Jazz Pharmaceuticals Advances Recombinant Crisantaspase Development Program

August 6, 2019

Positive Phase 1 study of JZP-458 completed
Pivotal Phase 2/3 study expected to initiate later this year in collaboration with Children's Oncology Group

DUBLIN, Aug. 6, 2019 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) today announced that the Phase 1 study of its recombinant crisantaspase molecule, JZP-458, met its efficacy and safety objectives. The company plans to initiate a single-arm, pivotal Phase 2/3 study evaluating JZP-458 as a potential treatment option for patients with acute lymphoblastic leukemia (ALL) or lymphoblastic lymphoma (LBL) who are hypersensitive to *E. coli*-derived asparaginase products.

"Jazz is committed to the ALL patient community, and we are pleased to advance this development program with the goal of bringing a new treatment option to ALL and LBL patients who are hypersensitive to *E. coli*-derived asparaginase products as soon as possible," said Robert Iannone, M.D., M.S.C.E., executive vice president, research and development of Jazz Pharmaceuticals. "Following a meeting with the U.S. Food and Drug Administration, we are finalizing the Phase 2/3 study protocol in collaboration with the Children's Oncology Group and plan to initiate the study later this year."

A recombinant crisantaspase Phase 1 study in healthy volunteers in the U.S. met safety and efficacy objectives with efficacy based on measurement of serum asparaginase activity levels. Results of this Phase 1 study will be submitted for presentation at an upcoming medical meeting.

About JZP-458
JZP-458 is a recombinant crisantaspase that uses a novel *Pseudomonas fluorescens* expression platform. It is being developed for use as a component of a multi-agent chemotherapeutic regimen in the treatment of pediatric and adult patients with acute lymphoblastic leukemia (ALL) or lymphoblastic lymphoma (LBL) who are hypersensitive to *E. coli*-derived asparaginase products. A Phase 1 study of healthy volunteers was recently completed, and a single-arm, pivotal Phase 2/3 study is planned for initiation later in 2019.

About Acute Lymphoblastic Leukemia (ALL)
Acute Lymphoblastic Leukemia (ALL) is a cancer of the blood and bone marrow that can progress quickly if not treated.[1] Leukemia is the most common cancer in children, and about three out of four of these cases are ALL.[2] Adults can also develop ALL, and about four of every 10 cases of ALL diagnosed are in adults.[3]The American Cancer Society estimates that almost 6,000 new cases of ALL will be diagnosed in the U.S. in 2019.[4] Asparaginase is a core component of multi-agent chemotherapeutic regimens in ALL.[5] However, asparaginase treatments derived from *E. coli* are associated with the development of hypersensitivity reactions.[6]

About Jazz Pharmaceuticals plc
Jazz Pharmaceuticals plc (Nasdaq: JAZZ), a global biopharmaceutical company, is dedicated to developing life-changing medicines for people with limited or no options. As a leader in sleep medicine and with a growing hematology/oncology portfolio, Jazz has a diverse portfolio of products and product candidates in development, and is focused on transforming biopharmaceutical discoveries into novel medicines. Jazz Pharmaceuticals markets Sunosi™ (solriamfetol), Xyrem® (sodium oxybate) oral solution, Defitelio® (defibrotide sodium), Erwinaze® (asparaginase *Erwinia chrysanthemi*) and Vyxeos® (daunorubicin and cytarabine) liposome for injection in the U.S. and markets Defitelio® (defibrotide), Erwinase® and Vyxeos® 44 mg/100 mg powder for concentrate for solution for infusion in countries outside the U.S. For country-specific product information, please visit www.jazzpharmaceuticals.com/medicines. For more information, please visit www.jazzpharmaceuticals.com and follow us on Twitter at @JazzPharma.

"Safe Harbor" Statement under the Private Securities Litigation Reform Act of 1995
This press release contains forward-looking statements, including, but not limited to, statements related to the company's plans regarding its recombinant crisantaspase development program, including plans to initiate a single-arm, pivotal Phase 2/3 study later this year evaluating JZP-458 as a potential treatment option for patients with ALL or LBL who have hypersensitivity to *E. coli*-derived asparaginase products, the company's goal to bring a new treatment option to such ALL and LBL patients as soon as possible, and other statements that are not historical facts. These forward-looking statements are based on the company's 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 clinical success thereof, including risks related to failure or delays in completing clinical trials; the regulatory approval process; and other risks and uncertainties affecting the company and its development programs, including those described from time to time under the caption "Risk Factors" and elsewhere in Jazz Pharmaceuticals plc'sSecurities and Exchange Commission filings and reports (Commission File No. 001-33500), including the company's Quarterly Report on Form 10-Q for the quarter ended March 31, 2019 and future filings and reports by the company, including the company's Quarterly Report on Form 10-Q for the quarter ended June 30, 2019. Other risks and uncertainties of which the company is not currently aware may also affect the company's 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 the company on its website or otherwise. The company 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.

References:

[1] National Cancer Institute. Adult Acute Lymphoblastic Leukemia Treatment (PDQ®)–Patient Version. Available at www.cancer.gov/types/leukemia/patient/adult-all-treatment-pdq. Accessed August 6, 2019.

[2] American Cancer Society. Key Statistics for Childhood Leukemia. Available at www.cancer.org/cancer/leukemia-in-children/about/key-statistics.html.

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[3] American Cancer Society. Key Statistics for Acute Lymphocytic Leukemia (ALL). Available at www.cancer.org/cancer/acute-lymphocytic-leukemia/about/key-statistics.html. Accessed August 6, 2019.

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[5] Salzer W, Bostrom B, Messinger Y et al. 2018. Asparaginase activity levels and monitoring in patients with acute lymphoblastic leukemia. *Leukemia & Lymphoma*. 59:8, 1797-1806, DOI: 10.1080/10428194.2017.1386305.

[6] Hijiya N, van der Sluis IM. Asparaginase-associated toxicity in children with acute lymphoblastic leukemia. *Leuk Lymphoma*. 2016;57(4):748–757. doi:10.3109/10428194.2015.1101098

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SOURCE Jazz Pharmaceuticals plc

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