Jazz Pharmaceuticals to Present Data Across Growing Oncology Pipeline and Portfolio at ASCO 2024

April 24, 2024

Overall survival and additional long-term follow-up data from the Phase 2b HERIZON-BTC-01 trial in previously treated HER2-positive metastatic biliary tract cancer (BTC) to be presented at ASCO 2024

DUBLIN, April 24, 2024 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) today announced that the company, along with its partners, will present or publish eight abstracts at the American Society of Clinical Oncology (ASCO) Annual Meeting from May 31-June 4, 2024 in Chicago. Presentations include data from trials of zanidatamab, Zepzelca® (lurbinectedin), and Rylaze® (asparaginase erwinia chrysanthemi (recombinant)-rywn). A poster featuring the study design for the JZP898 Phase 1 trial in progress will also be presented.

Updated data with longer follow-up, including overall survival findings, will be presented from the Phase 2b HERIZON-BTC-01 trial of zanidatamab, a HER2-targeted bispecific, biparatopic antibody, in previously treated HER2-positive biliary tract cancer (BTC). Data from the HERIZON-BTC-01 trial were the backbone of the recently completed Biologics License Application (BLA) submission for previously treated HER2-positive metastatic BTC.

"We are excited to present data across our oncology pipeline and portfolio at ASCO, particularly the opportunity to provide updated data from the pivotal Phase 2b HERIZON-BTC-01 trial of zanidatamab in HER2-positive BTC presented at last year's meeting. BTC is often associated with a poor prognosis, and we believe zanidatamab has the potential to be transformative to the current standard of care in BTC and multiple HER2-expressing cancers, including in cases resistant to prior HER2-targeted therapies," said Rob Iannone, M.D., M.S.C.E., executive vice president, global head of research and development of Jazz Pharmaceuticals. "The abstracts accepted for presentation by Jazz and our partners at this year's meeting demonstrate our commitment to advancing our portfolio of innovative oncology products and investigational therapies at all stages of development as we seek to deliver on our goal to help people with cancer live longer, fuller lives."

The full ASCO abstracts will be available on May 23, 2024, after 5 p.m. ET. The abstract titles are available at: https://www.asco.org/abstracts.

A full list of Jazz or partner-supported presentations at the 2024 ASCO Annual Meeting follows:

Zanidatamab

Presentation Title	Author	Presentation Details
Zanidatamab in previously-treated HER2+ BTC: OS and longer follow-up from the phase 2b HERIZON-BTC-01 study	Shubham Pant, Jia Fan, Do-Youn Oh, Hye Jin Choi, Jin Won Kim, Heung-Moon Chang, Lequn Bao, Hui-Chuan Sun, Teresa Macarulla, Feng Xie, Jean-Philippe Metges, Jie-Er Ying, John A Bridgewater, Mohamedtaki A Tejani, Emerson Y Chen, Harpreet Wasan, Michel Ducreux, Yi Zhao, Phillip M Garfin, James J Harding	• **Type**: Poster • **Session**: Gastrointestinal Cancer – Gastroesophageal, Pancreatic, and Hepatobiliary • **Date/Time**: Saturday, June 1, 2024, 1:30-4:30 PM CDT • **Number**: 4091

