US FDA grants Orphan Drug Designation for iOnctura's first-in-class autotaxin cancer therapy

- *Autotaxin inhibitor, IOA-289, recently received the proposed INN cambritaxestat*
- *Cambritaxestat is in the Phase I AION-02 trial in combination with chemotherapy in metastatic pancreatic cancer*
- *It is the first autotaxin inhibitor to be investigated in cancer patients*

Amsterdam, The Netherlands and Geneva, Switzerland, 7 March 2024 - iOnctura, a pioneering, clinical-stage biotechnology company developing transformative cancer therapies, today announces that the US Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) to its autotaxin inhibitor cambritaxestat for the treatment of pancreatic cancer.

After assessing its novel chemical and biological properties including an attractive non-clinical safety and efficacy profile, the US FDA has granted Orphan Drug Status, conferring certain benefits during development and commercialization.

Following a separate submission process the World Health Organization has proposed the International Nonproprietary Name (INN) of cambritaxestat.

Cambritaxestat is being developed as a first-in-class therapy for highly fibrotic cancer indications. The drug's lead indication is metastatic pancreatic cancer where it is being combined with standard of care nab-paclitaxel and gemcitabine in the Phase I AION-02 study.

Inhibition of autotaxin is a novel treatment strategy that offers a three-pronged attack on the tumor through direct cancer cell inhibition, immune effector stimulation and inhibition of fibrotic processes, giving drugs and immune cells better access to the tumor.

Translational research showing the potential of cambritaxestat in multiple cancer models, including pancreatic cancer, has recently been published in the ESMO journal Immuno-Oncology and Technology (IOTECH), Cancer Research, the Journal of Experimental & Clinical Cancer Research, and Cancers. Across these publications, cambritaxestat showed strong reduction of metastasis and tumor outgrowth in preclinical models, as well as safe and tolerable dosing in healthy volunteers.

Catherine Pickering, Chief Executive Officer, iOnctura, said: *"There is an urgent need to develop new therapies for pancreatic cancer which is currently the third largest cause of death by cancer in the U.S., and the fourth in Europe. Although survival of patients with pancreatic cancer has improved in recent years, it still stands at just 13% after five years. This Orphan Drug Designation will support our goal to accelerate cambritaxestat through the clinic to provide a new treatment to patients with limited options."*

ENDS

For more information contact:

iOnctura
Catherine Pickering
Chief Executive Officer
T : +41 79 952 72 52

E: c.pickering@iOnctura.com

Optimum Strategic Communications
Mary Clark / Vici Rabbetts / Elena Bates
T: +44 208 078 4357
E: ionctura@optimumcomms.com

About iOnctura
iOnctura is a clinical-stage biotech developing selective cancer therapies against targets that play critical roles in multiple tumor survival pathways. This pioneering approach to drug development is expected to offer significant clinical benefits over the traditional approach of targeting a single pathway alone. iOnctura has progressed two therapeutic candidates into mid-stage clinical development: Roginolisib, an allosteric modulator of PI3Kδ; and cambritaxestat, a highly selective, non-competitive autotaxin inhibitor. IOA-359, a TGF-β pathway inhibitor is also undergoing an extensive pre-clinical program in preparation for first-in-human studies. iOnctura is backed by specialist institutional investors including M Ventures, Inkef Capital, VI Partners, Schroders Capital, European Innovation Council and 3B Future Health Fund. iOnctura BV is headquartered in Amsterdam, The Netherlands with its wholly owned Swiss subsidiary, iOnctura SA, located in Geneva, Switzerland.

About cambritaxestat (IOA-289)
Cambritaxestat is an orally dosed molecule that has shown preclinically to inhibit the growth and proliferation of cancer cells, stimulate immune cell infiltration into tumors and inhibit the development of fibrosis. Cambritaxestat is being developed as a first-in-class therapy for highly fibrotic cancer indications that overexpress autotaxin including pancreatic, liver, colorectal, ovarian and breast cancers. A Phase I study of cambritaxestat in combination with chemotherapy in metastatic pancreatic cancer started in Q4 2022 (NCT05586516).