AM-Pharma announces positive Phase I data on recAP for Acute Kidney Injury

Trial shows recAP to be safe and well tolerated at maximum dose and ready for Phase II development

Bunnik, The Netherlands, 26 March 2014. AM-Pharma B.V., a biopharmaceutical company focused on the development of recAP (recombinant human Alkaline Phosphatase) for inflammatory indications, announces the results of its Phase I trial with both single and multiple ascending doses, which demonstrate that recAP is safe and well tolerated at all doses.

In total 50 healthy male and female volunteers were included in the randomised, double-blind, placebo-controlled, first-in-human, single and multiple dose escalation study, to investigate safety, tolerability, and pharmacokinetics of recAP administered intravenously. Volunteers in the single dose groups were administered one of four single doses, and those in the multiple dose groups were administered one of two multiple doses on three consecutive days. No safety issues were observed in any of the dose groups.

The trial also established recAP's pharmacokinetic properties. These include dose exposures up to and above the target therapeutic range as determined in AM-Pharma's earlier positive Phase II clinical trial results with bovine Alkaline Phosphatase (bovine AP), in the treatment of patients with Acute Kidney Injury (AKI).

"Our wealth of knowledge around Alkaline Phosphatase, gained from our earlier Phase II programmes with bovine AP, meant that at the start of this clinical trial we expected that recAP would be safe and well tolerated," said Erik van den Berg, CEO of AM-Pharma. "It is nevertheless very encouraging to see that recAP is showing favourable pharmacokinetics, and should therefore provide superior tissue distribution and extended half-life beyond that seen with bovine AP. With this confirmation, alongside the safety profile, we are confident that recAP is a promising candidate to show therapeutic efficacy in its Phase II development."

AM-Pharma is currently finalising the Phase II trial protocol, for recAP to treat AKI, which will take advantage of "Adaptive Trial Design" – an increasingly adopted, and regulatory-endorsed, methodology that allows predefined modifications and expansion of a trial based on initial study results. Adaptive Trial Design increases the chances to obtain statistically relevant data to validate clinical products, and thus shorten product development times.

Media contact:

80th Atom
Adam Michael
+44 1223 511338
+44 777 588 1813
Adam@80thAtom.com

Company contact:

AM-Pharma
Erik van den Berg (CEO)
+31 30 259 8838

Notes for Editors

About AM-Pharma www.am-pharma.com
AM-Pharma is a biopharmaceutical company focused on the preclinical and clinical development of Alkaline Phosphatase as protective treatment of acute kidney injury and inflammatory bowel diseases. AM-Pharma is based in Bunnik, The Netherlands. Based on the strong results of the Phase II trials with bovine Alkaline Phosphatase in Acute Kidney Injury and a Phase II trial in Ulcerative Colitis – a form of inflammatory bowel disease – AM-Pharma developed an innovative recombinant form of human Alkaline Phosphatase. This recombinant Alkaline Phosphatase will be used in future trials and for commercialization. AM-Pharma raised €29.2M in Q4 2011, enabling AM-Pharma to finalize the GMP production and the development through phase II.

About Acute Kidney Injury
Acute Kidney Injury (AKI) involves an inflammatory process in the kidney which can lead to complete loss of renal function. Hospital-acquired AKI affects annually around 2 million patients in Europe, US and Japan, of which around 700,000 patients die. It occurs in as many as 4% of hospital admissions and 40% of critical care admissions. Depending on the severity and cause of renal injury, mortality ranges from 10% to as high as 70%. In the US alone, around USD10 billion is spent each year on managing this big medical problem. The most important causes of AKI are sepsis, cardiovascular surgery, exposure to nephrotoxic drugs and trauma. AKI patients that need dialysis have the worst prognosis. Currently the only treatment option is dialysis and supportive care. No drugs are approved to treat this condition. Typically these patients are treated in Intensive Care, often with support of nephrologists. Due to the large number of patients suffering from AKI, the high medical need, worldwide annual sales of over USD2 billion could be achieved with an effective drug treatment.

About Alkaline Phosphatase
Alkaline Phosphatase (AP) is an enzyme that is naturally present in humans on epithelial cells of the gastrointestinal tract, kidney, liver and lungs. An important role of AP is the dephosphorylation of proinflammatory substances like lipopolysacharides (LPS) and extra-cellular ATP. The anti-inflammatory characteristics of AP was firstly published by Professor Poelstra and his group at Groningen University, the Netherlands. AM-Pharma has since shown that treatment with exogenous AP not only reduces local and systemic inflammation but also protects the kidney against further damage.

About recAP
AM-Pharma's therapeutic candidate, recAP (recombinant Alkaline Phosphatase), is a proprietary recombinant human AP constructed from two naturally occurring human isoforms of the AP enzyme. This hybrid is highly stable and active, and has been optimised for treating inflammatory conditions. It is being developed as an injectable for the treatment of Acute Kidney Injury and an oral formulation for Ulcerative Colitis. The enzyme is being produced by cGMP manufacture for preclinical and clinical trial supply and commercialisation.

– ENDS –