EUSA Pharma and Apeiron Biologics receive positive CHMP opinion for dinutuximab beta for the treatment of high-risk neuroblastoma in Europe

HEMEL HEMPSTEAD, England and VIENNA, Austria – 27 March 2017 – EUSA Pharma (EUSA), a specialty pharmaceutical company with a focus on oncology and oncology supportive care, and Apeiron Biologics today announced that the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) has adopted a positive opinion recommending the approval of dinutuximab beta for use in the treatment of high-risk neuroblastoma[1].

The marketing authorisation application included data developed from multiple clinical trials across Europe that included over 1,000 patients receiving dinutuximab beta. The clinical development was led by the SIOPEN academic neuroblastoma group and supported by Apeiron Biologics[2]. EUSA Pharma holds the exclusive global rights to dinutuximab beta.

***Lee Morley**, **EUSA Pharma's Chief Executive Officer**, said, "This positive CHMP opinion is an important milestone for EUSA as we work to bring dinutuximab beta to children suffering from the high risk form of the devastating disease, neuroblastoma. This cancer is responsible for up to 10% of childhood tumors, and with treatment options limited we are working hard to make this life saving therapy available to children around the world. Following this positive opinion in Europe, we plan to submit dinutuximab beta for approval in the United States in the coming year."*

***Dr. Hans Loibner**, **Apeiron Biologic's Chief Executive Officer**, said, "We are delighted with the CHMP positive opinion for dinutuximab beta, which follows our extensive development work with a number of partners, in particular the SIOPEN group. Dinutuximab beta represents an important potential treatment in an area of significant unmet need, and we look forward to working with EUSA to make this product available around the world."*

Dinutuximab beta is currently used in Europe under a managed access program as part of treatment regimens for high-risk neuroblastoma. Following the CHMP positive opinion, the European Commission will now issue a formal decision on approval, and if approved dinutuximab beta will be indicated for use in the 28 countries of the European Union in children aged 12 months and above, who have previously received induction chemotherapy and achieved at least a partial response, followed by myeloablative therapy and stem cell transplantation, as well as patients with history of relapsed or refractory neuroblastoma, with or without residual disease. In patients with a history of relapsed/refractory disease and in patients who have not achieved a complete response after first line therapy, dinutuximab beta should be combined with interleukin-2 (IL-2). Prior to the treatment of relapsed neuroblastoma, any actively progressing disease should be stabilised by other suitable measures.

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About dinutuximab beta and neuroblastoma

Neuroblastoma is an orphan oncology condition with significant unmet medical need. It accounts for up to 10% of childhood tumors and affects approximately 1,200 children in Europe each year. Dinutuximab beta is currently used extensively across Europe under a managed access scheme and is included in a number of treatment protocols for high-risk neuroblastoma.

Dinutuximab beta is an anti-GD2 monoclonal antibody that significantly improves event-free and overall survival in children with high-risk neuroblastoma, with a favorable safety profile compared to other immunotherapies. Dinutuximab beta forms an important part of treatment regimens for high-risk neuroblastoma and dinutuximab beta's novel features offer the potential for further development to expand its current role. Dinutuximab beta has orphan drug designation in the US and EU, and EUSA plans to file the product for approval in the United States in 2017.

About EUSA Pharma
Founded in March 2015, EUSA Pharma is a specialty pharmaceutical company with commercial operations in the US and Europe, and a wider distribution network in approximately 40 further countries. Currently, EUSA has a broad portfolio of approved and named-patient specialty hospital products, which the company has ambitious plans to expand through acquisition and in-licensing. EUSA is led by an experienced management team with a strong record of building successful specialty pharmaceutical companies, and is supported by significant funding raised from leading life science investor Essex Woodlands.

In addition to dinutuximab beta, EUSA Pharma's products include: Caphosol® for the treatment of oral mucositis, a common and debilitating side-effect of radiation therapy and high-dose chemotherapy; Collatamp®, a gentamicin-collagen implant licensed either in hemostasis or for the prevention and treatment of surgical site infection; Custodiol® solution for use in the preservation of organs for transplantation; Fomepizole® for the treatment of ethylene glycol poisoning; and Xenazine® for the treatment of abnormal movements associated with Huntington's chorea and hemiballismus. EUSA also has exclusive European, Latin American, North and South African and selected Middle East and Asian rights to tivozanib, which is currently under review by EMA for the treatment of advanced renal cell carcinoma. For more information visit www.eusapharma.com.

About Apeiron Biologics
Apeiron is a private biotechnology company based in Vienna, Austria, developing immunological therapies against cancer. Its most advanced product, dinutuximab beta, is licensed to EUSA Pharma, and is a chimeric monoclonal antibody against the GD2 antigen expressed on neuroblastoma and other tumors. In addition, Apeiron's portfolio consists of three further immune-oncology projects, of which two are clinical stage. Apeiron has a broad development program, which focuses on therapies that selectively boost the immune system via unique and novel checkpoint blockade mechanisms to fight cancer. For more information visit www.apeiron-biologics.com.

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2. www.apeiron-biologics.com.