Advanced BioDesign announces exceptional first-in-human results for its innovative treatment against Acute Myeloid Leukemia

- **Drug candidate ABD-3001 is a galenic form of DIMATE, a first-in-class suicide inhibitor of aldehyde dehydrogenase 1 (ALDH1), being tested in relapsed patients with limited therapeutic options.**
- **Preliminary data from the First-in-human ODYSSEY study show that ABD-3001 was well tolerated, with activity of the drug candidate on its target, as well as proven biological activity, observed at all doses tested.**
- **Preliminary signs of efficacy were observed in over 65% of patients treated, two of whom benefited from a long-term treatment effect.**
- **These results pave the way for the second phase of the ODYSSEY trial, during which three cohorts of patients are scheduled to receive full three-month treatment cycles, enabling initial efficacy results to be obtained.**

Lyon (France), June 4, 2024 - Advanced BioDesign, a biotech company specializing in the development of new therapeutic approaches to resistant cancers, has released the first data from its first-in-human ODYSSEY study, aimed at treating acute myeloid leukemia (AML) with the drug candidate ABD-3001.

"We are delighted to have demonstrated the good safety and tolerability of our drug candidate ABD-3001 in this Phase I/II trial in AML patients resistant to standard therapies. The objectives of this first part of the study were met, and even exceeded our expectations. Not only did we observe activity of our drug candidate on its target of interest, ALDH1, but we also observed biological activity at all the doses tested. These results are very encouraging and allow us to enthusiastically begin the second phase of this study, during which several treatment cycles will be evaluated at optimized dose regimens to confirm ABD-3001's position in the therapeutic arsenal for the treatment of acute myeloid leukemia. I would like to thank all our patients and investigators for their involvement", **said Ismail Ceylan, CEO of Advanced BioDesign**.

Professor Regis Costello, Head of the Hematology and Cell Therapy in Oncology Department in Marseille (CEPCM, Timone Hospital), and principal investigator and coordinator of the ODYSSEY study, explained: "*The pre-clinical data were already remarkable, given the selective action of DIMATE on tumor cells while sparing healthy cells. But the transition to human trials often brings its share of disappointments. This Phase I/II trial, which is not intended to demonstrate therapeutic efficacy, has already clearly identified hematological effects. What's more, the results obtained in two patients give us grounds for optimism about the further development of this product. Indeed, we were able to observe that with a single injection of DIMATE, their hematological parameters improved over a period of 3 months, which in the context of acute leukemia, whose name reflects the often lightning-fast progression of the disease, represents a very encouraging response. All these observations have led us to accelerate the pace of development of ABD-3001 in two respects. The first is to modify the second part of the protocol, i.e. to treat patients over 3 cycles to observe any strong signals of efficacy. The second is, given the exceptional nature of the data already collected, to apply to the authorities for a compassionate prescription scheme for patients who have responded positively to the treatment*."

The multi-center ODYSSEY trial is the only Phase I/II first-in-human study in France targeting a treatment for AML. Initiated 15 months ago, its primary objective is to assess the safety of ABD-3001, as well as to collect pharmacokinetic and pharmacodynamic data to define a treatment regimen for future studies in patients with acute myeloid leukemia, for whom therapeutic options are limited and prognosis unfavorable. The study follows an adaptive design, with an initial single ascending dose in six patient cohorts.

The preliminary results announced today follow the progress made by the 6th and final cohort of patients.

- They show that ABD-3001 was well tolerated overall, with no major safety issues. Most of the toxicities recorded in the study were due to the disease and not to the treatment. To date, only one toxicity, at 540 mg/m² (6th dose), has been considered dose-limiting.
- Biological activity markers (inhibition of ALDH1A1 target enzyme activity and JNK protein phosphorylation) demonstrated the action of ABD-3001 from the first dose.
- On the hematological level, an effect on leukocytes was observed in 50% of patients 6 and 9 days after treatment. These data demonstrate a biological effect of ABD-3001 without any dose correlation, the effect being observed from the first dose of 18 mg/m2.
- Finally, in two patients, an improvement in hematological parameters was observed over the long term (around 3 months). For one of these patients, the study investigator is considering a specific request to the ANSM to continue treatment under compassionate use protocol.

Acute Myeloid Leukemia (AML) is one of the most frequent and severe leukemias affecting adults over 60. The incidence of this form of cancer in Western countries is around 5 per 100,000 inhabitants, and survival at 5 years is no more than 20%. Today, 150,000 patients a year around the world have reached a therapeutic impasse.

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About the ODYSSEY clinical trial

ODYSSEY is a Phase I/II clinical trial for the treatment of acute myeloid leukemia (AML). It is a multicenter study, with centers in Paris, Lyon and Marseille, designed to assess the safety and tolerability of the drug candidate ABD-3001.

Following an adaptive design, the study integrates an ascending single-dose first part, on six patient cohorts, followed by a second part, during which three patient cohorts will receive full four-week treatment cycles, enabling initial efficacy results to be obtained.

Fully funded by Advanced BioDesign, the ODYSSEY clinical trial is coordinated by Professor Régis COSTELLO (Hôpital de la Conception, Marseille), in collaboration with Doctor Lina BENAJIBA (Hôpital Saint-Louis, Paris), and Doctor Maël HEIBLIG (Hôpital Lyon Sud, Lyon).

About Advanced BioDesign

Advanced BioDesign is a French biotechnology company developing an innovative new therapeutic approach against resistant cancers, with a first indication in acute myeloid leukemia (AML). Its first drug candidate, ABD-3001, is a first-in-class "suicide" inhibitor of class 1 aldehyde dehydrogenases (ALDH1). In January 2022, Advanced BioDesign obtained authorization from the French Agence Nationale de Sécurité du Médicament (ANSM) to launch its first human clinical trial, ODYSSEY, which began in November 2022. Based in Lyon, Advanced BioDesign is supported and accompanied by Xerys Invest funds, which have been financing its research and development programs since 2013.

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