PRESS RELEASE

apceth Biopharma presents positive data demonstrating efficacy of apceth-201 in preclinial models of acute GvHD

Munich, Germany, Oct 19, 2018 - apceth Biopharma GmbH, a company with the mission of improving patients' lives with next generation cell therapies, announced today positive results for apceth-201 in mouse models for acute graft-*versus*-host disease (aGvHD). The data was highlighted in a poster presentation at the Annual Congress of the European Society of Gene and Cell Therapy in Lausanne on October 16-19.

"We are very excited with these results that clearly show the transformative therapeutic potential of apceth-201 in an immune-mediated disease with high unmet medical need and few treatment options", said Dr Christine Guenther, CEO of apceth Biopharma. "Based on these promising and impressive data, we are currently preparing to initiate a phase 1/2 clinical study assessing the safety and efficacy of apceth-201 in adult steroid-refractory GvHD patients." Acute graft-*versus*-host disease is a frequent complication associated with allogeneic hematopoietic stem cell transplantation (HSCT). Immunosuppressants are used to manage aGvHD; however, steroid-refractory aGvHD develops in many cases and has an extremely poor prognosis.

apceth-201 significantly improved clinical score and overall survival in two aGvHD models

apceth-201 are allogeneic human mesenchymal stromal cells (MSCs) that have been genetically modified to express alpha-1 antitrypsin (AAT), a protease inhibitor exerting potent anti-inflammatory and tissue-protective functions to further augment the immunomodulatory potential of MSCs. *In vitro* assays demonstrated that apceth-201 efficiently suppresses T-cell proliferation, the maturation of dendritic cells, and also polarizes macrophages to an anti-inflammatory M2 type. The *in vivo* efficacy of apceth-201 was assessed in two different mouse models for aGvHD. In a humanized model vehicle-treated control animals succumbed quickly to GvHD, whereas median survival was doubled in apceth-201-treated animals. Mice receiving apceth-201 showed significantly improved clinical scores, a striking amelioration of bone marrow cellularity, and reduced levels of inflammatory markers. apceth-201 was further tested in a GvHD model system which closely mimics haploidentical HSCT. Vehicle-treated control animals again succumbed quickly to GvHD, whereas treatment with apceth-201 resulted in long-term survival of 57 % of the mice. Initially, all apceth-201-treated animals exhibited clinical scores comparable to the control animals. However, within a period of 25 days after the second cell injection, the clinical scores had returned to baseline, indicating complete resolution of GvHD. This promising data has led to planning of a phase 1/2 study using apceth-201 for the treatment of steroid-refractory aGvHD in adults.

About apceth Biopharma GmbH

apceth Biopharma is a pioneering company in cell therapies and regenerative medicine with an innovative portfolio of drug candidates for the treatment of inflammation, autoimmunity and solid tumors. apceth is developing two proprietary next generation gene therapy products, which are based on the introduction of therapeutic transgenes into mesenchymal stem cells (MSCs). apceth-201 expresses the immunomodulatory protein alpha-1 antitrypsin for the treatment of graft-versus-

host disease. The company's second program, apceth-301, expresses a potent immunostimulatory "cocktail" of cytokines, which locally activates the immune system to eradicate tumor cells. apceth-301 is currently being developed for glioblastoma as well as for other solid tumors.

apceth Biopharma is also a leading and certified Contract Development and Manufacturing Organization for cell and gene therapeutics with a broad international customer base. apceth owns state-of-the-art manufacturing facilities with Grade B/A, C and D cleanrooms (ISO 5, ISO 7, ISO 8, BSL2) and is certified according to regulatory requirements for cell and gene therapies (Advanced Therapy Medicinal Products, ATMPs). The company has a comprehensive expertise in GMP manufacturing of autologous and allogeneic cell types that are either native or genetically modified. It has long-standing experience with various cell products, including mesenchymal stem cells (MSCs), hematopoietic stem cells (HSC), lymphocytes, monocytes, dendritic cells, cord blood derived stem cells, and has the potential to expand to CAR-T and induced pluripotent stem cell (iPSC) technologies. apceth has successfully obtained manufacturing licenses for multiple cell therapy products for clinical as well as commercial use. apceth's CDMO team provides fully customized solutions in the development and production of every customer product and process. Located centrally in the heart of Europe, apceth can perform efficient and fast supply for patients all over the continent.

Contact

apceth Biopharma GmbH
Dr Christine Guenther, CEO
Max-Lebsche-Platz 30
81377 Munich
Germany

Phone: +49 (0)89 7009608 0
Email: contact@apceth.com
www.apceth.com