Pfizer Announces Phase 3 Top-Line Results for Rivipansel in Patients with Sickle Cell Disease Experiencing a Vaso-Occlusive Crisis

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Pfizer Inc. (NYSE:PFE) announced today that the Phase 3 Rivipansel (GMI-1070): E valuating Safety, Efficacy and Time to Discharge (RESET) pivotal study did not meet its primary or key secondary efficacy endpoints. The objective of the trial was to evaluate the efficacy and safety of rivipansel in patients aged six and older with sickle cell disease (SCD) who were hospitalized for a vaso-occlusive crisis (VOC) and required treatment with intravenous (IV) opioids. The primary endpoint was time to readiness-for-discharge and the key secondary efficacy endpoints were time-to-discharge, cumulative IV opioid consumption, and time to discontinuation of IV opioids.

“We are disappointed with the results, as we have been working in close partnership with the SCD community to advance rivipansel as a potential treatment option for acute VOC. We plan to share the study data at an upcoming scientific meeting as we want to ensure the learnings from this trial help inform future sickle cell programs that aim to improve care for SCD patients experiencing a VOC,” said Brenda Cooperstone, M.D., Senior Vice President and Chief Development Officer, Rare Disease, Pfizer Global Product Development. “We express our sincere gratitude to everyone who made this study possible, including the study investigators, and in particular, the patients and their families."

SCD is a debilitating blood disorder, characterized by acute pain crises or VOC. Treatment options for patients seeking medical care for a VOC are currently limited to symptomatic management with analgesics (including opioids and NSAIDs) and hydration, underscoring a significant need for new treatment options.

“We recognize this is a significant setback for the SCD community, who are eagerly awaiting new treatment options, and we share in their disappointment,” said Freda Lewis-Hall, M.D., DFAPA, Chief Patient Officer and Executive Vice President, Pfizer Inc. “Many of us have witnessed first-hand the devastating impact of SCD on patients and their families, but we have also been moved by their incredible strength and bravery, and we will continue to support this courageous community.”

Detailed analyses of the RESET study, including additional data on efficacy and safety endpoints, which are not available at this time, will be submitted for presentation at a future scientific meeting.

About RESET

RESET (B5201002) was a Phase 3, multicenter, randomized, double-blind, placebo-controlled, parallel-group study that evaluated the efficacy and safety of rivipansel in patients with SCD aged six and older, who were hospitalized for a VOC and required treatment with intravenous opioids. The primary endpoint of the study was time-to-readiness for discharge, defined as the difference between the start time and date of the first infusion of study drug and the time and date of medical staff-assessed readiness-for-discharge.

The RESET trial included 345 patients who were randomized1:1 to receive rivipansel or placebo, administered intravenously every 12 hours to a maximum of 15 doses. All study participants were followed for safety for 35 days after their last dose of study drug.

Eligible patients who completed the RESET trial were able to enter an open-label extension study (B5201003) and receive rivipansel for subsequent VOC episodes over an 18-month period. For additional information about the study, please visit https://www.clinicaltrials.gov.

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Rivipansel is an investigational treatment for VOC in people with SCD and not approved for use. In 2011, GlycoMimetics and Pfizer Inc. entered into a worldwide license agreement for the development and, if approved by applicable regulatory authorities, commercialization of rivipansel. Since completion of the Phase 2 clinical trial, Pfizer has been responsible for clinical development of rivipansel, including the RESET clinical trial.

About Sickle Cell Disease and Vaso-occlusive Crisis

SCD is the most common inherited blood disorder in the United States, impacting approximately 100,000 people.1 Worldwide, approximately 100 million people carry the SCD trait and an estimated five million live with the disease.1 While the majority of people with SCD are of African descent, the disease can affect all ethnic groups, especially those from areas where malaria is or was endemic, such as Africa, the Middle East, India and the Southern Mediterranean.

Acute pain crises or VOC are the most common clinical manifestation of SCD. A VOC occurs when sickled red blood cells irritate the lining of blood vessels and cause an inflammatory response leading to vascular occlusion, tissue ischemia and pain.

