Advicenne's Lead Candidate Approved for Orphan Drug Designation in the Treatment of Cystinuria in Europe

Nîmes, France, December 18, 2019 - 5:45 CET - Advicenne (Euronext : ADVIC - FR0013296746), a specialty pharmaceutical company focused on the development and commercialization of therapeutic products for rare kidney disease, today announced that its lead drug candidate, ADV7103, has been granted orphan drug designation by the European Medicines Agency (EMA) for the treatment of cystinuria in Europe.

"The EMA's positive opinion regarding orphan drug designation for ADV7103 is of significant strategic importance to the development of this product," **comments Dr. Luc-André Granier, Co-Founder and CEO of Advicenne.** "This designation entails important advantages relating to the clinical development of ADV7103, as well as direct access to the centralized procedure for marketing authorization and 10-year marketing exclusivity following authorization."

Orphan drug designation is granted to medicines in development for the treatment, prevention and diagnosis of serious or debilitating illnesses whose prevalence does not exceed 5 out of 10,000 people in the European Union and for which there exists no satisfactory solutions or alternative treatments.

Cystinuria is a genetic disease characterized by a buildup of the amino acid cystine in the kidneys and bladder, leading to the formation of cystine stones. These cystine stones generate various complications, such as hypertension, intense abdominal pain, recurrent urinary tract infections, renal function impairment . These complications can ultimately lead to kidney failure.

The prevalence of cystinuria in Europe is estimated at 1 out 10,000, or around 70,000 patients affected. A pivotal Phase II/III CORAL study is currently underway in order to evaluate the efficacy, safety, tolerance and compliance of ADV7103 in patients suffering from cystinuria in Europe.

In 2017, ADV7103 was granted orphan drug designation 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 has already demonstrated its efficacy and safety in the treatment of dRTA through Phase III clinical trials in Europe and is currently in Phase III trials for this indication in the United States and Canada.

What is Orphan Drug Designation?

Orphan drug designation is a status intended to encourage the development of medicines for the treatment of rare diseases. It offers a range of strategic incentives including European marketing exclusivity following marketing authorization in the EU. In addition, orphan drug designation can include subsidies and tax credits, clinical protocol assistance and reductions in regulatory fees during development, and even registration of the product.

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 the drug candidate has been submitted for centralized European review.

While we prepare the 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 has been listed on the Euronext Paris stock exchange since 2017 and was 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 a product that has not yet received marketing authorization from a health agency.