Advicenne's Flagship ADV7103 Receives Authorization for Pivotal Phase II/III Cystinuria Study in Belgium

Authorization extends patient recruitment base of European Phase II/III CORAL study, previously authorized in France

Nîmes, France, January 7, 2019 (5:45 p.m. CET) – Advicenne (Euronext: ADVIC), specializing in the development of adult and pediatric therapeutic products for the treatment of orphan renal and neurological diseases, announces today that it has received authorization from the Belgian health authority (FAMHP) to begin a pivotal Phase II/III CORAL clinical trial in cystinuria with its flagship drug candidate, ADV7103. The French pharmaceutical company also assesses its financial visibility at the close of 2018.

"*Today, patient care for those suffering from cystinuria is not optimal,"* observes **Professor Elena Levchenko**, Head of the Department of Pediatric Nephrology at UZ Leuven (University Hospitals Leuven), *"So I am very pleased to serve as Belgium's Principal Investigator for this European study, whose aim is to improve patient care and quality of life."*

Previously authorized for studies in patients suffering from dRTA, the recently approved pivotal Phase II/III clinical trial has been designed to evaluate the efficacy, safety, tolerance and compliance of ADV7103 in patients living with cystinuria. It does so in anticipation of the drug candidate's approval for this second indication in Europe, which is expected to double the size of its market population.

"The authorization to initiate a pivotal cystinuria clinical trial in Belgium increases our recruitment capacity for patients in this European study," **Dr. Luc-André Granier, Chief Executive Officer of Advicenne** adds, "and we are delighted to be partnering with UZ Leuven, which is one the best medical research centers in Europe and which has an internationally-recognized Department of Nephrology."

These developments will be financed with funds previously raised by Advicenne, which closed 2018 with a cash position of more than 26 M€ (30 M$). This sum is in line with Advicenne's forecasts and offers the company a financial visibility extending beyond the coming 24 months on the present basis.

About Cystinuria

Cystinuria is a rare inherited disease characterized by a transport abnormality of basic amino acids in the renal tubule causing the recurrent formation of large kidney stones. The disease can be diagnosed at all ages, but clinical symptoms generally appear in the first twenty years of life. Its prevalence in Europe is

on average one out of seven thousand, or approximately seventy thousand patients.[1] There is currently no first-line treatment authorized for this pathology in Europe.

About Advicenne

Advicenne (Euronext: ADVIC) specializes in pediatric-friendly therapeutics for the treatment of orphan renal and neurological diseases. The French pharmaceutical company's lead product, ADV7103, has achieved positive results in Europe in a pivotal Phase III study in children and adults with distal Renal Tubular Acidosis (dRTA). In addition to this indication, ADV7103 is being developed for Cystinuria, an inherited renal tubulopathy.

Advicenne plans to file ADV7103 for market authorization for dRTA in Europe in the coming months and anticipates its commercial launch in 2020 in Europe. In North America, the US FDA and Health Canada gave clearance to begin a pivotal Phase III clinical trial assessing ADV7103 in dRTA patients. Commercial launch in the United States is anticipated in 2021.

Advicenne is listed on the regulated market of Euronext in Paris (ISIN: FR0013296746; Euronext ticker: ADVIC). Established in 2007, the company is headquartered in Nîmes, France.

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** Unaudited data*

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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

[1] Eggermann T. et al, La cystinurie : une cause innée de lithiase urinaire, Orphanet Journal of Rare Diseases, 2012 ; 7:19

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 AMF, including those listed in Chapter 4, "Risk Factors," of its *document de référence* filed with the French Financial Markets Authority (the Autorité des marches financiers) on December 3, 2018 under number R.18-073. 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.