JAMA publishes Phase II recAP data for sepsis-associated Acute Kidney Injury

JAMA paper to be presented at ESICM conference in Paris
New data on mode of action to be presented at ASN conference in San Diego

Bunnik, The Netherlands, October 24th 2018. AM-Pharma B.V., a biopharmaceutical company focused on the development of recombinant human Alkaline Phosphatase (recAP) for inflammatory diseases, today publishes data in the prestigious *Journal of the American Medical Association* (JAMA) of its recently completed STOP-AKI Phase II study of recAP in the treatment of sepsis-associated Acute Kidney Injury (AKI). Simultaneously, the results will be presented at today's European Society of Intensive Care Medicine in Paris. Additionally, new data on recAP's mode of action will be presented tomorrow (October 25th) at the American Society of Nephrology Kidney Week in San Diego.

Professor Peter Pickkers, MD PhD, Chair of Experimental Intensive Care Medicine, Radboud University Medical Center, and principal investigator of the STOP-AKI study said: "Although the study did not meet its primary endpoint of short-term improvement in kidney function in the first 7 days, it did show long-term improvement in kidney function and very importantly a 40% relative reduction in mortality over placebo. In the absence of any drugs approved for this condition, these exciting clinical outcomes warrant further research to confirm these findings and to make this treatment available to patients."

Prof. Pickkers is presenting the results of the randomized clinical study at today's Hot Topics session at ESICM in Paris at 16:40 CET.

In addition, investigations at the institutions of Professor Mark D. Okusa and Professor Bruce Molitoris revealed more detail into recAP's mode of action. Their research showed that protection from kidney injury is mediated by dephosphorylation by recAP of ATP to adenosine and activation of adenosine A2a receptors (A2aR).

Dr. Okusa, Professor of Medicine and Chief, Division of Nephrology and Director, Center for Immunity, Inflammation and Regenerative Medicine at University of Virginia, School of Medicine, and Dr. Molitoris, Professor of Medicine, former Director of Nephrology and the Indiana Center for Biological Microscopy at Indiana University noted: "The mode of action appears to be an elegant, double effect; in the first instance recAP inactivates pro-inflammatory ATP, and the resulting formation of adenosine further reduces inflammation through the immunosuppressive adenosine A2a receptor pathway."

Professor Diane L. Rosin from Dr. Okusa's research group will present the results at the annual meeting of the American Society of Nephrology in San Diego; Session Title: *AKI: New Players and New Mechanisms*, October 25th from 18:00 PT (session room 6D).

Erik van den Berg, CEO of AM-Pharma said: "We are excited to share the clinical trial results in JAMA and scientific conferences. We are now preparing for the pivotal study to confirm the STOP-AKI study results, which could have a significant impact on patients with Acute Kidney Injury, for whom there is currently no treatment available."

Previously, the US Food and Drug Administration granted Fast Track designation for recAP for treatment of sepsis-associated AKI. AKI involves inflammatory processes in the kidney which can lead to complete loss of renal function and is associated with high mortality rates.

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Notes to Editors

About AM-Pharma www.am-pharma.com
AM-Pharma is a biopharmaceutical company focused on the development of recAP (recombinant Human Alkaline Phosphatase) for treatment of Acute Kidney Injury (AKI), Ulcerative Colitis (UC), Necrotizing Enterocolitis (NEC) and Hypophosphatasia (HPP). Based on strong results from Phase II trials with bovine Alkaline Phosphatase in AKI and UC, AM-Pharma has developed an innovative recombinant form of human Alkaline Phosphatase (recAP), and recently completed recruitment of 301 patients in the STOP-AKI adaptive Phase II trial for sepsis-associated AKI.

About Acute Kidney Injury
Acute Kidney Injury (AKI) involves inflammatory processes in the kidney which can lead to complete loss of renal function. Hospital-acquired AKI affects annually around 3 million patients in Europe, the US and Japan, and is associated with mortality in roughly 700,000 patients. 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, hospitals spend around $10 billion each year on managing this major medical problem. The most important causes of AKI are sepsis, cardiovascular surgery, exposure to nephrotoxic drugs and trauma. AKI patients that require dialysis have the worst prognosis. Currently the only treatment options are dialysis and supportive care. No drugs are approved to treat this condition. Typically, these patients are treated in Intensive Care Units, often with guidance by nephrologists.[2,3,4]

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, which is highly stable and active.

About STOP-AKI study
The trial, titled A Safety, Tolerability, Efficacy and QoL Study of Human recAP in the Treatment of Patients With SA-AKI (STOP-AKI), s an adaptive Phase II trial with two parts. In the first part, data from 120 patients were evaluated to select the most effective dose of recAP. In the second part, an additional 170 patients were recruited into two arms, receiving either the optimal dose of recAP identified initially, or placebo. The study recruited 301 patients in total, which makes it the largest interventional clinical study iwan AKI to date. The study was conducted in more than 50 intensive care units (ICU) in Western Europe and North America. (click here for full details on the protocol).

[1] U.S. Food and Drug Administration; available at http://www.fda.gov/ForPatients/Approvals/Fast/ucm405399.htm
[2] Murugan R. and Kellum J.A., (2011) Nat Rev Nephrol. Vol 7: 209-217
[3] Heung M. and Chawla L., (2014) Nephron Clin Pract. Vol 127: 30-34
[4] Chertow et al., (2005) J Am Soc Nephrol. Vol 16: 3365-3370 Soc Nephrol. Vol 16: 3365-3370

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