# Press Release

## Apogenix Receives FDA Orphan Drug Designation for Apocept™ to Treat Myelodysplastic Syndromes and Initiates Clinical Phase I Study

**Heidelberg, Germany, February 7, 2013** – Apogenix GmbH, a clinical stage biopharmaceutical company developing novel protein therapeutics for the treatment of cancer and inflammatory diseases, announced today that its lead product, Apocept™ (APG101), has been granted orphan drug designation from the US Food and Drug Administration (FDA) for the treatment of Myelodysplastic syndromes (MDS). MDS are clonal hematopoietic stem cell disorders characterized by ineffective hematopoiesis leading to blood cytopenias, especially anemia.

Simultaneously, Apogenix announced the initiation of a clinical Phase I trial with its lead compound Apocept™ in patients with MDS. The clinical trial is designed as an open-label study and is conducted in clinical centers throughout Germany. Recruitment of MDS patients for the study began in January 2013. Endpoints of the study include efficacy (improvement of erythropoiesis), safety, and tolerability parameters. Results of the trial are expected by mid-2014.

Apocept™ binds to the CD95 ligand (CD95L) and blocks the activation of the CD95 receptor. Excessive stimulation of the CD95 receptor on hematopoietic cells present in the bone marrow of MDS patients inhibits erythropoiesis. As a result, transfusion-dependent anemia develops, which is mostly refractory to erythropoiesis-stimulating agents. Preclinical studies using hematopoietic stem cells obtained from MDS patients show that Apocept™ dose-dependently stimulates erythropoiesis and thus may help treat anemia.

Dr. Harald Fricke, COO/CMO of Apogenix, commented: "MDS is the second indication for which Apocept™ received orphan designation in the US. With its novel mode of action, Apocept™ restores the causal impairment of erythropoiesis in MDS. After the successful proof of concept in a randomized controlled trial in glioblastoma demonstrating excellent efficacy of Apocept™ both in prolonging progression-free survival as well as overall survival, we are confident that the success story of Apocept™ will continue, with MDS representing the second field of application."

**About Apogenix**

Apogenix, a spin-out from the German Cancer Research Center (DKFZ), is developing novel protein therapeutics for the treatment of cancer and inflammatory diseases based either on the targeted modulation of apoptosis (programmed cell death) or on blocking the growth of tumor cells. The company's lead product candidate Apocept™ (APG101) is being developed for the treatment of glioblastoma, the most common and aggressive type of primary brain tumor. Since its inception in 2005, the company has raised more than €50 million with dievini Hopp BioTech Holding GmbH & Co. KG as main investor, and has been awarded public grants totaling over €8 million. Apogenix is based in Heidelberg, Germany.

**About Apocept™ (APG101)**

The company's lead product candidate, Apocept™, a first-in-class, fully human fusion protein combining the extracellular domain of the CD95 receptor and the Fc portion of IgG, successfully completed a Phase I study in 2009. In December 2009, Apogenix started a controlled Phase II trial with the compound for the treatment of recurrent glioblastoma. The patient recruitment for this study was completed in September 2011. The primary endpoint as well as a number of secondary endpoints of the trial were successfully reached in 2012. Apogenix was granted orphan drug designation for Apocept™ in 2009 for the treatment of glioblastoma in Europe and in the US.

**About Myelodysplastic Syndrome**

Myelodysplastic syndromes (MDS) are clonal hematopoietic stem cell disorders characterized by ineffective hematopoiesis leading to blood cytopenias, especially anemia. The median age for MDS is higher than 60 years. The incidence rate for MDS is about 4/10,000 per year and increases to 20-50/10,000 above the age of 70. The disease is often diagnosed during routine check-ups. MDS patients suffer from a reduced red blood cell count (anemia), feel tired, and are prone to infections. In most cases, this anemia is treated with blood transfusions resulting in an iron overload, which can damage the liver, for example. At the same time, the number of thrombocytes (blood platelets, responsible for coagulation) and leucocytes (white blood cells, responsible for immune defense) is decreasing. As a result, MDS patients frequently suffer from infections and bleedings, which can prove fatal.
For more information about MDS, please visit www.marrow.org.

**Contact**

Dr. Thomas Höger
CEO/CFO
Apogenix GmbH
D-69120 Heidelberg, Germany
Phone: +49 (6221) 5 86 08-0
Email: contact@apogenix.com
www.apogenix.com

**Media Contacts**

U.S.A.
Martina Schwarzkopf, Ph.D.
Russo Partners
Phone: + 1 (212) 845 4292
martina.schwarzkopf@russopartnersllc.com

Europe
Raimund Gabriel
MC Services AG
Phone: +49 (89) 210 228 30
raimund.gabriel@mc-services.eu