Jazz Oncology Clinical Research Interests

Jazz Pharmaceuticals

Jazz Oncology Clinical Research Interests, Overview

Preclinical

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- **CombiPlex for exploratory activities**
- **Undisclosed targets for oncology**
- **KRAS inhibitor targets for oncology**

Phase 1

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- **Zanidatamab ± chemotherapy**
  Advanced HER2-expressing cancers
- **Zanidatamab + tucatinib**
  HER2-positive BC (PRE-I-SPY; QuantumLeap Healthcare Collaborative)
- **JZP898 (conditionally activated IFNα prodrug) ± pembrolizumab**
  Advanced or metastatic solid tumors
- **JZP815 (Pan-Raf inhibitor)**
  Advanced or metastatic solid tumors harboring MAPK pathway alterations
- **JZP341 (long-acting *Erwinia* asparaginase)**
  Advanced or metastatic solid tumors
- **JZP351**
  Low-intensity dosing for higher risk MDS

Phase 2

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- **Zanidatamab**
  HER2-positive (IHC 3+) solid tumors (DiscovHER Pan-206)
- **Zanidatamab + chemotherapy**
  HER2-expressing GI cancers
- **Paclitaxel + ramucirumab ± zanidatamab**
  Advanced HER2-positive GEA (Canadian Cancer Trials Group)
- **Zanidatamab + SOC**
  BC (I-SPY 2; QuantumLeap Healthcare Collaborative)
- **Zanidatamab with palbociclib + fulvestrant**
  Locally advanced and/or metastatic HER2-positive/HR-positive BC
- **Zanidatamab**
  Early stage HER2-positive BC
- **Zanidatamab + evorpacept (ALX148)**
  Advanced HER2-expressing cancers[1]
- **JZP351 + other approved therapies**
  - De novo or R/R AML
  - R/R AML or HMA failure MDS
- **JZP351**
  - Newly diagnosed older adults with high-risk AML (ALFA)
  - High-risk MDS (EMSCO)
- **Lurbinectedin**
  R/R Ewing sarcoma (EMERGE 101)

Phase 3

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- **Zanidatamab + SOC**
  1L HER2-positive advanced or metastatic BTC (HERIZON-BTC-302)[a]
- **Zanidatamab with chemotherapy ± tislelizumab**
  1L HER2-positive advanced or metastatic GEA[2] (HERIZON-GEA-01)
- **Zanidatamab with chemotherapy**
  Previously T-DXd–treated HER2-positive BC (EmpowHER-303)
- **Lurbinectedin + atezolizumab**
  1L maintenance in ES SCLC[1] (IMforte; Roche)
- **Lurbinectedin ± irinotecan**
  2L in relapsed SCLC[1] (LAGOON; PharmaMar)
- **JZP351**
  Newly diagnosed adults with standard- or high-risk AML (AMLSG)
- **Dordaviprone (ONC201)**
  Newly diagnosed H3 K27M-mutant diffuse glioma (ACTION)

■ Jazz-sponsored studies ■ Jazz & MD Anderson Cancer Center Collaboration studies ■ Key Cooperative Group studies ■ Chimerix-sponsored study (now a part of Jazz Pharmaceuticals)

[1]Partnered collaboration; [2]Co-sponsored trial.

JZP351 is also known as CPX-351.

Each bullet represents a unique trial. Information listed here is based on active phase 1 to 3 clinical trials.

[a]Based on the results of a phase 2 trial (HERIZON-BTC-01), zanidatamab received accelerated approval by the US Food and Drug Administration for the treatment of adults with previously treated, unresectable or metastatic HER2-positive (IHC 3+) BTC. The confirmatory phase 3 HERIZON-BTC-302 trial is ongoing.

These compounds and/or potential new uses are investigational and have not been approved by the US Food and Drug Administration.
The information presented should not be construed as a recommendation for use.

