Jazz Pharmaceuticals to Showcase Rapidly Expanding Oncology Pipeline and Advances in Current Product Portfolio at ASCO 2023

April 27, 2023

Data from multiple studies of investigational bispecific drug zanidatamab, including pivotal Phase 2 biliary tract cancers (BTC) trial results, demonstrate breakthrough HER2-targeted pan-tumor potential

Nine abstracts accepted for presentation, including two oral presentations, reinforce Jazz's growing leadership in oncology and commitment to patients through clinically beneficial treatments and novel research

Company to host investor webcast on June 2, 2023, to review zanidatamab BTC data presentation

DUBLIN, April 27, 2023 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) today announced that the company, along with its partners, will present nine abstracts at the American Society of Clinical Oncology (ASCO) Annual Meeting from June 2-6, 2023. Presentations include clinical data from trials of zanidatamab, Zepzelca® (lurbinectedin) and Vyxeos® (daunorubicin and cytarabine).

"We are thrilled to present, together with our partner Zymeworks, an oral presentation of pivotal trial results in patients with HER2-positive BTC, an aggressive disease that currently has no FDA-approved HER2-directed treatment options," said Rob Iannone, M.D., M.S.C.E., executive vice president, global head of research and development of Jazz Pharmaceuticals. "The breadth of oncology data being presented by Jazz and our partners reflects our relentless focus on raising the standard of care for some of the most difficult-to-treat cancers."

Pivotal data from the Phase 2b study of the bispecific antibody zanidatamab in previously treated HER2-amplified BTC was selected by ASCO as an oral presentation. In addition, updated results from a Phase 1b/2 study of zanidatamab in combination with docetaxel as a first-line therapy for patients with advanced HER2-positive breast cancer will be presented by the trial sponsor, BeiGene.

Other presentations at the annual meeting feature data from our oncology pipeline and current product portfolio, across a range of solid tumors and hematological malignancies, include:

- An oral presentation from a Jazz-supported Investigator Sponsored Trial (IST) featuring results from a Phase 1b study evaluating the efficacy of lurbinectedin in combination with doxorubicin in soft-tissue sarcoma (STS), which is intended to serve as a lead-in to a randomized Phase 2 trial in leiomyosarcoma (LMS)
- A poster presentation of a post-hoc analysis using Phase 3 trial data to investigate the impact of *Vyxeos* versus 7+3 on cardiac impairment in older adults with newly diagnosed high-risk or secondary acute myeloid leukemia (AML)

The full ASCO abstracts will be available on May 25, 2023, after 5 p.m. ET. The abstract titles are available at: https://meetings.asco.org/meetings/2023-asco-annual-meeting/299/program-guide/scheduled-sessions

The full list of Jazz or partner-supported presentations at the 2023 ASCO Annual Meeting are:

Zanidatamab Presentations

Presentation Title	Author	Presentation Details
Results from the pivotal phase 2b HERIZON-BTC-01 study: Zanidatamab in previously treated HER2-amplified biliary tract cancer	Pant, S. et al.	**Type:** Oral Abstract Session **Session:** Gastrointestinal Cancer —Gastroesophageal, Pancreatic, and Hepatobiliary **Date:** Friday, June 2 (2:45 -5:45 p.m. CDT) **Abstract number**: 4008
Zanidatamab, a HER2-targeted bispecific antibody, in combination with docetaxel as first-line therapy for patients with advanced HER2-positive breast cancer: Updated results from a Phase Ib/II study	Wang, X. et al.	**Type:** Poster Session **Session:** Breast Cancer–Metastatic **Date:** June 4, 2023 (8:00 -11:00 a.m. CDT) **Abstract number**:1044

Zepzelca Presentations

Presentation Title	Author	Presentation Details
A phase III study of lurbinectedin alone or in combination with irinotecan versus investigator's choice (topotecan or irinotecan) in patients with relapsed small cell lung cancer (SCLC; LAGOON trial)	Besse, B. et al.	**Type**: Poster Session **Session:** Lung Cancer—Non-Small Cell Local-Regional/Small Cell/Other Thoracic Cancers **Date**: June 4, 2023 (8:00 -11:00 a.m. CDT) **Abstract number:** TPS8613

