Targeted mutation-based therapy for intrahepatic cholangiocarcinoma

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Supplementary Table 1. Ongoing clinical trials targeted mutation-based therapy for intrahepatic cholangiocarcinoma

NCT Number	Title	Status	Conditions	Interventions (Drug)	Major Outcome Measures	Phases	Enrollment	Study Designs
FGFR2								
NCT04093362	Futibatinib Versus Gemcitabine-Cisplatin Chemotherapy as First-Line Treatment of Patients With Advanced Cholangiocarcinoma Harboring FGFR2 Gene Rearrangements	Active, not recruiting	Advanced Cholangiocarcinoma	TAS-120, Cisplatin/Gemcitabine	PFS, ORR, and DCR	Phase 3	216	Randomized, Open Label
NCT03656536	A Study to Evaluate the Efficacy and Safety of Pemigatinib Versus Chemotherapy in Unresectable or Metastatic Cholangiocarcinoma	Recruiting	Unresectable Cholangiocarcinoma	Pemigatinib, Gemcitabine, Cisplatin	PFS, ORR, and DCR	Phase 3	434	Randomized, Open Label
NCT04353375	Phase II Study of HMPL-453 Tartrate in Patients With Advanced Intrahepatic Cholangiocarcinoma With FGFR2 Fusion	Not yet recruiting	Advanced Bile Duct Cancer	HMPL-453	ORR, DCR, TTR, DoR, PFS, and OS	Phase 2	29	Single Group, Open Label

NCT05678270	A Study of ICP-192 in Patients With FGFR2-Rearranged Unresectable or Metastatic Intrahepatic Cholangiocarcinoma	Recruiting	Intrahepatic Cholangiocarcinoma (ICC)	ICP-192	ORR, PFS, DCR, DOR, TTR, OS, and AE	Phase 2	64	Single Group, Open Label
NCT05174650	Treatment of Atezolizumab and Derazantinib in Patients With Advanced iCCA With FGFR2 Fusions/Rearrangements	Recruiting	Intrahepatic Cholangiocarcinoma	Atezolizumab+ Derazantinib	AE, ORR, DCR, DOR, PFS, and OS	Phase 2	37	Single Group, Open Label
NCT04526106	REFOCUS: A First-in-Human Study of Highly Selective FGFR2 Inhibitor, RLY-4008, in Patients With ICC and Other Advanced Solid Tumors	Recruiting	Intrahepatic Cholangiocarcinoma| Other Solid Tumors, Adult	RLY-4008	Part 1: Determination of maximum tolerated dose (MTD) and recommended Phase 2 dose (RP2D) of RLY-4008; Part 1: AE; Part 2 and Part 3:ORR, DOR, and DCR	Phase 1|Phase 2	490	Non-Randomized, Parallel, Open Label
IDH								

NCT05242822	A Study to Evaluate KIN-3248 in Participants With Advanced Tumors Harboring FGFR2 and//or FGFR3 Gene Alterations	Recruiting	Solid Tumor (including Cholangiocarcinoma)	KIN-3248	Part A (dose escalation) - incidence of dose limiting toxicities (DLTs) and AEs|Part B (dose expansion) -ORR, DCR, DOR, and PFS	Phase 1	120	Non-Randomized, Parallel, Open Label
NCT05814536	IDH1 Inhibitor AB-218 in Patients With Advanced IDH1 Mutant Cholangiocarcinoma and Other Solid Tumor	Not yet recruiting	Cholangiocarcinoma	AB-218 capsule	DLT, TEAE, ORR, DCR, DOR, PFS and OS	Phase 1	63	Single Group Assignment, Open Label
NCT03878095	Testing Olaparib and AZD6738 in IDH1 and IDH2 Mutant Tumors	Recruiting	Solid Tumor (including Cholangiocarcinoma)	Olaparib	ORR, PFS, OS, DOR, and AE	Phase 2	50	Single Group Assignment, Open Label
NCT03991832	Study of Olaparib and Durvalumab in IDH-Mutated Solid Tumors	Recruiting	Solid Tumor (including Cholangiocarcinoma)	Olaparib, Durvalumab	ORR, PFS, OS, DOR, and AE	Phase 2	58	Single Group Assignment, Open Label

NCT04762602	A Study of HMPL-306 in Advanced Solid Tumors With IDH Mutations	Recruiting	Isocitrate Dehydrogenase Gene Mutation	HMPL-306	Part 1: DLTs|Part 1 and Part 2: Aes, ORR, CBR, DoR, and PFS	Phase 1	90	Non-Randomized, Sequential Assignment, Open Label
HER2								
NCT03924466	Repeatability of 68-GaNOTA-Anti-HER2 VHH1 PET/CT in Breast Carcinoma Patients	Recruiting	Solid Tumor (including Cholangiocarcinoma)	68GaNOTA-Anti-HER2 VHH1	Repeatability of lesional PET/CT characteristics	Phase 2	55	Single Group Assignment, Open Label
NCT05253053	To Evaluate Efficacy and Safety of TT-00420 as Monotherapy and Combination Therapy in Patients With Advanced Solid Tumors	Recruiting	Solid Tumor (including Cholangiocarcinoma)	TT-00420 + Atezolizumab + Nab-Paclitaxel	DLT, ORR, DCR, DOR, PFS, and OS	Phase 1|Phase 2	114	Non-Randomized, Sequential Assignment, Open Label
MSI-H/dMMR								
NCT03544723	Safety and Efficacy of p53 Gene Therapy Combined With Immune Checkpoint Inhibitors in Solid Tumors.	Unknown status	Solid Tumor|Lymphoma	Ad-p53	ORR, AE, DoR,and PFS	Phase 2	40	Single Group Assignment, Open Label

ORR: objective response rate; PFS: progression-free survival; DCR: disease control rate; DOR: duration of response; OS: overall survival; AE: adverse event.