Pfizer Rare Disease

Rare disease includes some of the most serious of all illnesses and impacts millions of patients worldwide, representing an opportunity to apply our knowledge and expertise to help make a significant impact on addressing unmet medical needs. The Pfizer focus on rare disease builds on more than two decades of experience, a dedicated research unit focusing on rare disease, and a global portfolio of multiple medicines within a number of disease areas of focus, including rare hematologic, neurologic, cardiac and inherited metabolic disorders.

Pfizer Rare Disease combines pioneering science and deep understanding of how diseases work with insights from innovative strategic collaborations with academic researchers, patients, and other companies to deliver transformative treatments and solutions. We innovate every day leveraging our global footprint to accelerate the development and delivery of groundbreaking medicines and the hope of cures.

Click here to learn more about our Rare Disease portfolio and how we empower patients, engage communities in our clinical development programs, and support programs that heighten disease awareness.

Pfizer Inc.: Breakthroughs that change patients’ lives

At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety and value in the discovery, development and manufacture of health care products, including innovative medicines and vaccines. Every day, Pfizer colleagues work across developed and emerging markets to advance wellness, prevention, treatments and cures that challenge the most feared diseases of our time. Consistent with our responsibility as one of the world's premier innovative biopharmaceutical companies, we collaborate with

health care providers, governments and local communities to support and expand access to reliable, affordable health care around the world. For more than 150 years, we have worked to make a difference for all who rely on us. We routinely post information that may be important to investors on our website at www.pfizer.com. In addition, to learn more, please visit us on www.pfizer.com and follow us on Twitter at @Pfizer and @Pfizer_News, LinkedIn, YouTube and like us on Facebook at Facebook.com/Pfizer.

DISCLOSURE NOTICE: The information contained in this release is as of August 2, 2019. Pfizer assumes no obligation to update forward-looking statements contained in this release as the result of new information or future events or developments.

This release contains forward-looking information about the top-line results from the Phase 3 RESET pivotal study evaluating the efficacy and safety of rivipansel in patients aged six and older with sickle cell disease and Pfizer’s rare disease portfolio that involves substantial risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statements. Risks and uncertainties include, among other things, the uncertainties inherent in research and development, including the ability to meet anticipated clinical endpoints, commencement and/or completion dates for our clinical trials, regulatory submission dates, regulatory approval dates and/or launch dates, as well as the possibility of unfavorable new clinical data and further analyses of existing clinical data; the risk that clinical trial data are subject to differing interpretations and assessments by regulatory authorities; whether regulatory authorities will be satisfied with the design of and results from our clinical studies; whether and when any drug applications may be filed in any jurisdictions for any rare disease product candidates; whether and when any such applications may be approved by regulatory authorities, which will depend on myriad factors, including making a determination as to whether the product's benefits outweigh its known risks and determination of the product's efficacy and, if approved, whether such product candidates will be commercially successful; decisions by regulatory authorities impacting labeling, manufacturing processes, safety and/or other matters that could affect the availability or commercial potential of any rare disease product candidates; competitive developments; continuing evaluation of the estimated costs and benefits associated with the rivipansel program; potential impact of unanticipated costs, expenses, and liabilities; and risks and uncertainties generally characterizing developing new biopharmaceutical products, including those described under the heading “Risk Factors” in Pfizer’s Annual Report on Form 10-K for the fiscal year ended December 31, 2018, which may be found at www.sec.gov and www.pfizer.com.

A further description of risks and uncertainties can be found in Pfizer's Annual Report on Form 10-K for the fiscal year ended December 31, 2018 and in its subsequent reports on Form 10-Q, including in the sections thereof captioned "Risk Factors" and "Forward-Looking Information and Factors That May Affect Future Results", as well as in its subsequent reports on Form 8-K, all of which are filed with the U.S. Securities and Exchange Commission and available at www.sec.gov and www.pfizer.com.

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1 Yale SH1, Nagib N, Guthrie T. Approach to the vaso-occlusive crisis in adults with sickle cell disease. Am Fam Physician. 2000 Mar 1;61(5):1349-56, 1363-4.

Media Contact: Steve Danehy 212-733-1538Steven.Danehy@pfizer.com Investor Contact: Chuck Triano 212-733-3901Charles.E.Triano@pfizer.com