Zepzelca

Presentation Title	Author	Presentation Details
Real-world safety and Healthcare Resource Utilization (HCRU) of lurbinectedin in patients with SCLC: Jazz EMERGE 402 updated analysis	Firas Badin, Philip Lammers, Mehul Patel, Leonid Shunyakov, Dennis Slater, Catherine Labbé, Navit Naveh, Anne Boccuti, Raj Hanvesakul, Wenyan Li, Badri Rengarajan, Balazs Halmos	**Type**: ePublication **Number**: e23266
Efficacy and safety of lurbinectedin with irinotecan in a phase 2 expansion cohort of patients with synovial sarcoma (PharmaMar-supported presentation)	Gregory M Cote, Javier Martin Broto, Sant P Chawla, Bruno Bockorny, Alejandro Falcon, Antonio Lopez Pousa, Luis Paz-Ares, Benjamin Verret, Sara Martinez, Sonia Extremera, Martin Cullell-Young, Salvador Fudio, Carmen Kahatt, Ali Zeaiter, Axel Le Cesne	**Type**: Poster **Session**: Sarcoma **Date/Time**: Saturday, June 1, 2024, 1:30-4:30 PM CDT **Number**: 11560
Randomized Controlled, Open-label, Phase IIb/III Study of Lurbinectedin in Combination with Doxorubicin versus Doxorubicin Alone as First-line Treatment in Patients with Metastatic Leiomyosarcoma – *Trial in progress* (PharmaMar-supported presentation)	Gregory M Cote, Sant P Chawla, George Demetri, Bernd Kasper, Robin Jones, Javier Martin Broto, Joseph Wooley, Mia Weiss, Salvatore Tafuto, Giuseppe Badalamenti, Irene Carrasco, Paloma Peinado, Jean-Yves Blay, Gaston Boggio, Cristian Fernandez, Antonio Nieto, Carmen Kahatt, Ali Zeaiter, Axel Le Cesne	**Type**: Poster **Session**: Sarcoma **Date/Time**: Saturday, June 1, 2024, 1:30-4:30 PM CDT **Number**: TPS11590
A phase II study of lurbinectedin ± avelumab in small cell carcinoma of the bladder (LASER) – *Trial in Progress* [IST]	Nicholas Simon, Elias Chandran, Scot Niglio, Andre R Kydd, Saad Atiq, Lisa Ley, Lisa Cordes, Tzu-fang Wang, Salah Boudjadi, Elizabeth Smith, Dilara Akbulut, Seth Steinberg, Andrea B Apolo	**Type**: Poster **Session**: Genitourinary Cancer – Kidney and Bladder

		Date/Time: Sunday, June 2, 2024, 9:00 AM - 12:00 PM CDT **Number**: TPS4629
Efficacy and safety of lurbinectedin in combination with irinotecan in patients with relapsed SCLC: results from a phase 2 expansion cohort (PharmaMar-supported presentation)	Luis Paz-Ares, Alejandro Falcon, Alejandro Navarro, Amparo Sanchez, Gregory M Cote, Javier Molina-Cerrillo, Vicente Alonso, Javier Baena, Ana Gil, Santiago Ponce-Aix, Helena Bote de Cabo, Sara Martinez, Julia Martinez, Jose Antonio López-Vilariño, Carmen Kahatt, Ali Zeaiter, Javier Gomez, Jon Zugazagoitia	**Type**: Poster **Session**: Lung Cancer – Non-Small Cell Local-Regional/Small Cell/Other Thoracic Cancers **Date/Time**: Monday, June 3, 2024, 1:30-4:30 PM CDT **Number**: 8094

JZP898

Presentation Title	Author	Presentation Details
A phase 1/1b first-in-human (FIH) study of JZP898 as monotherapy and in combination with pembrolizumab in adult patients with advanced or metastatic solid tumors – *Trial in Progress*	Meredith McKean, Thomas Hutson, Sarina A Piha-Paul, Ida Micaily, Mingxiang Liao, Robin Humphreys, Graham Brock, Natasha Sahr, Vian Amber, Alexander Spira	**Type**: Poster **Session**: Developmental Therapeutics—Molecularly Targeted Agents and Tumor Biology **Date/Time**: Saturday, June 1, 2024. 9:00 AM–12:00 PM CDT **Number**: TPS3190

Rylaze

Presentation Title	Author	Presentation Details
Recombinant *Erwinia* asparaginase (JZP458) in acute lymphoblastic leukemia/lymphoblastic lymphoma (ALL/LBL): post hoc analysis of adverse events of interest from AALL1931	Etsuko Aoki, Luke Maese, Mignon L Loh, Shirali Agarwal, Vijayalakshmi Chandrasekaran, Yali Liang, Nalina Dronamraju, Robert Iannone, Lewis B Silverman, Elizabeth A Raetz, Rachel E Rau	**Type**: Poster **Session**: Hematologic Malignancies – Leukemia, Myelodysplastic Syndromes, and Allotransplant **Date/Time**: Monday, June 3, 2024, 9:00 AM–12:00 PM CDT **Number**: 6521

About Zanidatamab
Zanidatamab is an investigational bispecific antibody that can simultaneously bind two non-overlapping epitopes of HER2, known as biparatopic binding. This unique design and increased binding results in multiple mechanisms of action, including dual HER2 signal blockade, removal of HER2 protein from the cell surface, and immune-mediated cytotoxicity leading to encouraging antitumor activity in patients. Zanidatamab is being developed in multiple clinical trials as a targeted treatment option for patients with solid tumors that express HER2. Zanidatamab is being developed by Jazz and BeiGene, Ltd. (BeiGene) under license agreements from Zymeworks, which first developed the molecule.

