EBMT
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PRESS RELEASE

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EBMT trial shows improvements in treatment of Severe Aplastic Anaemia

Barcelona, Monday, August 31, 2020 - The European Society for Blood and Marrow Transplantation (EBMT), Europe's collaborative peer network of professionals working in the field of stem cell transplantation and cellular therapy, announced today the results of the phase III RACE trial during EBMTs virtual 46th Annual Meeting. Preliminary data show that adding Eltrombopag to standard immunosuppressive treatment is safe and increases response rates in patients with Severe Aplastic Anaemia (SAA).

SAA is a condition in which the bone marrow does not produce enough new blood cells. It is a rare, yet potentially fatal disease which can be treated with bone marrow transplantation or, for patients who are not eligible to receive a transplantation, with immunosuppressive treatment. The most commonly used immunosuppressive regimen includes horse ATG (hATG) in combination with Cyclosporine A (CsA). However, about 35% of patients do not respond to treatment or eventually relapse.

Eltrombopag is a thrombopoietin receptor agonist that was developed to stimulate thrombopoiesis (production of platelets), but it was subsequently shown to restore trilineage haematopoiesis. A previous single-arm study showed that adding Eltrombopag to standard immunosuppressive treatment appeared to improve the response rate as compared to the use of hATG plus CsA alone. The first results of the randomised controlled RACE trial now confirm that adding Eltrombopag to standard immunosuppression leads to significantly higher response rates compared to standard immunosuppressive treatment alone.

The RACE trial is sponsored by the EBMT with the financial support of Novartis and Pfizer. Professors. Régis Peffault de Latour (Head of the French Reference Center for Aplastic Anemia and PNH, Saint-Louis Hospital, and University of Paris) and Antonio M. Risitano (Federico II University, Naples, and Head of Hematology and the BMT Unit, Ospedale Moscati, Avellino, Italy) served as Principal Investigators of the study, and they designed the study together with Professor Carlo Dufour (Head of the Hemato Oncology and Stem Cell Transplantation Department. G.Gaslini Childrens' Research Hospital, Genova, Italy). Under the energetic and efficient coordination of Sofie Terwel, the trial was successfully conducted by the RACE study team at the EBMT Clinical Trial Office. The study was presented by Prof. Peffault de Latour at the presidential symposium of EBMT's virtual Annual Meeting and was granted the Van Bekkum Award, the most prestigious EBMT award for the best abstract submitted to the physician's programme.

The international, investigator-driven, open-label, phase III, randomised trial evaluated 197 patients with SAA. Patients were aged 15 years or older, had acquired SAA, and had not received prior immunosuppressive treatment. Patients were randomised to receive either standard immunosuppression (hATG 40 mg/kg x4d and CsA 5 mg/kg/d) or standard immunosuppression + Eltrombopag at the dose of 150 mg/d from day +14 until 6 months (or 3 months, in case of early complete response). The primary endpoint of the study is complete response (CR) at 3 months, with CR being defined as haemoglobin 100 g/L, neutrophils 1.0 g/L and platelets 100g/L, according to standard international criteria.

It was shown that three months after treatment start, patients who received the combination of hATG, CsA plus Eltrombopag had a significantly higher complete response rate compared to patients treated with hATG and CsA alone. These higher response rates were sustained at 6 months. Moreover, Eltrombopag was generally well-tolerated, with a comparable occurrence of adverse events in the two treatment arms. This

trial also shows that in this rare disease, large randomised trials can successfully be run in collaboration with many expert centres in Europe.

“Eltrombopag is registered in Europe for second line treatment of aplastic anaemia, so it is only available to patients who cannot receive bone marrow transplantation and have failed immunosuppressive treatment” explains Prof. Peffault de Latour. Prof. Risitano states: “The RACE trial data shows that eltrombopag increased response rates for naïve SAA patients who are not eligible for hematopoietic stem cell transplantation. The RACE study team is continuing to follow up the trial participants up to two years and furthermore aims to set up a long-term follow-up study to monitor the effectiveness and safety of Eltrombopag up to ten years.”. “The EBMT Clinical Trial Office is already actively working on this new project, which likely will provide the final evidence about the benefit of using triple therapy as initial treatment for Severe Aplastic Anemia.” concludes Prof. Dufour.

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About the EBMT

The European Society for Blood and Marrow Transplantation (EBMT) is a not-for-profit medical and scientific organisation established in 1974. It is dedicated to fighting life-threatening blood cancers and diseases and improving patients’ lives with more than 5,000 physicians, nurses, scientists and other healthcare professionals - participating in a unique collaborative network of peers involved in haematopoietic stem cell transplantation (HSCT) and cellular therapy research.

The EBMT patient Registry is the only data source of its kind in Europe. The data are submitted continuously by more than 500 centres and holds data on more than 700,000 HSCT procedures. It contains data on patients who have undergone HSCT, immunosuppressive therapies or cell or gene therapies. The Registry underpins extensive European research that translates into changes in clinical practice and improvements in patient outcome and care.

For further information about the EBMT, please visit the website: https://www.ebmt.org and follow us on Twitter: @TheEBMT

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