Advicenne Announces Positive Pivotal Phase III Clinical Data for ADV7103 in Adults and Children Suffering from distal Renal Tubular Acidosis (dRTA)

ADV7103 could potentially be the first approved treatment for dRTA

Nîmes, France, 11th September, 2017 – Advicenne, a late-stage biopharmaceutical company focused on the development of paediatric-friendly therapeutics for the treatment of orphan renal and neurological diseases, announces positive top-line data from a pivotal phase III study assessing ADV7103 in adults and children suffering from distal Renal Tubular Acidosis (dRTA). dTRA is a disease characterised by an unbalanced pH in the body associated with additional disorders such as biochemical impairments that may result in failure to thrive, rickets/osteomalacia, lithiasis and nephrocalcinosis that can lead to renal failure.

The objective of the study was to evaluate the efficacy, safety and acceptability of ADV7103, an innovative oral product, formulated in paediatric-friendly coated granules, that combines two active pharmaceutical ingredients. ADV7103 is given twice a day in contrast to the current standard of care (SoC), which are usually various unapproved products administered every four to six hours to attempt to re-balance the body's pH and to normalise blood potassium level (kalaemia).

The Phase III study of ADV7103 was shown to restore the main biological defects observed with the disease meeting positively primary and secondary endpoints.

Normal blood bicarbonate levels were attained in most patients treated with doses of ADV7103 ranging from 0.75 to 8.45 mEq/kg/day. Mean doses of 1.7, 2.3, 3.8 and 6.1 mEq/kg/day ADV7103 were given, respectively, in adults, adolescents, children, and infants. Non-inferiority of ADV7103 vs. SoC or baseline literature data was consistently demonstrated (per protocol, intention-to-treat, as well as sensitivity analyses). Kalaemia was normalised with ADV7103 with only two doses per day.

These analyses were able to show that ADV7103 is superior to the SoC ($p<0.0047$).
Further important advantages of ADV7103 which were demonstrated in the study were its good palatability, ease of administration and gastrointestinal tolerability.

The data from this pivotal phase III study was presented at the 50th European Society of Paediatric Nephrology (ESPN) 2017 Annual Meeting which was held from the 7th to 9th of September in Glasgow, UK, during an oral communication on Saturday, 9th September.

Dr Luc-André Granier, CEO and cofounder of Advicenne, commented, "We are delighted to announce the positive results from this pivotal phase III study which clearly show that ADV7103 has the potential to provide a meaningful and durable benefit to patients with dRTA. The success we have achieved with ADV7103 reflects our ability to leverage our scientific knowledge, network of key opinion leaders, formulation expertise and unwavering commitment to patients to deliver paediatric friendly therapeutics for the treatment of orphan renal diseases for which there are currently no approved treatment options."

Advicenne also sponsored a scientific Symposium event dedicated to dRTA on 8th September at ESPN.

The European Commission granted orphan drug designation (ODD) to ADV7103 for the treatment of dRTA in June 2017.

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About distal Renal Tubular Acidosis (dRTA)

dRTA is a disease that occurs when the kidneys do not properly remove acids from the blood into the urine. As a result, too much acid remains in the blood which generates an unbalanced pH in the blood that can generate failure to thrive and rickets (a condition that affects bone development in children) as well as a range of additional clinical disorders such as a potassium deficiency (hypokalaemia) in the blood serum which alters the function of several organs and most prominently affects the cardiovascular system; and a high concentration of calcium in the blood and urine (hypercalcemia and hypercalciuria respectively) which can lead to kidney stones and calcinosis that can potentially cause renal impairment, ultimately leading to renal failure. The disease, either genetic (usually occurring during childhood) or acquired as a result of autoimmune disease, is estimated to affect 30 000 to 50 000 patients in Europe.

About Advicenne

Advicenne is a late-stage biopharmaceutical company focusing on the development of paediatric friendly therapeutics for the treatment of orphan renal and neurological diseases. The Company's most advanced product is ADV7103 which has shown positive results in a pivotal phase III study in children and adults with distal Renal Acidosis (dTRA). In addition to ADV7103, the Company has a portfolio of products targeting critical unmet needs in nephrology and neurology. Advicenne also develops a clinical and pre-clinical pipeline of potential treatments for additional orphan diseases in collaboration with Key Opinion Leaders.

The Company was established in 2007 in Nimes (France). Since its inception, the Company has raised close to €30 million in equity from leading venture capital investors Innobio (Bpifrance), IXO Private Equity, IRDI SORIDEC Gestion, Cemag Invest and MI Care.

Additional information about Advicenne is available through its website:
http://advicenne.com/en/

Disclaimer
This press release contains information regarding clinical development of ADV7103.