Efficacy and safety of lurbinectedin in elderly patients with relapsed SCLC	Cousin, S. et al.	**Type:** Poster Session **Session:** Lung Cancer—Non-Small Cell Local-Regional/Small Cell/Other Thoracic Cancers **Date:** June 4, 2023 (8:00 -11:00 a.m. CDT) **Abstract number**: 8591
IFCT-2105 LURBICLIN real-world effectiveness and treatment sequences in patients with extensive stage small cell lung cancer (ES-SCLC) who received lurbinectedin as part of the French Early Access Program (EAP-ATU)	Girard, N. et al.	**Type:** Poster Session **Session:** Lung Cancer—Non-Small Cell Local-Regional/Small Cell/Other Thoracic Cancers **Date:** June 4, 2023 (8:00 -11:00 a.m. CDT) **Abstract number**: 8584
Efficacy of combination lurbinectedin and doxorubicin from the Phase 1b soft-tissue sarcoma (STS) lead-In to a randomized Phase 2 trial in leiomyosarcoma (LMS) **[Jazz-Supported IST]**	Cote G. et al.	**Type:** Oral Abstract Session **Session:** Sarcoma **Date:** June 5, 2023 (11:30 a.m. -2:30 p.m. CDT) **Abstract number**: 11507

Vyxeos (JZP351/CPX315) Presentations

Presentation Title	Author	Presentation Details
Survival outcomes with CPX-351 vs 7+3 by baseline bone marrow blast percentage in older adults with newly diagnosed high-risk or secondary acute myeloid leukemia: A 5-year follow-up study	Ritchie, K. et al.	**Type**: Poster Session **Session:** Hematologic Malignancies —Leukemia, Myelodysplastic Syndromes, and Allotransplant **Date**: June 5, 2023 (8:00 -11:00 a.m. CT) **Abstract number:** 7027
Cardiotoxicity of CPX-351 vs 7+3 in patients with untreated high-risk acute myeloid leukemia	Mitchell, J.D. et al.	**Type:** Poster Session **Session:** Hematologic Malignancies —Leukemia, Myelodysplastic Syndromes, and Allotransplant **Date:** June 5, 2023 (8:00 -11:00 a.m. CT) **Abstract number**: 7029
UCHMC 1812: A phase 1b trial of CPX-351 plus gemtuzumab ozogamicin for relapsed/refractory acute myeloid leukemia **[Jazz-Supported IST]**	Park, S. et al.	**Type:** Poster Session **Session:** Hematologic Malignancies —Leukemia, Myelodysplastic Syndromes, and Allotransplant **Date:** June 5, 2023 (8:00 -11:00 a.m. CT) **Abstract number**: 7024

Webcast Information

The Company will host a webcast on Friday, June 2, 2023, at 6:45 p.m. CT / 7:45 p.m. ET / 12:45 a.m. IST (June 3) to provide a review of the zanidatamab BTC data presented at the 2023 ASCO Annual Meeting. Jazz senior management will discuss development and commercialization of zanidatamab, and Dr. Shubham Pant, M.D., MBBS, who is presenting the zanidatamab BTC findings at ASCO, will provide an overview of the data. Dr. Pant is a Professor in the Department of Gastrointestinal Medical Oncology with a joint appointment in the Department of Investigational Cancer Therapeutics at The University of Texas MD Anderson Cancer Center.

Interested parties may register for the call in advance here or via the Investors section of the Jazz Pharmaceuticals website at www.jazzpharmaceuticals.com. To ensure a timely connection, it is recommended that participants register at least 15 minutes prior to the scheduled webcast.

A replay of the webcast will be available via the Investors section of the Jazz Pharmaceuticals website at www.jazzpharmaceuticals.com.

About Zanidatamab
Zanidatamab is an investigational bispecific antibody, based on Zymeworks' Azymetric™ platform, that can simultaneously bind two non-overlapping epitopes of HER2, known as biparatopic binding. This unique design results in multiple mechanisms of action including dual HER2 signal blockade, increased binding and removal of HER2 protein from the cell surface, and potent effector function leading to encouraging antitumor activity in patients. Zymeworks, along with collaborators Jazz and BeiGene, Ltd. (BeiGene), are developing zanidatamab in multiple clinical trials as a targeted treatment option for patients with solid tumors that express HER2.

The U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy designation for zanidatamab in patients with previously treated HER2 gene-amplified biliary tract cancers (BTC), and two Fast Track designations for zanidatamab: one as a single agent for refractory BTC and one in combination with standard of care chemotherapy for first-line gastroesophageal adenocarcinoma (GEA). Additionally, zanidatamab has received Orphan Drug designations from FDA for the treatment of BTC and GEA, as well as Orphan Drug designation from the European Medicines Agency for the treatment of gastric cancer. Zanidatamab was also granted Breakthrough Therapy designation from the Center for Drug Evaluation (CDE) in China.