Ongoing Oncology Studies

Agent	Therapeutic Area	Phase	ID Number (Study Name, *Sponsor*)	Study Description	Study Design	Patient Population	Objective
Zanidatamab	BTC	Phase 3 •••	NCT06282575 (HERIZON-BTC-302)	Zanidatamab + SOC for 1L HER2-positive advanced or metastatic BTC[1]	Open-label, multicenter, randomized	Target enrollment: 286 Population: Pts ≥18 years of age with locally advanced or metastatic HER2-positive BTC	Primary: PFS in pts with IHC 3+ tumors Secondary: OS (IHC 3+ and all pts), PFS (all pts), cORR, DOR, TEAEs, maximum serum concentration of zanidatamab, ADAs, PROs
	GI cancers	Phase 2 ••	NCT03929666	Zanidatamab + chemotherapy for HER2-expressing GI cancers[1,2]	Open-label, multicenter	Target enrollment: 74 Population: Pts ≥18 years of age with HER2-expressing GI cancers	Primary: DLTs, AEs, ORR Secondary: DCR, DOR, CBR, PFS, OS, ADAs, concentration of zanidatamab
	GEA	Phase 2 ••	NCT06043427 (GA4, *Canadian Cancer Trials Group*)	Paclitaxel + ramucirumab ± zanidatamab in advanced HER2-positive GEA[3]	Open-label, randomized	Target enrollment: 168 Population: Pts ≥18 years of age with unresectable or HER2-positive metastatic GEA	Primary: PFS Secondary: OS, objective response rate, AEs, HRQOL
		Phase 3 •••	NCT05152147 (HERIZON-GEA-01)	Zanidatamab with chemotherapy ± tislelizumab for 1L HER2-positive advanced or metastatic GEA[4]	Open-label, multicenter, randomized	Target enrollment: 918 Population: Pts ≥18 years of age with HER2-positive advanced or metastatic GEA	Primary: PFS, OS Secondary: ORR, DOR, AEs, HRQOL, serum concentration of zanidatamab, tislelizumab, and trastuzumab, ADAs
	BC	Phase 1/1b •	NCT05868226 (PRE-I-SPY, *QuantumLeap Healthcare Collaborative*)	Zanidatamab + tucatinib in HER2-positive BC[2,3]	Open-label, multicenter, multi-arm	Target enrollment: 54 Population: Pts ≥18 years of age with solid tumors or HER2-positive BC for the zanidatamab + tucatinib arm	Primary: AEs, DLTs, MTD, RP2D, ORR, DOR Secondary: PFS, CBR
		Phase 2a ••	NCT04224272	Zanidatamab with palbociclib + fulvestrant in locally advanced and/or metastatic HER2-positive/HR-positive BC[1,2]	Open-label, multicenter	Target enrollment: 86 Population: Pts ≥18 years of age with HER2-positive/HR-positive advanced BC	Primary: DLTs, AEs, PFS at 6 months, laboratory abnormalities Secondary: Maximum serum concentration of zanidatamab, ADAs, ORR, DOR, DCR, OS, PFS
		Phase 2 ••	NCT01042379 (I-SPY 2, *QuantumLeap Healthcare Collaborative*)	Zanidatamab + SOC for BC[2,3]	Open-label, multicenter, randomized	Target enrollment: 5000 Population: Pts ≥18 years of age with BC	Primary: pCR Secondary: Qualification and exploratory markers to predict pCR and RCB, RFS at 3 and 5 years, OS, AEs, SAEs, laboratory abnormalities, MRI volume
			NCT05035836 (*MDACC*)	Zanidatamab in early stage HER2-positive BC[5]	Open-label, single-arm	Target enrollment: 20 Population: Pts ≥18 years of age with HER2-positive BC	Primary: pCR Secondary: RCB, radiographic response and volumetric change in tumor size, safety, tolerability, AEs, TEAEs, tumor-based predictive biomarkers of response
		Phase 3 •••	NCT06435429 (EmpowHER-303)	Zanidatamab with chemotherapy for previously T-DXd-treated HER2-positive BC[1]	Open-label, multicenter, randomized	Target enrollment: 550 Population: Pts ≥18 years of age with HER2-positive BC	Primary: PFS Secondary: OS, cORR, DOR, TEAEs, serum concentration of zanidatamab, ADAs, PROs
	BC, GEA, other solid tumors	Phase 1b/2 •	NCT05027139	Zanidatamab + evorpacept (ALX148) in advanced HER2-expressing cancers[1,6]	Open-label, multicenter, single-arm	Target enrollment: 93 Population: Pts ≥18 years of age with advanced HER2-expressing cancers	Primary: DLTs, AEs, laboratory abnormalities, ORR Secondary: DCR, CBR, DOR, PFS, PFS at 6 months, OS, maximum serum and trough concentration of zanidatamab and evorpacept, ADAs
	Pan tumor	Phase 2 ••	NCT06695845 (DiscovHER Pan-206)	Zanidatamab in HER2-positive (IHC 3+) solid tumors (pan-tumor)[1]	Open-label, multicenter	Target enrollment: 200 Population: Pts ≥18 years of age with HER2-overexpressing (IHC 3+) locally advanced or metastatic solid tumors (except BTC)	Primary: cORR by ICR Secondary: Investigator-assessed cORR, DOR, DCR, TTR, PFS, OS, AEs, ADAs, PROs, HRQOL, serum concentration of zanidatamab
	Solid tumors	Phase 1 •	NCT02892123	Zanidatamab ± chemotherapy in advanced HER2-expressing cancers[1,2]	Open-label, multicenter, single-arm	Target enrollment: 279 Population: Pts ≥18 years of age with HER2-expressing cancer	Primary: DLTs, AEs Secondary: Serum concentration of zanidatamab, ADAs, objective response, PFS

