



RESEARCH TOPIC CLI31

Multicenter clinical and translational study of first-line treatment with cisplatin, gemcitabine, and immune checkpoint inhibitors for advanced biliary tract cancer

Clinical Unit name

Hepatopancreatobiliary Oncology Section, Medical Oncology and Hematology Unit
IRCCS – Humanitas Research Hospital

Supervisor

Lorenza Rimassa

lorenza.rimassa@hunimed.eu

Abstract

Biliary tract cancers (BTC) are a heterogeneous group of rare and aggressive tumors, with limited therapeutic options and a dismal prognosis. Chemotherapy has represented the sole treatment option for unresectable disease for over a decade. Recently, immune checkpoint inhibitors (ICI) combined with chemotherapy have been shown to improve outcomes. However, predictive biomarkers of response are lacking, and understanding the underlying mechanisms driving response to ICI remains a highly unmet need.

The primary endpoint of this study is to define predictive factors of response to chemoimmunotherapy in BTC.

Patients with advanced BTC not previously treated with systemic therapy for advanced disease will be enrolled in this multicenter study and treated with cisplatin, gemcitabine, and ICI.

At the same time, translational analyses will be focused on dissecting the cellular and molecular pathways occurring in the tumor microenvironment during treatment. Specific characteristics of the microenvironment defined by these analyses will be correlated with response to chemo-immunotherapy and outcome, including OS and PFS.

Tumor samples will be collected at diagnosis and at the first tumor assessment, blood samples will be collected at pre-specified time points until disease progression.

Scientific references

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- 3) Kelley RK, Ueno M, Yoo C, et al. Pembrolizumab in combination with gemcitabine and cisplatin compared with gemcitabine and cisplatin alone for patients with advanced biliary tract cancer (KEYNOTE-966): a randomised, double-blind, placebo-controlled, phase 3 trial. *Lancet*. 2023 Apr 14:S0140-6736(23)00727-4



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Type of contract

PhD scholarship of € 22.400 gross per year awarded by Humanitas University. This sum is exempt from IRPEF income tax according to the provisions of art. 4 of Law no. 476 of 13th August 1984, and is subject to social security contributions according to the provisions of art. 2, section 26 and subsequent sections, of Law no. 335 of 8th August 1995 and subsequent modifications.

Borsa di dottorato pari a € 22.400 annui lordi erogata da Humanitas University. Importo non soggetto a tassazione IRPEF a norma dell'art. 4 della L. 13 agosto 1984 n. 476 e soggetto, in materia previdenziale, alle norme di cui all'art. 2, commi 26 e segg., della L. 8 agosto 1995, n. 335 e successive modificazioni.