



# U.S. FDA Grants Priority Review for Pfizer's New Drug Application for Glasdegib in Patients with Previously Untreated Acute Myeloid Leukemia

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Submission Based on Data from Randomized Phase 2 Trial, Which Showed Glasdegib in Combination with Chemotherapy Nearly Doubled Overall Survival Compared to Chemotherapy Alone

Pfizer Inc. (NYSE:PFE) today announced that the U.S. Food and Drug Administration (FDA) accepted the company's New Drug Application and granted Priority Review designation for glasdegib, an investigational oral smoothened (SMO) inhibitor, being evaluated for the treatment of adult patients with previously untreated acute myeloid leukemia (AML) in combination with low-dose cytarabine (LDAC), a type of chemotherapy.

"Patients with acute myeloid leukemia who are ineligible for intensive chemotherapy are in critical need of new treatment options to improve their overall survival," said Mace Rothenberg, M.D., chief development officer, Oncology, Pfizer Global Product Development. "In an investigational Phase 2 study, glasdegib in combination with low-dose cytarabine showed a significant improvement in overall survival compared to patients who received low-dose cytarabine alone. Glasdegib is the first smoothened inhibitor to potentially offer such a benefit to patients with acute myeloid leukemia, and we are proud that our application was accepted by the FDA for Priority Review."

The FDA grants Priority Review designation to medicines that may offer significant advances in treatment or may provide a treatment where no adequate therapy exists. The Prescription Drug User Fee Act (PDUFA) goal date for a decision by the FDA is in December 2018.

The submission is based on results from the Phase 2 BRIGHT 1003 study, a randomized, open-label, multicenter trial investigating glasdegib combined with LDAC (n=88) versus LDAC alone (n=44) in 132 patients with previously untreated AML or high-risk myelodysplastic syndrome (MDS) who were not eligible for intensive chemotherapy. Results demonstrated a significant improvement in the primary endpoint of overall survival (OS). Median OS was 8.8 months for patients treated with glasdegib plus LDAC compared with 4.9 months for patients treated with LDAC only. This difference represented a 49.9 percent reduction in the risk of death for patients treated with glasdegib plus LDAC (HR: 0.501, 95% CI: 0.334, 0.752, one-sided p-value 0.0003). The BRIGHT 1003 results were presented in 2016 at the 58th American Society of Hematology Annual Meeting.

The most frequently ( $\geq 30\%$  of patients) reported adverse events (AEs) in patients treated with glasdegib plus LDAC compared to LDAC alone were anemia (45% vs 42%), febrile neutropenia (36% vs 27%), nausea (36% vs 12%), decreased appetite (32% vs 12%), fatigue (31% vs 20%) and thrombocytopenia (30% vs 27%). The most frequently ( $\geq 15\%$  of patients) reported serious AEs for patients treated with glasdegib plus LDAC compared to LDAC alone were febrile neutropenia (29% vs 20%) and pneumonia (21% vs 17%).

### About Glasdegib

Glasdegib is an investigational, oral, once-daily therapy that is thought to inhibit the SMO receptor, thereby disrupting the Hedgehog pathway. Abnormal Hedgehog pathway activation is thought to play a role in the development of multiple types of cancers, including solid tumors and hematologic malignancies. It has not received regulatory approval in any country.

The Phase 3 BRIGHT AML 1019 trial (NCT03416179), which is evaluating the addition of glasdegib to intensive or non-intensive chemotherapy in patients with newly diagnosed AML, began enrolling earlier this year.

### About Acute Myeloid Leukemia

Acute myeloid leukemia (AML) is the most common type of acute leukemia in adults and accounts for approximately 80 percent of all cases of acute leukemia.<sup>1</sup> An estimated 19,520 people are expected to be diagnosed with AML in the U.S. in 2018.<sup>1</sup> Despite recent advancements, only approximately one in four patients with AML survive longer than five years, and additional treatment options are needed to reduce incidence of disease progression and relapse.<sup>2,3</sup> This is especially true for patients who are unable to receive intensive chemotherapy and are triaged to other treatments associated with

poorer outcomes.

## About Pfizer Oncology

Pfizer Oncology is committed to pursuing innovative treatments that have a meaningful impact on people living with cancer. Our growing pipeline of biologics, small molecules, and immunotherapies is focused on identifying and translating the best scientific breakthroughs into clinical application for patients across a diverse array of solid tumors and hematologic cancers. Today, we have 10 approved oncology medicines and 14 assets currently in clinical development. By maximizing our internal scientific resources and collaborating with other companies, government and academic institutions, as well as patients and non-profit and professional organizations, we are bringing together the brightest and most enterprising minds to take on the toughest cancers. Together we can accelerate breakthrough treatments to patients around the world and work to redefine life with cancer.

Pfizer Inc.: Working together for a healthier world®

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. Our global portfolio includes medicines and vaccines as well as many of the world's best-known consumer health care products. 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](http://www.pfizer.com). In addition, to learn more, please visit us on [www.pfizer.com](http://www.pfizer.com) and follow us on Twitter at @Pfizer and @Pfizer\_News, LinkedIn, YouTube, and like us on Facebook at [Facebook.com/Pfizer](https://www.facebook.com/Pfizer).

**DISCLOSURE NOTICE:** The information contained in this release is as of June 27, 2018. 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 a product candidate, glasdegib, and Pfizer Oncology, including their potential benefits, 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 trial commencement and completion dates and regulatory submission dates, as well as the possibility of unfavorable clinical trial results, including unfavorable new clinical data and additional analyses of existing clinical data; the risk that clinical trial data are subject to differing interpretations, and, even when we view data as sufficient to support the safety and/or effectiveness of a product candidate, regulatory authorities may not share our views and may require additional data or may deny approval altogether; whether regulatory authorities will be satisfied with the design of and results from our clinical studies; whether and when new drug applications may be filed in any other jurisdictions for glasdegib or for any other oncology products; whether and when the new drug application for glasdegib pending with the FDA or any such other applications may be approved by regulatory authorities, which will depend on the assessment by such regulatory authorities of the benefit-risk profile suggested by the totality the efficacy and safety information submitted, and, if approved, whether glasdegib or any such other oncology products will be commercially successful; decisions by regulatory authorities regarding labeling and other matters that could affect the availability or commercial potential of glasdegib or any other oncology products; and competitive developments.

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, 2017 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](http://www.sec.gov) and [www.pfizer.com](http://www.pfizer.com).

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1 American Cancer Society. Key statistics for acute myeloid leukemia. Available at <https://www.cancer.org/cancer/acute-myeloid-leukemia/about/key-statistics.html>. Accessed January 23, 2018.

2 SEER Cancer Stat Facts: Acute Myeloid Leukemia. National Cancer Institute. Bethesda, MD, April 2017. Available at: <http://seer.cancer.gov/statfacts/html/amyl.html>. Accessed January 23, 2018.

3 Dombret H, Seymour JF, Butrym A, et al. International phase 3 study of azacitidine vs conventional care regimens in older patients with newly diagnosed AML with >30% blasts. Blood 2015; 126(3): 291-9.

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