PRESS RELEASE

AAA receives orphan drug designation from FDA and EMA for Gallium-68 DOTATATE for use in patients with Gastro-Entero-Pancreatic Neuroendocrine Tumors

04 March 2014, Saint-Genis-Pouilly, France – Advanced Accelerator Applications (AAA), a fast growing international player in Molecular Nuclear Medicine (MNM), announced today that they have received orphan drug designation status for their radiopharmaceutical, Gallium-68 DOTATATE. The orphan drug designation has been granted by both the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) for use of Gallium-68 DOTATATE as a diagnostic agent for the management of Gastro-Entero-Pancreatic Neuroendocrine Tumors (GEP-NETs). The designation should foster rapid development of the agent for the benefit of GEP-NET patients in the U.S. and Europe.

Gallium-68 DOTATATE is a radiopharmaceutical used in PET/CT imaging of GEP-NETs. The product will be prepared using AAA’s patented kit, which is reconstituted in hospital radiopharmacies without the use of a radiochemistry module, thus making the product available to all hospitals, even those who do not have a fully equipped GMP production radiopharmacy unit.

Existing data show that the Gallium-68-labeled PET radiopharmaceutical should represent a major improvement compared to the current standard. Available data indicates that Gallium-68 DOTATATE not only has greater sensitivity and specificity for tumor detection than the current standard, but it is also expected to significantly reduce radiation doses received by patients.

Stefano Buono, Chief Executive Officer of AAA, commented: “GEP-NETs constitute a life-threatening disease and effective patient management requires accurate diagnostic tools. The orphan drug designation of AAA’s Gallium-68 DOTATATE will accelerate the development of this agent and hopefully allow it to be available to patients in the next few years.”

"Receiving orphan drug designation for Gallium-68 DOTATATE is an important step in the overall approval process. It reinforces our position and interest in NETs, both on the diagnostic and treatment front. Strong literature evidence already exists about the efficacy of Gallium-68 DOTATATE, which we believe reduces our development risks for this product," added AAA’s Chief Operating Officer Gérard Ber.

The EMA's orphan medicinal product designation is designed to promote the development of drugs that may provide significant benefit to patients suffering from rare, life-threatening diseases. In addition to ten years of market exclusivity, the orphan drug designation also provides special incentives for sponsors including eligibility for protocol assistance and possible exemptions or reductions in certain regulatory fees during development or at the time of application for marketing approval.

Similarly, FDA orphan drug designation is intended to encourage companies to develop
therapies for the treatment of diseases that affect fewer than 200,000 individuals in the U.S. This designation could provide AAA with seven years of marketing exclusivity for AAA’s Gallium-68 DOTATATE as a diagnostic agent for the management of GEP-NETs if it receives first approval by the FDA. Prior to FDA approval, orphan designation by the FDA provides the opportunity to obtain grant funding to help finance costs of clinical trial expenses, tax credits for clinical research expenses and potential waiver of the FDA’s application user fees.

**About Gallium-68 DOTATATE kit**

Gallium-68 DOTATATE or Gallium [Ga-68]-N-[(4,7,10-Tricarboxymethyl-1,4,7,10-tetraazacyclododec-1-yl)acetyl]-D-phenylalanyl-L-cysteinyl-L-tyrosyl-D-tryptophyl-L-lysyl-L-threoninyl-L-cysteinyl-L-threonine-cyclic(2-7)disulfide is a diagnostic PET imaging agent currently under development. The pre-formulated GMP kit is used to prepare the final product, which is a ready-to-inject 68-Ga-somatostatin analogue peptide solution.

Gallium-68 DOTATATE is a radiopharmaceutical agent used to help diagnose Gastro-Entero-Pancreatic Neuroendocrine cancers via PET/CT imaging. Existing data show that AAA’s Ga-68-labeled GEP-NET PET radiopharmaceutical should represent a major improvement compared to the existing standard.

**About Gastro-Entero-Pancreatic Neuroendocrine Tumors**

Gastro-Entero-Pancreatic Neuroendocrine Tumors (GEP-NETs) are a rare group of tumors that start in the cells of the neuroendocrine system, a network of endocrine glands and cells throughout the body. GEP-NETs develop in the digestive system, usually starting in the stomach, bowel or pancreas and constitute a life-threatening disease.

**About Molecular Nuclear Medicine (MNM)**

Molecular Nuclear Medicine is a medical specialty which uses trace amounts of active substances, called radiopharmaceuticals, to create images of organs and lesions and to treat various diseases, like cancer. The technique works by injecting into the patient’s body targeted radiopharmaceuticals that accumulate in the organs or lesions that reveal specific biochemical processes.

Molecular Nuclear Diagnostics employ a variety of imaging devices and radiopharmaceuticals. PET (Positron Emission Tomography) and SPECT (Single Photon Emission Tomography) are highly sensitive imaging technologies that enable physicians to diagnose different types of cancer, cardiovascular diseases, neurological disorders and other diseases in their early stages.

PET (Positron Emission Tomography) is a MNM imaging technique used in diagnosis and biomedical research. In PET, a chemical compound labeled with a short-lived positron-emitting radionuclide of carbon, oxygen, nitrogen, or fluorine is injected into the body. The activity of such a radiopharmaceutical is quantitatively measured throughout the target organs. Data are analyzed and reconstructed by means of a computer to produce images of the organs being scanned.
About Advanced Accelerator Applications

Advanced Accelerator Applications (AAA) is a European pharmaceutical company founded in 2002 to develop innovative diagnostic and therapeutic products. AAA’s main focus is in the field of Molecular Imaging and targeted, individualized therapy for the management of patients with serious conditions (Personalized Medicine). AAA currently has 17 production and R&D facilities able to manufacture both diagnostics and therapeutic MNM products, and has over 290 employees in 11 countries (France, Italy, UK, Germany, Switzerland, Spain, Poland, Portugal, Israel, U.S., Canada). In 2013 AAA is expecting to reach revenues of €56.6 million (+27% vs. 2012) and EBITDA of €14 million (+49% vs. 2012). For more information please visit: www.adacap.com

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