Advicenne Announces First Patient Enrolled in US ARENA-2 Pivotal Phase III Clinical Trial Evaluating ADV7103 in distal Renal Tubular Acidosis (dRTA)

Nîmes, France, August 29, 2019 (5:45 p.m. CEST) - Advicenne (Euronext Paris & Brussels: ADVIC - FR0013296746), a specialty pharmaceutical company dedicated to developing and commercializing innovative treatments for those suffering from rare kidney disease, today announced the enrollment of the first patient in its ARENA-2 study, currently being conducted in the United States. The pivotal Phase III clinical trial evaluates Advicenne's lead drug candidate, ADV7103, in the treatment of distal Renal Tubular Acidosis (dRTA).

Authorized in both the United States and Canada, the ARENA-2 trial is expected to include approximately 40 patients. 8 study centers are already open and results are expected in the beginning of 2021. They will be followed by the filing of an application for registration in the United States, with commercialization there anticipated as early as 2021. The present study was initiated following a European ARENA-1 clinical trial that demonstrated positive results after 24 months of treatment.

Professor Larry Greenbaum, Director of Pediatric Nephrology at Emory University in Atlanta and President of the American Society of Pediatric Nephrology (ASPN), commented: *"We are delighted about the start of this study in the United States, where no standard treatment for dRTA currently exists, despite its severe clinical implications."*

"*The enrollment of the first patient in the United States is in line with our wish to make our product internationally available to patients suffering from dRTA,"* **added Dr. Luc-André Granier, Co-Founder and CEO of Advicenne,** "*Based on our strong results in Europe, we are confident about the outcome of this North American study."*

Advicenne has also filed an application for orphan drug designation (ODD) for ADV7103 in the United States.

About ARENA-2

ARENA-2 is a pivotal Phase III, prospective, multicenter, randomized, double-blind, placebo-controlled clinical study that is expected to include approximately 40 patients in the United States and Canada. Its principal objective is to evaluate the safety and efficacy of ADV7103 versus placebo in preventing the development of metabolic acidosis in infant (6 months to 18 years of age) and adult (18 to 65 years of age) subjects with primary dRTA.

About Distal Renal Tubular Acidosis (dRTA)

dRTA is an orphan disease characterized by a failure in the renal excretion of the acids generated through metabolism. The excess of acids thus accumulated in the blood leads to an imbalance in pH (acidosis) as well as multiple other complications. These include growth retardation and rickets (a disease affecting bone development) in children, and a series of metabolic disorders such as low potassium levels (hypokalemia), high calcium levels (hypercalcemia), elevated calcium in the urine (hypercalciuria) resulting in kidney stones, the formation of calcium deposits in the kidneys (calcinosis) as well as possible kidney failure.

Whether genetic, or acquired as a consequence of an autoimmune disease, dRTA affects an estimated 30,000 patients in Europe and approximately 20,000 in the United States.

About Advicenne

Advicenne (Euronext ADVIC) is dedicated to developing and commercializing innovative treatments for those suffering from rare kidney disease. Our lead drug candidate is currently in late-stage clinical trials for two indications.

In 2017, ADV7103 was granted orphan drug designation by the European Commission in the treatment of distal renal tubular acidosis (dRTA), a rare kidney disorder that occurs when the kidneys are unable to effectively remove the buildup of circulating acids in the blood. ADV7103 is currently in Phase III clinical trials for this indication in Europe, the United States and Canada, and a marketing application for candidate has been submitted for centralized European review.

While we prepare its European commercial launch for dRTA, we are simultaneously conducting trials for ADV7103 in the treatment of cystinuria, a genetic disease characterized by a buildup of the amino acid cystine in the kidneys and bladder. ADV7103's Phase II/III European clinical trials for this second indication were recently expanded to Belgium.

At Advicenne, we are committed to innovating in the areas of formulation and dosage. Tasteless and easy to administer, our products are commercialized in small-size formats that offer flexible, personalized dosing – because path-breaking treatments for rare diseases should be available to patients of all ages.

Headquartered in Nîmes, France, Advicenne is listed on the Euronext Paris stock exchange since 2017 and cross-listed on the Euronext Brussels stock exchange in 2019.

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Forward-Looking Statements

This press release contains certain forward-looking statements relating to the business of Advicenne, which shall not be considered per se as historical facts. Such statements include projections and estimates, and the hypotheses on which these are based, as well as observations relating to operations, ongoing projects, objectives, the development of products and their future performance, and expectations regarding financial results.
In some cases, forward-looking statements can be identified by words such as "could," "should," "may," "expects," "anticipates," "believes," "intends," "estimates," "aims," "targets" or similar words. Although the management of Advicenne believes that these forward-looking statements are reasonably made, investors should be aware that they are subject to a number of known and unknown risks and uncertainties and other factors that may cause actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by these forward-looking statements. In particular, the expectations of Advicenne could be affected by, among other things, uncertainties involved in the placing on the market and commercialization of Advicenne products or any other risks and uncertainties developed or identified in any public documents filed by Advicenne with the French Financial Markets Authority (*Autorité des marchés financiers* (AMF)), including those listed in Chapter 4, "Risk Factors," of its reference document, filed with the latter on December 3, 2018, under number R.18-073, and in Section 8 of its annual financial report published on April 30, 2019. Notwithstanding the compliance with article 223-1 of the General Regulation of the AMF (the information disclosed must be "accurate, precise and fairly presented"), Advicenne disclaims any intention or obligation to publicly update or revise any forward-looking statements, whether as a result of new information, future events or otherwise.
Any information relating to the use of drug candidates contained in the present press release is based on the results of ongoing studies at the time of the release's publication. A drug candidate is product that has not yet received marketing authorization from a health agency.