The U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy designation for zanidatamab development 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 1L 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 BTC and 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 last 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 last 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 last 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, Seville oranges, or products that contain grapefruit juice and Seville oranges 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 JZP898
JZP898 (also known as WTX-613) is an investigational differentiated, conditionally-activated interferon alpha (IFNα) INDUKINE™ molecule. JZP898 is an engineered IFNα2b cytokine pro-drug that is activated specifically within the tumor microenvironment where it can stimulate IFNα receptors on cancer-fighting immune effector cells. JZP898 was created leveraging Werewolf Therapeutics' proprietary PREDATOR™ protein engineering technology, which integrates specialized protein design elements to enhance activity, stability and tumor selectivity within a single molecule, called INDUKINE molecules.

About RYLAZE® (asparaginase erwinia chrysanthemi (recombinant)-rywn)
RYLAZE, also known as JZP458, is approved in the U.S. for use as a component of a multi-agent chemotherapeutic regimen for the treatment of acute lymphoblastic leukemia (ALL) and lymphoblastic lymphoma (LBL) in adult and pediatric patients one month or older who have developed hypersensitivity to *E. coli*-derived asparaginase. RYLAZE has orphan drug designation for the treatment of ALL/LBL in the United States. RYLAZE is a recombinant erwinia asparaginase that uses a novel *Pseudomonas fluorescens* expression platform. JZP458 was granted Fast Track designation by the U.S. Food and Drug Administration (FDA) in October 2019 for the treatment of this patient population. RYLAZE was approved as part of the Real-Time Oncology Review program, an initiative of the FDA's Oncology Center of Excellence designed for efficient delivery of safe and effective cancer treatments to patients.[4]

The full U.S. Prescribing Information for RYLAZE is available at: **https://pp.jazzpharma.com/pi/rylaze.en.USPI.pdf**

Important Safety Information for RYLAZE

RYLAZE should not be given to people who have had:

- Serious allergic reactions to RYLAZE
- Serious swelling of the pancreas (stomach pain), serious blood clots, or serious bleeding during previous asparaginase treatment

RYLAZE may cause serious side effects, including:

- Allergic reactions (a feeling of tightness in your throat, unusual swelling/redness in your throat and/or tongue, rash, or trouble breathing), some of which may be life-threatening
- Swelling of the pancreas (stomach pain), which, if left untreated, may be fatal
- Blood clots (may be experienced as headache, arm or leg swelling, shortness of breath, or chest pain), which may be life-threatening
- Bleeding, which may be life-threatening
- Liver problems

Contact your doctor immediately if any of these side effects occur.

Some of the most common side effects with RYLAZE include: liver problems, nausea and vomiting, bone and muscle pain, infection, tiredness, headache, fever with low white blood cell count, fever, bleeding, mouth swelling (sometimes with sores), pain in the abdomen, decreased appetite, allergic reactions, high blood sugar levels, diarrhea, swelling of the pancreas, and low levels of potassium in your blood.

RYLAZE can harm your unborn baby. Inform your doctor if you are pregnant, planning to become pregnant, or nursing. Females of reproductive potential should use effective contraception (other than hormonal contraceptives) during treatment and for 3 months following the final dose. Do not breastfeed while receiving RYLAZE and for 1 week after the final dose.

Tell your healthcare provider if there are any side effects that are bothersome or that do not go away.

These are not all the possible side effects of RYLAZE. For more information, ask your healthcare provider.

Call your doctor for medical advice about any 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 (1-800-332-1088).

About Jazz Pharmaceuticals
Jazz Pharmaceuticals plc (Nasdaq: JAZZ) is a global biopharma 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, including leading therapies for sleep disorders and epilepsy, and a growing portfolio of cancer treatments. Our patient-focused and science-driven approach powers pioneering research and development advancements across our robust pipeline of innovative therapeutics in oncology and neuroscience. Jazz is headquartered in Dublin, Ireland with research and development laboratories, manufacturing facilities and employees in multiple countries committed to serving patients worldwide. Please visit www.jazzpharmaceuticals.com for more information.

Jazz Pharmaceuticals plc Caution Concerning Forward-Looking Statements
This press release contains forward-looking statements, including, but not limited to, statements related to growing our portfolio of innovative oncology products and investigational therapies at all stages of development as we seek to deliver on our goal to help people with cancer live longer, fuller lives, 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, 2023, 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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