AM-Pharma STOP-AKI Phase II trial nominated for “Most Innovative Clinical Trial Design” in 2016 CARE Awards

Tuesday, April 19, 2016 - 08:00am

Bunnik, The Netherlands, 19 April 2016. AM-Pharma B.V., a biopharmaceutical company focused on the development of recAP (recombinant human Alkaline Phosphatase) has been shortlisted for the “Most Innovative Clinical Trial Design” in the inaugural Clinical & Research Excellence Awards in Boston, organised by Informa Business Intelligence (IBI).

The nomination is based on AM-Pharma's two-part adaptive Phase II clinical trial design of recAP in patients with sepsis-associated Acute Kidney Injury. Part one of this STOP-AKI trial is a dose escalation study in 120 patients to identify the optimal dose. Part two of the trial is a proof of concept study in 170 patients to provide early efficacy data on the selected optimal dose from part one.

The adaptive trial design, running parts 1 and 2 consecutively, shortens the Phase II development time, compared to the more traditional, separate, Phase IIa and Phase IIb trials. Furthermore, data from the patients that received the optimal dose in part 1 can also be included in the final statistical analysis. The combination of data from part 1 and 2 enables the trial to recruit sufficient patients, to provide statistical significance, sooner.

According to IBI, the entries have been reviewed to produce a shortlist that displays the wealth of innovation, dedication and hard work that the pharmaceutical and biotech industries have demonstrated over the past year.

“We are delighted to have made the shortlist for these new CARE Awards,” said Erik van den Berg, CEO of AM-Pharma. “We have worked very closely with clinical experts and the

regulators to create this STOPAKI trial protocol. The potential to reduce development times not only has commercial benefits, but could have a significant impact on patients with Acute Kidney Injury, for which there is currently no treatment available.

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About AM-Pharma www.am-pharma.com AM-Pharma is a biopharmaceutical company focused on the preclinical and clinical development of recAP (recombinant Human Alkaline Phosphatase) as a treatment of Acute Kidney Injury (AKI), Ulcerative Colitis (UC), and Hypophosphatasia (HPP). Based on strong results from Phase II trials with bovine Alkaline Phosphatase in AKI and UC, AM-Pharma developed an innovative recombinant form of human Alkaline Phosphatase (recAP), which is currently in Phase II development for sepsis-associated AKI. In May 2015, AM-Pharma signed a deal with Pfizer, which made an upfront payment of $87.5 million for a minority equity interest, and exclusive option to acquire the Company, with additional potential payments of up to $512.5 million upon option exercise and potential launch of any product that may result from the agreement.

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 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 nephrologists123.

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. It is in Phase II development for the potential treatment of AKI, with the potential to be developed for HPP. An oral formulation has been developed for the treatment of UC. The enzyme is produced by cGMP manufacture for preclinical and clinical trial supply and

commercialization.

1 Murugan R. and Kellum J.A., (2011) Nat Rev Nephrol. Vol 7: 209-217 2 Heung M. and Chawla L., (2014) Nephron Clin Pract. Vol 127: 30-34 3 Chertow et al., (2005) J Am Soc Nephrol. Vol 16: 3365-3370 Soc Nephrol. Vol 16: 3365-3370 –

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