About Zepzelca® (lurbinectedin)

Zepzelca is an alkylating drug that binds guanine residues within DNA. This triggers a cascade of events that can affect the activity of DNA binding proteins, including some transcription factors, and DNA repair pathways, resulting in disruption of the cell cycle and eventual cell death.[1]

The FDA approved *Zepzelca* under accelerated approval in June 2020 for the treatment of adult patients with metastatic SCLC with disease progression on or after platinum-based chemotherapy. The approval is based on overall response rate (ORR) and duration of response demonstrated in an open-label, monotherapy clinical study. In December 2021, Jazz and PharmaMar announced the initiation of LAGOON, a confirmatory Phase 3 clinical trial of *Zepzelca* for the treatment of patients with relapsed small cell lung cancer. If positive, LAGOON could confirm the benefit of *Zepzelca* in the treatment of small cell lung cancer (SCLC) when patients progress following 1L treatment with a platinum-based regimen and support full approval in the U.S.

Zepzelca is a prescription medicine used to treat adults with SCLC that has spread to other parts of the body (metastatic) and who have received treatment with chemotherapy that contains platinum, and it did not work or is no longer working. *Zepzelca* is approved based on response rate and how long the response lasted. Additional studies will further evaluate the benefit of *Zepzelca* for this use.

Important Safety Information for ZEPZELCA

Before receiving ZEPZELCA, tell your healthcare provider about all of your medical conditions, including if you:

- have liver or kidney problems.
- are pregnant or plan to become pregnant. ZEPZELCA can harm your unborn baby.

Females who are able to become pregnant:

- Your healthcare provider should do a pregnancy test before you start treatment with ZEPZELCA.
- You should use effective birth control (contraception) during treatment with and for 6 months after your final dose of ZEPZELCA.
- Tell your healthcare provider right away if you become pregnant or think that you are pregnant during treatment with ZEPZELCA.

Males with female partners who are able to become pregnant should use effective birth control during treatment with and for 4 months after your final dose of ZEPZELCA.

Females who are breastfeeding or plan to breastfeed. It is not known if ZEPZELCA passes into your breastmilk. Do not breastfeed during treatment with ZEPZELCA and for 2 weeks after your final dose of ZEPZELCA. Talk to your healthcare provider about the best way to feed your baby during treatment with ZEPZELCA.

Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Certain other medicines may affect how ZEPZELCA works.

What should I avoid while using ZEPZELCA?

Avoid eating or drinking grapefruit, or products that contain grapefruit juice during treatment with ZEPZELCA.

ZEPZELCA can cause serious side effects, including:

- Low blood cell counts. Low blood counts including low neutrophil counts (neutropenia) and low platelet counts (thrombocytopenia) are common with ZEPZELCA, and can also be severe. Some people with low white blood cell counts may get fever, or an infection throughout the body (sepsis), that can cause death. Your healthcare provider should do blood tests before you receive each treatment with ZEPZELCA to check your blood cell counts.

Tell your healthcare provider right away if you develop:

- fever or any other signs of infection
- unusual bruising or bleeding
- tiredness
- pale colored skin
- **Liver problems**. Increased liver function tests are common with ZEPZELCA, and can also be severe. Your healthcare provider should do blood tests to check your liver function before you start and during treatment with ZEPZELCA.

Tell your healthcare provider right away if you develop symptoms of liver problems including:

- loss of appetite
- nausea or vomiting
- pain on the right side of your stomach area (abdomen)
- Your healthcare provider may temporarily stop treatment, lower your dose, or permanently stop ZEPZELCA if you develop low blood cell counts or liver problems during treatment with ZEPZELCA.

The most common side effects of ZEPZELCA include:

- Tiredness
- low white and red blood cell counts
- increased kidney function blood test (creatinine)
- increased liver function blood tests
- increased blood sugar (glucose)
- nausea
- decreased appetite
- muscle and joint (musculoskeletal) pain
- low level of albumin in the blood
- constipation
- trouble breathing
- low levels of sodium and magnesium in the blood
- vomiting
- cough
- diarrhea

These are not all of the possible side effects of ZEPZELCA.

Call your doctor for medical advice about side effects. You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088. You may also report side effects to Jazz Pharmaceuticals at 1-800-520-5568.

More information about Zepzelca, including Full Prescribing Information and Patient Information, is available here.

ZEPZELCA is a trademark of Pharma Mar, S.A. used by Jazz Pharmaceuticals under license.

About Vyxeos® (daunorubicin and cytarabine) liposome for injection
Vyxeos is a liposomal combination of daunorubicin, an anthracycline topoisomerase inhibitor, and cytarabine, a nucleoside metabolic inhibitor.

In the U.S., *Vyxeos* (daunorubicin and cytarabine) liposome for injection is indicated for the treatment of newly-diagnosed therapy-related acute myeloid leukemia (t-AML) or AML with myelodysplasia-related changes (AML-MRC) in adults and pediatric patients 1 year and older.[2]

More information about Vyxeos in the United States, including Full Prescribing Information, BOXED Warning and Medication Guide, is available here.

