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Press release

Action Pharma's phase IIb clinical trial with AP214 to prevent kidney injury associated with major cardiac surgery demonstrates positive top-line results

Aarhus, Denmark, September 26, 2011

Action Pharma A/S today announced positive top-line results for its phase IIb AP214 trial in patients undergoing major cardiac surgery. The study demonstrated that AP214 is safe, reduces kidney injury, and improves long-term (90 days) outcomes on a composite end point (death, dialyses and kidney function) in patients undergoing cardiac surgery with cardiopulmonary bypass who are at increased risk of developing acute kidney injury. In addition, this study validates and expands on previous positive AP214 safety and efficacy data from a phase IIa trial.

The clinical phase IIb trial is randomized, multi-center, double-blind, placebo-controlled with two dose levels of AP214 administered during surgery and early in the postoperative period. The trial studied 77 patients undergoing cardiac surgery across 8 sites, 3 in Denmark and 5 in the US (study conducted by CTI Clinical Trial and Consulting Services, Cincinnati, Ohio, the USA, as clinical CRO).

A top-line efficacy analysis indicates that AP214 resulted in:

- Statistically significant improvement in clinical outcomes based on a 90 day composite endpoint (death, dialyses, and kidney function) compared to placebo – a possible endpoint for registration trials based on the Company's previous discussions with the FDA
- Statistically significant improvement in GFR (glomerular filtration rate) at day 90 compared to baseline
- Lower incidence of acute kidney injury (AKI) within 48 hours (AKIN score) or 7 days (RIFLE score)
- Lower serum creatinine levels at day 7

Furthermore, AP214 was safe and well-tolerated comparable to placebo. The clinical results are expected to be presented as late breaking clinical abstract at the annual meeting of the ASN (American Society of Nephrology) to be held on November 8-13 in Philadelphia, PA, USA.

"These positive phase IIb clinical data are potentially very good news for patients", says Ingelise Saunders, CEO of Action Pharma. She continues, "based on the positive results we will now accelerate our discussions with major pharmaceutical companies focused on this group of high risk patients."

"The investors and Board of Directors of Action Pharma are very excited by the positive top-line results from the phase IIb clinical trial with AP214. This is a key milestone for the Company and the investors look forward to continuing the support of Action Pharma as it strives to make this potential new drug available to patients", says Sten Verland of Sunstone Capital, and Chairman of the Board of Directors of Action Pharma.

Professor Daniel Steinbrüchel, Department of Cardiac and Thoracic Surgery, Danish State Hospital, Copenhagen, and lead investigator for the trial, adds: "There exists a serious unmet medical need as well as a significant interest both from clinical leadership and regulatory bodies, including the FDA, for the development of new potent pharmaceuticals in this area. A therapeutic that addresses these needs, and is also applicable to the extensive related circumstances in which acute kidney injury can be a major cause of morbidity and mortality, would be a highly significant improvement in patient care."

Action Pharma estimates that the potential market for renal protection in cardiac surgery exceeds EUR 500 million in the U.S. alone with considerable expansion potential in follow-on indications throughout cardiac and vascular surgery, as well as interventional cardiovascular procedures as the closest related areas. Acute kidney injury results in increased mortality, co-morbidity and prolonged hospitalization, and at present, there are no current effective preventive or therapeutic interventions available to address this serious complication of numerous clinical settings. Action Pharma has the opportunity to be first to market addressing a high unmet need with a novel therapeutic that has demonstrated safety and efficacy in Phase II trials.

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About Action Pharma A/S

Action Pharma is a privately owned Danish biotech company. Action Pharma develops novel drug candidates targeting melanocortin receptors and bring these to the stage of clinical proof of concept for subsequent partnering. The drug candidates are the first in several new drug classes and exploit a novel mode of action profiles with an efficacy that is superior compared to compounds currently on the market. The Action Pharma team has significant scientific expertise and has published more than 400 scientific papers.

AP214 is being developed to prevent post-surgical kidney injury after major thoracic surgery. AP214 has recently completed a phase IIb clinical trial evaluating the efficacy, safety and tolerability of AP214 in preventing kidney injury and systemic inflammatory response in patients undergoing cardiac surgery, who are at increased risk of kidney injury. Every year, more than 500,000 patients in the USA and in the EU undergo major thoracic surgery. Approximately 10-20% of these patients experience various degrees of kidney injury, which again is associated with a marked increase in mortality, co-morbidity and prolonged hospitalization. Currently, there is no treatment to prevent or treat kidney injury associated with major thoracic surgery. Thus, there is a major unmet medical need. AP214 mediates its potent effect via the type 1 and type 3 melanocortin receptors. Results from phase IIa and IIb clinical trials in the US and EU, showed encouraging efficacy, safety and tolerability data for AP214.

Action Pharma has a strong investor base of leading European investors, including Sunstone Capital, Global Life Science Ventures, SLS Invest, InnovationsKapital, Incuba Venture, and Oestjysk Innovation. For more information, please visit www.actionpharma.com