Advicenne confirms prevalence of distal renal tubular acidosis (dRTA) and cystinuria at ISPOR conference

Nîmes, France, June 13, 2019 (6:45 p.m. CEST) - Advicenne (Euronext: ADVIC - FR0013296746), a pharmaceutical company specializing in the development of adult and pediatric therapeutic products for the treatment of orphan diseases, today announced the presentation of three posters at the 2019 conference of the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) in New Orleans (United States). ISPOR is an international organization that brings together experts and professionals in health economics and research.

The three published posters presented studies conducted with the participation of the Advicenne teams in order to estimate the prevalence of distal renal tubular acidosis (dRTA) and cystinuria, two rare pathologies which the company's lead drug candidate, ADV7103, has been developed to treat. The studies included patient data from the United Kingdom and the United States for primary dRTA. They also made it possible to analyze the use of medical resources, outcomes and other data relating to patients suffering from these pathologies.

Presentation titles:

- **Distal renal tubular acidosis, a rare renal condition: prevalence UK study using CPRD database**

Distal renal tubular acidosis is a serious orphan disease characterized by a lack of renal excretion of metabolically generated acids. The excess acid thus accumulated in the blood can lead to multiple complications, including stunting of growth and rickets in children, as well as a range of metabolic issues that can lead to kidney stones, calcinosis and kidney failure. The data presented made it possible to estimate the prevalence of dRTA in the United Kingdom at 0.46 to 1.6 per 10,000 people.

- **Estimate of prevalence of primary distal renal tubular acidosis among the US population with employer-sponsored health insurance**

Primary dRTA is caused by genetic abnormalities that lead to metabolic acidosis due to insufficient acid excretion by the kidneys. In addition to these complications, primary dRTA can lead to deafness in some patients. Taking into account the proportion of primary dRTA to the totality of cases of dRTA (117/1084, or about 1%, according to Marketscan), the number of affected people in the United States who are also covered by employer-sponsored health insurance plans is estimated at 665, or a prevalence of 0.38 for every 100,000.

- **Cystinuria, a rare renal condition that is often undiagnosed: prevalence UK study using CPRD database**

Cystinuria is a rare genetic disease characterized by a buildup of the amino acid cystine in the urine which leads to the formation of cystine stones in the kidneys, ureter and bladder. Such cystine stones generate various complications that can ultimately lead to renal failure. As a result of this retrospective study, the

prevalence of cystinuria in the United Kingdom was estimated at between 0.48 and 0.62 per 10,000 people.

Ludovic Robin, Chief Business and Strategy Officer of Advicenne, commented: *"These results, based on a comprehensive approach, confirm and refine our estimates of the affected population, which includes an estimated 40,000 or more patients suffering from dRTA in Europe and the United States, and more than 24,000 suffering from cystinuria in Europe, the prevalence study for this second indication in the US is ongoing. These studies confirm the importance of our drug candidate as an effective solution for the treatment of these diseases."*

Dr. Luc-André Granier, Chief Executive Officer of Advicenne, concluded: *"I am proud of the contribution of our teams to these studies, which help to better describe dRTA and cystinuria. These two diseases are often under-diagnosed, especially because of the associated conditions and non-specific symptoms, and new data is particularly valuable in order to better understand their impact. We are confident that with our product, ADV7103, we will soon be able to provide a therapeutic solution for patients suffering from these debilitating conditions."*

About Advicenne

Advicenne (Euronext: ADVIC) specializes in pediatric-friendly therapeutics for the treatment of orphan diseases. The French specialty pharmaceutical company's lead product, ADV7103, has achieved positive results in Europe in a pivotal Phase III study of distal Renal Tubular Acidosis (dRTA) in children and adults, leading to its recent submission for European marketing authorization. The commercial launch of ADV7103 in Europe is anticipated for late-2020.

In North America, ADV7103 has received clearance from the US FDA and Health Canada for a pivotal Phase III clinical trial for the treatment of dRTA patients. Commercial launch in the United States is anticipated for 2022.

In addition to dRTA, ADV7103 is currently in Phase III clinical studies for a second indication, cystinuria, an inherited renal tubulopathy.

Advicenne is listed on the Euronext Paris and Brussels stock exchanges (ISIN: FR0013296746; Euronext ticker: ADVIC). Established in 2007, the company is headquartered in Nîmes, France.

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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 estimates for future performance and estimates regarding anticipated operating losses, future revenues, capital requirements and needs for additional financing. 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, they are based largely on the current expectations of Advicenne as of the date of this press release and 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 financial annual 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.