iOnctura Expands Roginolisib Trials to the US, Advancing Multi-Indication Strategy in Oncology

- US cancer centers begin enrolling patients into the global, randomized Phase II study of roginolisib in metastatic uveal melanoma
- Dana-Farber Cancer Institute launches new trial of roginolisib for Chronic Lymphocytic Leukemia, backed by Department of Defense grant

Geneva, Switzerland; Amsterdam, The Netherlands; and Cambridge, Massachusetts, USA, 9 September 2025 - iOnctura, a clinical-stage precision oncology company focused on neglected and hard-to-treat cancers, today announces the expansion of its clinical trial program for lead candidate roginolisib into the United States. Several US trial sites are now enrolling patients in the OCULE-01 Phase II study in metastatic uveal melanoma, with an additional study, led by Dr Jennifer Brown, underway in chronic lymphocytic leukemia (CLL) at Dana-Farber Cancer Institute.

Catherine Pickering, CEO and co-founder of iOnctura said, "We are proud to begin the next phase of iOnctura's global growth by making roginolisib available to US patients in our clinical studies. The enthusiastic response from US investigators, leading research centers, and patient communities highlights the significant opportunity to impact outcomes for individuals with limited treatment options."

Roginolisib, an orally dosed small molecule allosteric modulator of PI3Kδ, is being investigated in multiple randomized Phase II studies in solid and hematological malignancies. The global Phase II randomized OCULE-01 study (NCT06717126) will assess whether roginolisib improves overall survival in patients with metastatic uveal melanoma who have progressed on prior therapy. Patients from both the US and Europe are now being treated in the study, which started in March 2025.

Roginolisib is also being investigated in patients with non-small cell lung cancer (NSCLC) (NCT06879717) and myelofibrosis (NCT06887803). In these diseases, targeting the PI3Kδ pathway in combination with standard therapies has potential to reverse resistance[1,2].

Seeing the promise of roginolisib as a combination therapy, the US Department of Defense (DoD) recently awarded a substantial grant to Dr. Jennifer Brown at Dana-Farber Cancer Institute in Boston, MA, to explore the potential of roginolisib in relapsed / refractory CLL in a Phase I / II study (NCT06644183).

Dr. Jennifer R Brown, M.D. Ph.D., Director of the CLL Center of the Division of Hematologic Malignancies at Dana-Farber Cancer Institute and Principal Investigator on the study and grant said, "We are enthusiastic

[1] Exposito et al., Cancer Res (2023) 83 (15): 2513–2526
[2] Moyo et al., Clin Cancer Res (2023) 5; 29(13): 2375-2384

about the potential of roginolisib in enhancing treatment outcomes for CLL patients who have relapsed after prior Bruton's tyrosine kinase inhibitor therapy. We feel roginolisib offers a promising avenue to improve remission depth and duration in combination with venetoclax and rituximab, whilst minimizing treatment-related side effects."

Recently published preclinical evidence by Sasi, Tarantelli et al. supports the synergistic potential of roginolisib and venetoclax in the treatment of CLL[3].

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About iOnctura

iOnctura is a clinical-stage precision oncology company combating neglected and hard-to-treat cancers with a pipeline of first-in-class small molecules. The bold new treatments extend lives and improve healthspans, changing the outlook for patients and their families. Lead asset, roginolisib, is an allosteric modulator of PI3Kδ with a unique chemical structure and binding mode. Allosteric modulation is a new archetype for precise inhibition of PI3Kδ, promising clinical activity without the detrimental tolerability seen with previous generations of inhibitors. Roginolisib is being investigated in multiple randomized Phase II studies in solid and hematological malignancies. iOnctura is headquartered in Amsterdam, The Netherlands with subsidiaries located in Geneva, Switzerland and Cambridge, MA, USA. iOnctura is backed by specialist institutional investors including Syncona, M Ventures, Inkef Capital, EIC Fund, VI Partners, Schroders Capital and XGEN Venture.

About roginolisib

Roginolisib is an allosteric modulator of PI3Kδ with a unique chemical structure and binding mode. Allosteric modulation is a new archetype for precise inhibition of PI3Kδ, promising clinical activity without the detrimental tolerability seen with previous generations of inhibitors. The PI3K signaling pathway is one of the most commonly dysregulated pathways across multiple cancer types. The potential of roginolisib has been validated by positive clinical signals in Phase I in solid tumor and hematological malignancies, including a doubling of overall survival compared to historical controls in rare eye cancer, uveal melanoma. The company has carefully designed its clinical program to allow full development in uveal melanoma, while in parallel validating the program in larger market indications. The Phase II OCULE-01 study in uveal melanoma started in March 2025, the PULMO-01 study in NSCLC started in May 2025. Further studies in other malignancies, including myelofibrosis, are being initiated.

[3] Sasi, Tarantelli et al., Haematologica, published online June 2025

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