



Ionis advances cancer program from collaboration into clinical study

ION537 will be evaluated at MD Anderson in a Phase 1 study of patients with liver and head and neck cancers

Ionis' collaboration with The University of Texas MD Anderson Cancer Center to discover and develop novel antisense cancer therapies recently achieved an important milestone: the first patient has been dosed with ION537 in a Phase 1 clinical study. ION537 is designed to target YAP1, a transcription factor that preclinical studies have shown to be critical in certain hepatocellular and head and neck cancers. ION537 was the first program initiated in the Ionis-MD Anderson collaboration, which has moved quickly from target validation to human cancer trials.

The collaboration brings MD Anderson's expertise in preclinical and translational research and the drug discovery and development capabilities of its [Therapeutics Discovery division](#) together with Ionis' proven success developing antisense medicines, with a goal to accelerate Ionis' antisense technology platform in cancer. Ionis worked closely with MD Anderson researchers on preclinical studies to validate the molecular target, YAP1, and MD Anderson's [Translational Research to Advance Therapeutics and Innovation in Oncology \(TRACTION\)](#) platform for translational studies that enabled the Phase 1 study. Previously undruggable, YAP1 is a transcription factor that is uniquely addressable with Ionis' technology. It is an emerging, important therapeutic target, not only in tumor cell survival but also in regulating the immune microenvironment of tumors, according to Dr. A. Robert MacLeod, vice president and franchise head of oncology at Ionis.

"Development of ION537 represents the synergistic combination of capabilities from Ionis and MD Anderson that have enabled the deployment of our antisense technology to previously undruggable cancer targets. We are excited about the broad potential of ION537 to bring benefit to cancer patients with many different tumor types," said Dr. MacLeod, adding that, "though the Phase 1 trial of ION537 is initially focused on hepatocellular and head and neck cancers with specific molecular features, the target has broader potential for treating numerous forms of cancer alone or in combination with immuno-oncology therapeutics."



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KEY TAKEAWAY

The progress of ION537 exemplifies how an industry and academic collaboration can identify a previously undruggable target uniquely addressable with Ionis' technology, and rapidly advance a novel precision medicine targeting specific cancers with a clinical unmet medical need.

Ionis and MD Anderson are evaluating ION537 in a single center, open label, non-randomized, two-part study. In total, the study is planning to enroll up to 102 participants. Patients for the study will be identified using an assay co-developed by Ionis and MD Anderson and validated through MD Anderson's CLIA-certified laboratory¹, making the study a true example of precision medicine.

To learn more about the Phase I study of ION537 visit:

[A Study of ION537 in Patients With Molecularly Selected Advanced Solid Tumors - Full Text View - ClinicalTrials.gov](#)

1. The Clinical Laboratory Improvement Amendments (CLIA) regulate laboratory testing and require clinical laboratories to be certified by the Center for Medicare and Medicaid Services (CMS) before they can accept human samples for diagnostic testing.