Advicenne Announces Positive 6-Month Extension Study Data from Pivotal Phase III study of ADV7103 in Adults and Children Suffering from distal Renal Tubular Acidosis (dRTA)

- *Data presented at the American Society of Nephrology (ASN) meeting in New Orleans*
- *Positive data will form basis for application seeking market authorization of ADV7103 to the European Medicines Agency in Europe for dRTA expected in 2018*
- *Clinicians are expecting registration of the product for the treatment of dRTA*

Nîmes, France, **6 November 2017** – Advicenne, a late-stage pharmaceutical company focused on the development of pediatric-friendly therapeutics for the treatment of orphan renal and neurological diseases, announces positive 6 months follow-up data from the pivotal phase III study (B22CS) assessing ADV7103 in adults and children suffering from 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 preliminary results of the 6-month follow up study assessed the safety and efficacy of twice daily dosing of ADV7103 for the treatment of dRTA in both adult and pediatric patients. The extension study (B22CS) followed the pivotal phase III trial (B21CS), which showed ADV7103's ability to restore the main biological defects observed with the disease, meeting primary and secondary endpoints. The product's efficacy, was shown to be maintained at 6 months in this open label extension study, with blood bicarbonate levels above 21 mM - the normal level - in 79% of the patients. Individual ADV7103 doses ranged from 1.3 to 7.2 mEq/kg/day.

Overall, patients and/or their parents were extremely satisfied with ADV7103. This was measured using a visual analogue scale (VAS) questionnaire quoting from 0 (no improvement at all) to 100% (extremely important improvement). The change of treatment from standard of care to ADV7103 allowed an average improvement of the patients' quality of life of 80.5%; depending on the age group considered the improvement ranged from 76 to 98%.

ADV7103 is the company's lead product and has been designed to address the disease both in adults and in children. The disease in children is generally from genetic origin while adults mostly develop dRTA as a result of autoimmune disease. Positive Phase III results for ADV7103 were announced in September this year and demonstrated ADV7103's ability to normalise the main biological defects observed with the disease.

Dr Luc-André Granier, CEO and cofounder of Advicenne, commented, "The 6-month follow up data presented at ASN are very encouraging as they reinforce the clear benefits that our lead product ADV7103 delivers. These data, together with the recent positive phase III results with ADV7103, which were presented at the European Society for Paediatric Nephrology (ESPN) in September, highlight the potential of ADV7103 to become the first treatment for dRTA, a renal orphan disease with high unmet medical needs."

Dr Granier added, "Our strong links to key opinion leaders in nephrology, alongside our development expertise and scientific knowledge have been key to the successful clinical development of ADV7103. The Advicenne team's focus has been driven by their unwavering commitment to deliver pediatric-friendly therapeutics to patients for the treatment of orphan renal diseases for which there are currently no approved treatment options."

The poster entitled "*Safety and efficacy of ADV7103, an innovative prolonged-release oral alkalising combination product, after 6-months treatment in distal renal tubular acidosis (dRTA) patients*" was presented at ASN on 2nd November 2017. The abstract can be accessed here.

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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 that can lead to 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. In addition, a high concentration of calcium in the blood and urine (hypercalcemia and hypercalciuria) 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 approximately 30,000 patients in Europe and 20,000 in the US. Current standard of care 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.

About Advicenne

Advicenne is a late-stage pharmaceutical company focusing on the development of pediatric-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 Tubular Acidosis (dRTA). ADV7103 is also being developed in a second indication for the treatment of Cystinuria, an inherited renal tubulopathy and is expected to enter into a pivotal Phase II/III clinical trial in 2018 in Europe.

In addition to ADV7103, the Company has a portfolio of clinical and pre-clinical products targeting critical unmet needs in nephrology and neurology 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: www.advicenne.com

Disclaimer

This press release contains information regarding clinical development of ADV7103.