iOnctura announces FDA clearance of IND application for roginolisib, a first-in-class allosteric modulator of PI3Kδ

- *Roginolisib is in development for solid and hematologic malignancies including uveal melanoma*

Geneva, Switzerland and Amsterdam, The Netherlands, 31 October 2023 - iOnctura, a clinical-stage biotech developing selective cancer therapies against targets that play critical roles in multiple tumor survival pathways, today announces that the US Food and Drug Administration (FDA) has granted permission to proceed with clinical investigations of roginolisib in the United States of America.

Roginolisib (IOA-244) is in development for solid and hematologic malignancies including uveal melanoma, a rare cancer in which malignant (cancer) cells form in the uveal tract of the eye. When the cancer metastases, which it does in approximately 50% of patients, there are limited treatment options and projected overall survival is only a year.

As the first novel allosteric modulator of PI3Kδ, roginolisib marks a new era in drug development within the class. The unique binding mode, combined with high selectivity for PI3Kδ, is expected to translate to an improved safety and tolerability profile relative to that of earlier generation inhibitors.

"As we remain on track to deliver final clinical data from the initial patients treated with roginolisib in 2024, the clearance of our IND application demonstrates our commitment to driving roginolisib through the clinic" **said Catherine Pickering, Chief Executive Officer of iOnctura.** *"We believe roginolisib has the potential to slow or halt the progression of uveal melanoma, so providing an important treatment option for patients who currently have no approved therapeutic options after they progress on their first line therapy."*

Roginolisib is being investigated in the DIONE-01 trial, a two-part, first-in-human Phase I study (ClinicalTrials.gov, identifier NCT04328844). The study is fully enrolled with final data expected Q1 2024. Across all patients treated to date, roginolisib monotherapy has shown 7% Grade 3/4 toxicities, with no dose limiting toxicities, drug-related serious adverse event (SAE) or drug related adverse event (AE) leading to dose interruption or discontinuation. Whilst median Overall Survival has not yet been reached, 62% of patients were alive at 12 months, which compares favourably to historical controls of 34% in the same setting[1].

Long-term administration of roginolisib is well tolerated and patients have been treated for up to 40 months in the study. Promising clinical activity has been observed across patients with both solid and hematological cancers.

ENDS

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[1] Rantala et al., Melanoma Res. 2019 Dec;29(6):561-568

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About iOnctura

iOnctura is a clinical-stage biotech developing selective cancer therapies against targets that play critical roles in multiple tumor survival pathways such as cellular proliferation; escape from immune detection; and drug resistance. iOnctura's 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 (IOA-244), an allosteric modulator of PI3Kδ; and IOA-289, a highly selective, non-competitive autotaxin (ATX) 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, 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 roginolisib

Roginolisib (IOA-244) is a first-in-class small molecule allosteric modulator of PI3Kδ. Its unique structural and selectivity features drive a unique way of inhibiting PI3Kδ which translates into a highly beneficial tolerability and clinical benefit profile. PI3Kδ over-expression stimulates multiple cancer mechanisms and has an oncogenic role in many tumor types. Roginolisib has a multi-modal effect on cancer; directly preventing cancer cell proliferation, harnessing an anti-tumor immune response via an effect on regulatory T-cells and cytotoxic T cells and potentiating the effect of immunotherapy. Roginolisib is currently in the extension phase of the DIONE-01 trial, a two-part, first-in-human dose study evaluating the drug in advanced cancers and as a combination partner for conventional and immune-therapies (NCT04328844).