Agent	Therapeutic Area	Phase	ID Number (Study Name, *Sponsor*)	Study Description	Study Design	Patient Population	Objective
JZP898	Solid tumors	Phase 1 •	NCT06108050	JZP898 ± pembrolizumab in advanced or metastatic solid tumors[1]	Open-label, multicenter, dose-finding, dose-expansion	Target enrollment: 177 Population: Pts ≥18 years of age with previously treated advanced or metastatic solid tumors	Primary: DLTs, TEAEs, SAEs, dose modifications, investigator-assessed objective response rate Secondary: PK parameters, dose proportionality, DOR, DCR, PFS, OS, ADAs
JZP815	Solid tumors	Phase 1 •	NCT05557045	JZP815 in advanced or metastatic solid tumors harboring MAPK pathway alterations[1]	Open-label, multicenter	Target enrollment: 320 Population: Pts ≥18 years of age with advanced/metastatic solid tumors with MAPK pathway alterations	Primary: DLTs, TEAEs, SAEs, other safety, dose interruptions/reductions, ORR, DOR Secondary: PK analyses, ORR, PFS, OS
Lurbinectedin	Ewing sarcoma	Phase 1/2 •	NCT05734066 (EMERGE 101)	Lurbinectedin in R/R Ewing sarcoma[1]	Open-label, sequential assignment	Target enrollment: 60 Population: Pediatric pts with previously treated solid tumors and pediatric/young adult pts with R/R Ewing sarcoma	Primary: DLTs, TEAEs, objective response rate per investigator assessment Secondary: PK, ORR, PFS, DOR, DCR, CBR, OS
	ES SCLC	Phase 3 • • •	NCT05091567 (IMforte, *Roche*)	Lurbinectedin + atezolizumab as 1L maintenance in ES SCLC[6]	Open-label, multicenter, randomized	Target enrollment: 690 Population: Pts ≥18 years of age with ES SCLC	Primary: IRF-assessed PFS, OS Key secondary: Investigator-assessed PFS, confirmed ORR, DOR, PFS at 6 and 12 months, OS at 12 and 24 months, AEs, ADAs
	Relapsed SCLC	Phase 3 • • •	NCT05153239 (LAGOON, *PharmaMar*)	Lurbinectedin ± irinotecan in 2L relapsed SCLC[6]	Open-label, multicenter, randomized	Target enrollment: 705 Population: Pts ≥18 years of age with SCLC	Primary: OS Secondary: PFS, ORR, DOR, PROs
Dordaviprone (ONC201)	Diffuse glioma	Phase 3 • • •	NCT05580562 (ACTION)	ONC201 in H3 K27M-mutant diffuse glioma following radiotherapy[1]	Double-blind, placebo-controlled, multicenter, randomized	Target enrollment: 450 Population: Pts with histologically diagnosed H3 K27M-mutant diffuse glioma	Primary: OS, PFS as assessed by RANO-HGG criteria Secondary: AEs, clinical laboratory changes from baseline, PFS using RANO-HGG criteria, corticosteroid response, performance status response
JZP341	Solid tumors	Phase 1 •	NCT05631327	JZP341 (long-acting *Erwinia* asparaginase) in advanced or metastatic solid tumors[1]	Open-label, multiple-dose, multicenter, dose-finding, dose-expansion	Target enrollment: 88 Population: Pts ≥18 years of age with advanced or metastatic solid tumors	Primary: DLTs, TEAEs, PK analyses, NSAA response rate Key secondary: Safety, PD and PK analyses, investigator-assessed ORR and DOR, PFS, OS, DCR
JZP351	AML	Phase 2 • •	NCT03629171 (*MDACC*)	JZP351 + venetoclax in de novo or R/R AML[5]	Open-label, single-arm	Target enrollment: 52 Population: Pts ≥18 years of age with R/R or untreated AML	Primary: Composite CR, AEs Key secondary: EFS, OS, biomarker changes
			NCT05260528 (ALFA2101, *ALFA*)	JZP351 for newly diagnosed older adults with high-risk AML[3]	Open-label, multicenter, randomized	Target enrollment: 210 Population: Pts ≥50 years of age with newly diagnosed high-risk AML	Primary: CR+CRi with MRD status Key secondary: MRD, CR, CRi, ORR, pts proceeding to HCT, early mortality, OS, RFS, EFS, CIR, safety, hospitalizations, mutations, HRQOL
		Phase 3 • • •	NCT03897127 (AML SG30-18, *AMLSG*)	JZP351 for newly diagnosed adults with standard- and high-risk AML[3]	Open-label, randomized	Target enrollment: 882 Population: Pts ≥18 years of age with newly diagnosed standard- or high-risk AML	Primary: OS in de novo AML Key secondary: OS in all pts, EFS with CRi in de novo AML and all pts, ORR in de novo AML
	AML or MDS	Phase 2 • •	NCT03672539 (*MDACC*)	JZP351 + other approved therapies for R/R AML or MDS following HMA failure[5]	Open-label, single-arm	Target enrollment: 50 Population: Pts ≥18 years of age with CD33-positive R/R AML or high-risk MDS following HMA failure	Primary: ORR, AEs Key secondary: DOR, EFS, OS
	MDS	Phase 1 •	NCT03896269 (*MDACC*)	JZP351 low-intensity dosing for higher risk MDS[5]	Dose escalation/expansion	Target enrollment: 38 Population: Pts ≥18 years of age with MDS or CMML unfit for HCT	Primary: MTD, AEs, objective response rate Key secondary: ORR, OS, DOR, RFS
		Phase 2 • •	NCT04061239 (PALOMA, *EMSCO*)	JZP351 for high-risk MDS[3]	Direct, prospective comparison of JZP351 vs conventional care	Target enrollment: 150 Population: Pts ≥18 to ≤75 years of age with high-risk MDS	Primary: 2-year EFS Key secondary: ORR, safety, pts with MRD, pts proceeding to HCT, HRQOL

