Advicenne achieves major milestones in the US thanks to several positive FDA opinions on ADV7103

- ***FDA agrees to submission of a marketing authorization application for ADV7103 in dRTA without further clinical studies.***
- ***The positive orphan drug designation decision for ADV7103 in cystinuria has opened constructive discussions with the FDA on development in this indication.***
- ***These achievements will accelerate partnership outlook for the commercialization of ADV7103 in the US, where the Company had already received expressions of interest.***

PARIS--(BUSINESS WIRE)-- Regulatory News:

Advicenne (Euronext Growth Paris ALDVI - FR0013296746), a specialty pharmaceutical company dedicated to the development and commercialization of innovative treatments for those suffering from rare renal diseases, announced today the achievement of major milestones in the development program of its ADV7103 drug in the U.S, and the search for commercial partners.

ADV7103 in dRTA (Distal Renal Tubular Acidosis): European data deemed satisfactory by the FDA for filing without additional clinical studies in the US

After numerous exchanges with experts from the US Food & Drug Administration (FDA), the Agency has concluded that European clinical data can be used to support an application for registration in dTRA. As a result, Advicenne will not conduct a specific clinical study in the U.S., significantly reducing the time and cost required to file a marketing authorization application in the U.S. for this indication.

The FDA's positive opinion was based on consideration of all the clinical data available from the European clinical program. Specifically, the pivotal phase III study (B21CS) and the long-term safety extension study (B22CS), which provide Advicenne with clinical data on efficacy and safety for patients followed over a period of more than 6 years. These studies, presented to *the European Renal Association* (ERA) and *the European Society of Pediatric Nephrology* (ESPN), are supplemented by data from real-life cohorts (*Real World Evidence - RWE*), and extensive European pharmacovigilance data.

Following an initial phase of discussions, the FDA considered that the drug's safety data met its requirements in terms of long-term tolerability. This first phase opened the path to a discussion on the relevance of efficacy data from these same studies. This was the purpose of the second phase of exchanges with complementary analysis data from the studies.

Advicenne is now working on the production of a marketing authorization application, accompanied by additional analyses of the natural history of patients and the disease and expects to submit a marketing authorization application in 2025. ADV7103 has orphan drug status in the dRTA indication in the US.

The results of the above studies will be reported at an analysts' meeting to be scheduled shortly.

ADV7103 in cystinuria: preparation for clinical development in consultation with the FDA

ADV7103 has been granted orphan drug designation (ODD) for the treatment of cystinuria in March 2024, adding to the existing orphan drug status for this indication in Europe. This validated Advicenne's regulatory and clinical strategy with the FDA. The ODD application was supported by preliminary clinical results in European patients with cystinuria and extensive data on the population size and expert opinion on the unmet need in this rare disease. Following this success, the Company wishes to discuss the proposed clinical development plan that will serve as the basis for a marketing authorization in this indication. A round of discussions will be launched before the end of the year.

This clinical program proposed has strong support from experts in this condition from both Europe and the United States, as well as from the International Cystinuria Foundation, representing the patient population. There is broad agreement that alkalinization is a cornerstone of treatment and that there is no satisfactory alkalinizing treatment that provides well tolerated, 24-hour pH control, indicated by recent data from the European Cystinuria Registry showing only on fourth of cystinuria patients followed in Europe have a urinary pH above 7.5, the therapeutic target set by international recommendations.

Pending the conclusion of discussions with the FDA and agreement on the clinical program, Advicenne plans to initiate a clinical trial in cystinuria. This study would recruit patients simultaneously in the United States and Europe and enable a registration application to be filed in both markets. In Europe, ADV7103 also has orphan drug status in this indication. Cystinuria, affects some 30,000 patients in the USA and 40,000 in Europe, represents a significant potential market for ADV7103.

Acceleration towards an agreement with potential commercial partners in the United States

Several pharmaceutical companies had shown an interest in ADV7103 in its two indications in the US, a territory Advicenne wishes to cover with a partner. Advicenne has received an initial proposal for the rights to ADV7103 in the US, the terms of which remain confidential. The recent news flow is expected to generate further interest, which the Company intends to pursue actively, particularly from the end of 2024.

Didier Laurens, Chief Executive Officer of Advicenne, commented: *"The latest exchanges with the US Food and Drug Administration have been particularly pragmatic and fruitful. They demonstrate the quality of the clinical data we have accumulated in recent years and the expertise of our teams. With the approval of our marketing authorization application for ADV7103 without having to conduct an additional clinical study in the US in dTRA on the one hand, and the possible simplification of its clinical development in cystinuria, on the other, Advicenne now has all the cards to materialize soon a structuring commercial agreement on this large market in the two indications covered by our treatment."*

ABOUT ADVICENNE

Advicenne (Euronext: ALDVI) is a specialty pharmaceutical company founded in 2007, specializing in the development of innovative treatments in Nephrology. Its lead product Sibnayal® has received Marketing Approval for distal renal tubular acidosis (dRTA) in EU and GB. ADV7103 is currently in late-stage development in cystinuria in Europe and in dRTA and cystinuria in the US and Canada.

Headquartered in Paris, Advicenne, listed on the Euronext Paris stock exchange since 2017, has now been listed on Euronext Growth Paris since its transfer on March 30, 2022. For additional information, see: https://advicenne.com/.

Disclaimer
This press release contains certain forward-looking statements concerning Advicenne group and its business, including its prospects and product candidate development. Such forward-looking statements are based on assumptions that Advicenne considers to be reasonable. However, there can be no assurance that the estimates contained in such forward-looking statements will be verified, which estimates are subject to numerous risks including the risks set forth in the 2023 Universal Registration Document filed with the French financial market authority on April 5, 2024 (a copy of which is available on www.advicenne.com) and to the development of economic conditions, financial markets and the markets in which Advicenne operates. The forward-looking statements contained in this press release are also subject to risks not yet known to Advicenne or not currently considered material by Advicenne. The occurrence of all or part of such risks could cause actual results, financial conditions, performance, or achievements of Advicenne to be materially different from such forward-looking statements. Advicenne expressly declines any obligation to update such forward-looking statements.

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