The NCCN Oncology Research Program (ORP) strives to improve the quality of life for patients and reduce cancer-related deaths by advancing cancer therapies through research. Since the program's establishment in 1999, the NCCN ORP has brought millions of dollars in research grants to investigators at NCCN Member Institutions. Research grants are provided to NCCN through collaborations with pharmaceutical and biotechnology companies; these grants are in turn used to support scientifically meritorious cancer research efforts.

NCCN ORP studies typically explore new avenues of clinical investigation and seek answers to important cancer-related questions. All studies are approved and funded through a scientific peer-review process and are overseen by the ORP.

This feature highlights an NCCN study funded through the grant mechanism.

Phase II Study of FGFR4 Inhibitor Futibatinib in Combination With Anti–PD-1 Antibody Pembrolizumab in Patients With Advanced or Metastatic Hepatocellular Carcinoma With FGF19 Expression After First-Line Therapy

Principal Investigator: Nguyen Tran, MD
Condition: Advanced or metastatic hepatocellular carcinoma
Institution: Mayo Clinic

This phase II trial studies the effect of futibatinib + pembrolizumab in treating patients with FGF19-positive BCLC stage A, B, or C advanced or metastatic hepatocellular carcinoma (HCC). Aberrant expression of FGF19 is associated with tumor proliferation and invasive ability, with associated poor prognosis. FGF19 ligand binds exclusively to FGFR4. Futibatinib is a novel and highly selective irreversible pan-FGFR inhibitor, and when combined with immunotherapy, such as pembrolizumab, may alter the tumor microenvironment. Preclinical data suggest the combination may have synergistic effects on the tumor. Combining an FGFR inhibitor with immunotherapy has led to decreased infiltration of immunosuppressive tumor-associated macrophages, increased NK and B cells, and a higher proliferative, activated state of T and NK cells. Furthermore, the combination resulted in a significant increase in both T-cell fraction and clonality, resulting in improved survival. This improved survival provided a strong rationale for the current study.

Primary Objective:

- Determine the efficacy of combination of futibatinib and pembrolizumab in patients with advanced HCC and high FGF19 expression who have received at least one line of therapy using progression-free survival at 6 months

Secondary Objectives:

- Assess the safety and tolerability of combination futibatinib + pembrolizumab through adverse event monitoring
- Determine the overall objective response rate and overall survival of patients with advanced HCC treated with combination futibatinib + pembrolizumab
- Assess change in overall health-related quality of life, as measured by the global health domain of the EORTC Quality-of-Life Questionnaire-Core 30 (EORTC-QLQ-C30) between baseline and time of first restaging scan

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For more information on specific trials, including patient selection criteria, use the contact information listed with each study.

For more information on the NCCN ORP, including a complete detailing of the clinical studies currently underway at NCCN Member Institutions, go to www.nccn.org/education-research/nccn-oncology-research-program/orp-main-page.