1L, first-line; 2L, second-line; ADA, anti-drug antibody; AE, adverse event; ALFA, Acute Leukemia French Association; AML, acute myeloid leukemia; AMLSG, Acute Myeloid Leukemia Study Group; BC, breast cancer; BTC, biliary tract cancer; CBR, clinical benefit rate; CIR, cumulative incidence of relapse; CMML, chronic myelomonocytic leukemia; cORR, confirmed objective response rate; CR, complete remission; CRi, complete remission with incomplete hematologic recovery; DCR, disease control rate; DLT, dose-limiting toxicity; DOR, duration of response; EFS, event-free survival; EMSCO, European Myelodysplastic Neoplasms Cooperative Group; ES, extensive stage; GEA, gastroesophageal adenocarcinoma; GI, gastrointestinal; H3, histone 3; HCT, hematopoietic cell transplantation; HER2, human epidermal growth factor receptor 2; HMA, hypomethylating agent; HR, hormone receptor; HRQOL, health-related quality of life; ICR, independent central review; IHC, immunohistochemistry; IRF, independent review facility; KRAS, Kirsten rat sarcoma virus; MAPK, mitogen-activated protein kinase; MDACC, MD Anderson Cancer Center; MDS, myelodysplastic syndrome; MRD, measurable residual disease; MRI, magnetic resonance imaging; MTD, maximum tolerated dose; NSAA, nadir serum asparaginase activity; ORR, overall response rate; OS, overall survival; pCR, pathologic complete response; PD, pharmacodynamic; PFS, progression-free survival; PK, pharmacokinetic; PRO, patient-reported outcome; pt, patient; R/R, relapsed or refractory; RANO-HGG, Response Assessment in Neuro-Oncology criteria for high-grade gliomas; RCB, residual cancer burden; RFS, relapse-free survival; RP2D, recommended phase 2 dose; SAE, serious adverse event; SCLC, small cell lung cancer; SOC, standard of care; T-DXd, trastuzumab deruxtecan; TEAE, treatment-emergent adverse event; TTR, time to response.

[1]Jazz-sponsored study; [2]Multiple trials ongoing; [3]Key Cooperative Group study; [4]Co-sponsored trial; [5]Jazz and MD Anderson Cancer Center collaboration study; [6]Partnered collaboration.

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