acute myeloid leukemia and myelodysplastic syndromes.

Final study results and analysis of the pivotal phase 3 clinical trial of treosulfan conducted by medac, which was accepted for publication with American Journal of Hematology, concluded that the study demonstrates clinically relevant superiority of treosulfan over a widely applied "reduced-intensity conditioning" busulfan regimen with regard to its primary endpoint, event-free survival. The publication also includes favorable conclusions on two key secondary endpoints, finding that overall survival with treosulfan was superior compared to busulfan and that non-relapse mortality for patients in the treosulfan arm was lower than for patients in the busulfan arm. For more information about the study and the publication, including a link to the full publication, see Medexus's June 6, 2022 press release, available on the Investors—News & Events section of Medexus's corporate website.

Treosulfan was approved by Health Canada in June 2021, and Medexus commercially launched treosulfan in Canada under the brand name Trecondyv® in September 2021. Treosulfan is currently the subject of a regulatory review process with the U.S. Food and Drug Administration.

About Medexus

Medexus is a leader in innovative rare disease treatment solutions with a strong North American commercial platform and a portfolio of proven best-in-class products. Our current focus is on the therapeutic areas of hematology, auto-immune diseases, and allergy. We continue to build a highly differentiated company with a growing portfolio of innovative and high-value orphan and rare disease products that will underpin our growth for the next decade.

Our current leading products are Rasuvo™ and Metoject®, a unique formulation of methotrexate (auto-pen and pre-filled syringe) designed to treat rheumatoid arthritis and other auto-immune diseases; IXINITY®, an intravenous recombinant factor IX therapeutic for use in patients 12 years of age or older with Hemophilia B (a hereditary bleeding disorder characterized by a deficiency of clotting factor IX in the blood, which is necessary to control bleeding); and Rupall®, an innovative prescription allergy medication with a unique mode of action. We also hold exclusive US and Canadian rights to commercialize Gleolan (aminolevulinic acid hydrochloride or ALA HCl), an FDA-approved, orphan drug designated optical imaging agent currently indicated in patients with glioma (suspected World Health Organization Grades III or IV on preoperative imaging) as an adjunct for the visualization of malignant tissue during surgery.

We have also licensed Treosulfan, a preparative regimen for allogeneic hematopoietic stem cell transplantation to be used in combination with fludarabine, for commercialization in the United States and Canada. Treosulfan was approved by Health Canada in June 2021 and is marketed in Canada as Trecondyv®. Treosulfan is currently under review by the U.S. Food and Drug Administration.

Our mission is to provide the best healthcare products to healthcare professionals and patients. We strive to deliver on this mission by acting on our core values: Quality, Innovation, Customer Service, and Collaboration.

Contacts

For more information, please contact any of the following:

Medexus

Ken d'Entremont, Chief Executive Officer
Medexus Pharmaceuticals Inc.
Tel: 905-676-0003
Email: ken.dentremont@medexus.com

Marcel Konrad, Chief Financial Officer
Medexus Pharmaceuticals Inc.
Tel: 312-548-3139
Email: marcel.konrad@medexus.com

Investor Relations

Victoria Rutherford
Adelaide Capital
Tel: 1-480-625-5772
Email: victoria@adcap.ca

Forward-Looking Statements

Certain statements made in this press 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 or expressions are often intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Specific forward-looking statements contained in this press release include, but are not limited to, statements regarding the timing and expected outcome of the FDA approval process for Treosulfan and a related launch of the product in the United States and expectations regarding the product's prospects 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. 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 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; future capital requirements and dilution; intellectual property protection and infringement risks; competition (including potential for generic competition); reliance on key management personnel; Medexus's ability to implement its business plan; Medexus's ability to leverage its U.S. and Canadian infrastructure to promote additional growth; regulatory approval by relevant health authorities, including the FDA; product reimbursement by third party payers; litigation or expiry with respect to patents or other intellectual property rights; litigation risk; stock price volatility; government regulation; and potential third party claims. Given these risks, undue reliance should not be placed on these forward-looking statements, which are made only as of the date hereof. Other than as specifically required by law, Medexus undertakes no obligation to update any forward-looking statements to reflect new information, subsequent

or otherwise.



Source: Medexus Pharmaceuticals Inc