June 6, 2022

Treosulfan Pivotal Study Results Published

Study's primary endpoint and key secondary endpoints were met

Study found event-free survival and overall survival superior after treosulfan compared to RIC busulfan, also found non-relapse mortality lower in treosulfan arm than busulfan arm

Study results accepted for publication with American Journal of Hematology, now available online

Medexus to host webconference with key opinion leader to discuss data on June 6, 2022 at 4:00 PM Eastern Time

TORONTO and CHICAGO, June 06, 2022 (GLOBE NEWSWIRE) -- Medexus Pharmaceuticals (**Medexus**) (TSX: MDP) (OTCQX: MEDXF) today announced that the American Journal of Hematology has accepted for publication the final study results and analysis of the pivotal phase 3 clinical trial of treosulfan conducted by medac, a strategic partner of Medexus. The publication has undergone full peer review and has already been published online.

The open-label, multicenter, randomized parallel study was designed to compare event-free survival (**EFS**) after treosulfan-based conditioning with a widely applied reduced-intensity conditioning (**RIC**) busulfan regimen in older or comorbid patients with acute myeloid leukemia (**AML**) or myelodysplastic syndrome (**MDS**) undergoing allogeneic hematopoietic stem cell transplantation (**allo-HSCT**). The primary endpoint of the study was EFS of patients with disease recurrence, graft failure, or death from any cause as events. Secondary endpoints were overall survival, cumulative incidence of relapse or progression, cumulative incidence of graft failure, and non-relapse mortality. The study was performed in 31 clinical institutions across five European countries and enrolled 570 patients between June 2013 and December 2016.

The publication concludes that the study demonstrates clinically relevant superiority of treosulfan over RIC busulfan with regard to its primary endpoint, EFS. The study found that EFS of patients in the study (median age 60 years) was superior after treosulfan compared to RIC busulfan. 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. The authors conclude that a treosulfan regimen appears particularly suitable for older AML and MDS patients.

Frequencies of treatment-emergent adverse events (**AEs**) of all grades and serious AEs were equally distributed between the study arms. Further, frequencies of patients with treatment-emergent serious AEs categorized by organ class and term were comparably low

and no unknown safety risks were identified.

See “About the study” below for additional information, including a summary of statistical information, about the study’s findings. The full publication, which includes further discussion of the study’s design and findings, is available at the following link:

https://doi.org/10.1002/ajh.26620

Medexus invites investors and other interested parties to view and listen to a live webcast at **4:00 pm Eastern Time** on **Monday, June 6, 2022**, with a physician-scientist whose research has focused on treosulfan, who will discuss the results of the study.

To participate in the live webcast, please visit the Investors—News & Events section of Medexus’s corporate website or join using the following link:

https://us02web.zoom.us/webinar/register/WN_LVZwcCa0QECzcThDPBJuxw

FDA review of treosulfan NDA

As previously announced on May 24, 2022, medac’s new drug application for treosulfan is currently the subject of an ongoing regulatory review process with the U.S. Food and Drug Administration (**FDA**). medac is preparing the updated data files and supporting information requested by the FDA in its May 2022 letter, and medac continues to expect to respond to the FDA’s information requests within the 12-month timeline required by the FDA’s July 2021 Complete Response Letter. Medexus will continue to provide additional information as it becomes available.

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.

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 the study

The phase 3 study was designed to compare EFS after treosulfan-based conditioning with a widely applied RIC busulfan regimen in older or comorbid patients with AML or MDS undergoing allo-HSCT. This publication presents the final study results and pre-specified subgroup analyses of all 570 randomized patients with completed longer term follow-up.

Patients presenting allo-HSCT-specific comorbidity index > 2 or aged ≥ 50 years were randomly assigned (1:1) to intravenous (**IV**) fludarabine with either treosulfan (30 g/m² IV) or busulfan (6.4 mg/kg IV) after stratification by disease risk group, donor type, and participating institution. The primary endpoint was EFS with disease recurrence, graft failure, or death from any cause as events.

EFS of patients (median age 60 years) was superior after treosulfan compared to RIC busulfan: 36-months-EFS rate 59.5% (95% CI, 52.2 to 66.1) vs 49.7% (95% CI, 43.3 to 55.7) with a hazard ratio (**HR**) of 0.64 (95% CI, 0.49 to 0.84), P = 0.0006. Likewise, overall survival (**OS**) with treosulfan was superior compared to busulfan: 36-months-OS rate 66.8% vs 56.3%; HR 0.64 (95% CI, 0.48 to 0.87), P = 0.0037. Post hoc analyses revealed that these differences were consistent with the confirmatory interim analysis, and thereby the treosulfan regimen appears particularly suitable for older AML and MDS patients.

The 36-month NRM for patients in the treosulfan arm (14.2% [95% CI, 9.5 to 18.9]) was lower than for patients in the busulfan arm (21.0% [95% CI, 16.1 to 26.0]) corresponding to a significantly reduced HR (HR 0.63 [95% CI, 0.41 to 0.97] adjusted Fine and Gray-model P value = 0.0343). No adverse influence of increasing numbers of aggregated allo-HSCT-CI categories compared with absence of any comorbidity on NRM was detectable by multivariate analysis. Thus, comorbidities apparently did not impact NRM or its difference between study arms.

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, part of 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 the subject of a regulatory review process with 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, and statements made in the webcast discussed in this press release may 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 potential benefits of treosulfan and the expected timing for submission of information to the FDA. Specific forward-looking statements that may be contained in the webcast referred to in this press release may include, but are not limited to, statements regarding the potential benefits of treosulfan and the timing and expected outcome of the FDA approval process for treosulfan, including submission of information to the FDA and the FDA's acceptance and review of that information. Since forward-looking statements relate to future events and conditions, by their very nature they require making assumptions and involve inherent risks and uncertainties. Relevant risks and uncertainties include, among other things, the uncertainties inherent in research and development, including the ability to meet anticipated clinical endpoints, commencement and/or completion dates for clinical trials, regulatory submission dates, regulatory approval dates and/or launch dates, as well as the possibility of unfavorable new clinical data and further analyses of existing clinical data; the risk that clinical trial data are subject to differing interpretations and assessments by regulatory authorities; whether regulatory authorities will be satisfied with the design of and results from clinical studies; whether and when drug applications may be filed in a given market for the relevant product candidate; whether and when any such applications may be approved by regulatory authorities, which will depend on many factors, including making a determination as to whether the product candidate's benefits outweigh its known risks and determination of the product candidate's efficacy and, if approved, whether the product will be commercially successful; decisions by regulatory authorities impacting labeling, manufacturing processes, safety, and/or other matters that could affect the availability or commercial potential of the product; and competitive developments. A further description of material risk factors can be

found 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. Given these risks, undue reliance should not be placed on these forward-looking statements, which are made only as of the date of this press release or, in the case of the webcast discussed in this press release, the date of the webcast. 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 note

Uniform resource locators, or website addresses, that appear in this press 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 press release and is not incorporated by reference into this press release or any of Medexus's public filings.

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