Important Safety Information for VYXEOS®

WARNING: VYXEOS has different dosage recommendations from other medications that contain daunorubicin and/or cytarabine. Do not substitute VYXEOS for other daunorubicin and/or cytarabine-containing products.

VYXEOS should not be given to patients who have a history of serious allergic reaction to daunorubicin, cytarabine, or any of its ingredients.

VYXEOS can cause a severe decrease in blood cells (red and white blood cells and cells that prevent bleeding, called platelets) which can result in serious infection or bleeding and possibly lead to death. Your doctor will monitor your blood counts during treatment with VYXEOS. Patients should tell the doctor about new onset fever or symptoms of infection or if they notice signs of bruising or bleeding.

VYXEOS can cause heart-related side effects. Tell your doctor about any history of heart disease, radiation to the chest, or previous chemotherapy. Inform your doctor if you develop symptoms of heart failure such as:

- shortness of breath or trouble breathing
- swelling or fluid retention, especially in the feet, ankles, or legs
- unusual tiredness

VYXEOS may cause allergic reactions including anaphylaxis. Seek immediate medical attention if you develop signs and symptoms of anaphylaxis such as:

- trouble breathing
- severe itching
- skin rash or hives
- swelling of the face, lips, mouth, or tongue

VYXEOS contains copper and may cause copper overload in patients with Wilson's disease or other copper-processing disorders.

VYXEOS can damage the skin if it leaks out of the vein. Tell your doctor right away if you experience symptoms of burning, stinging, or blisters and skin sores at the injection site.

VYXEOS can harm your unborn baby. Inform your doctor if you are pregnant, planning to become pregnant, or nursing. Do not breastfeed while receiving VYXEOS. Females and males of reproductive potential should use effective contraception during treatment and for 6 months following the last dose of VYXEOS.

The most common side effects are bleeding events, fever, rash, swelling, nausea, sores in the mouth or throat, diarrhea, constipation, muscle pain, tiredness, stomach pain, difficulty breathing, headache, cough, decreased appetite, irregular heartbeat, pneumonia, blood infection, chills, sleep

disorders, and vomiting.

Call your doctor for medical advice about side effects. You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088. You may also report side effects to Jazz Pharmaceuticals at 1-800-520-5568.

About Jazz Pharmaceuticals plc
Jazz Pharmaceuticals plc (Nasdaq: JAZZ) is a global biopharmaceutical company whose purpose is to innovate to transform the lives of patients and their families. We are dedicated to developing life-changing medicines for people with serious diseases—often with limited or no therapeutic options. We have a diverse portfolio of marketed medicines and novel product candidates, from early- to late-stage development, in neuroscience and oncology. Within these therapeutic areas, we are identifying new options for patients by actively exploring small molecules and biologics, and through innovative delivery technologies and cannabinoid science. Jazz is headquartered in Dublin, Ireland and has employees around the globe, serving patients in nearly 75 countries. Please visit www.jazzpharmaceuticals.com for more information.

Caution Concerning Forward-Looking Statements
This press release contains forward-looking statements, including, but not limited to, statements related raising the standard of care for some of the most difficult-to-treat cancer types and other statements that are not historical facts. These forward-looking statements are based on Jazz Pharmaceuticals' current plans, objectives, estimates, expectations and intentions and inherently involve significant risks and uncertainties. Actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of these risks and uncertainties, which include, without limitation, risks and uncertainties associated with pharmaceutical product development, and other risks and uncertainties affecting Jazz Pharmaceuticals and its development programs, including those described from time to time under the caption "Risk Factors" and elsewhere in Jazz Pharmaceuticals plc's Securities and Exchange Commission filings and reports (Commission File No. 001-33500), including Jazz Pharmaceuticals' Annual Report on Form 10-K for the year ended December 31, 2022 and future filings and reports by Jazz Pharmaceuticals. Other risks and uncertainties of which Jazz Pharmaceuticals is not currently aware may also affect Jazz Pharmaceuticals' forward-looking statements and may cause actual results and the timing of events to differ materially from those anticipated. The forward-looking statements herein are made only as of the date hereof or as of the dates indicated in the forward-looking statements, even if they are subsequently made available by Jazz Pharmaceuticals on its website or otherwise. Jazz Pharmaceuticals undertakes no obligation to update or supplement any forward-looking statements to reflect actual results, new information, future events, changes in its expectations or other circumstances that exist after the date as of which the forward-looking statements were made.

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References:

[1] ZEPZELCA (lurbinectedin) Prescribing Information. Palo Alto, CA: Jazz Pharmaceuticals, Inc.
[2] Vyxeos (daunorubicin and cytarabine) Prescribing Information. Palo Alto, CA: Jazz Pharmaceuticals, Inc.

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