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BESPONSA® Approved in the EU for Adult Patients with Relapsed or Refractory B-cell Precursor Acute Lymphoblastic Leukemia

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Pfizer Inc. (NYSE:PFE) today announced that the European Commission has approved BESPONSA® (inotuzumab ozogamicin) as monotherapy for the treatment of adults with relapsed or refractory CD22-positive B-cell precursor acute lymphoblastic leukemia (ALL). This indication includes treatment of adults with Philadelphia chromosome positive (Ph+) as well as Philadelphia chromosome negative (Ph-) relapsed or refractory B-cell precursor ALL. Adults with Ph+ relapsed or refractory CD22-positive B-cell precursor ALL should have failed treatment with at least one tyrosine kinase inhibitor (TKI). With this approval, BESPONSA becomes the first and only antibody drug conjugate (ADC) available for patients with this type of leukemia in the European Union (EU).

“The European Commission’s approval of BESPONSA represents an important milestone for patients, the oncology community and Pfizer,” said Andreas Penk, M.D., regional president, Pfizer Oncology. “This is the first approval for BESPONSA and provides patients in the EU, who are battling an especially hard-to-treat leukemia, with a new treatment option beyond chemotherapy.”

ALL is an aggressive type of leukemia that can be fatal within a matter of months if left untreated.1 The goal of treatment in relapsed or refractory (resistant) ALL is to achieve complete remission without excessive toxicity so patients may proceed to additional therapeutic intervention, particularly stem cell transplant, which is the most recognized option to prolong patient survival, maintenance therapy or other therapy.2 In adult patients with relapsed or refractory ALL, median overall survival is just three to six months.3,4,5 The current standard of care is intensive chemotherapy6, which is effective in less than 50 percent of relapsed or refractory patients and associated with poor long-term survival, high toxicities, lengthy inpatient stays and continuous infusions.7

“Acute lymphoblastic leukemia that has recurred or is refractory following first-line therapy is a rare and rapidly progressive disease with poor prognosis,” said Professor David Marks, Department of Hematology, University Hospitals Bristol NHS Foundation Trust, Bristol, United Kingdom. “The approval of BESPONSA (inotuzumab ozogamicin) provides a much needed treatment option for physicians and patients alike, that may help improve outcomes for some of the most vulnerable leukemia patients in Europe.”

The European Commission’s approval of BESPONSA is supported by results from the Phase 3 INO-VATE ALL trial, in which 326 adult patients with relapsed or refractory B-cell precursor ALL were enrolled and which compared BESPONSA to standard of care chemotherapy. The INO-VATE ALL study had two primary endpoints, complete response with or without hematologic recovery (CR/CRi) and overall survival (OS). Results from the trial were published in The New England Journal of Medicine in June 2016.

In the U.S., BESPONSA received Breakthrough Therapy designation from the Food and Drug Administration (FDA) in October 2015 for ALL. A Biologics License Application (BLA) for BESPONSA for the treatment of adult patients with relapsed or refractory B-cell precursor ALL was accepted for filing and granted Priority Review by the FDA in February 2017. The Prescription Drug User Fee Act (PDUFA) goal date for a decision by the FDA is August 2017.

With a growing hematology pipeline, Pfizer is committed to extending therapeutic progress in acute and chronic leukemias that leverage select pathways and mechanism of actions (MOAs). Specifically, our investigational products aim to treat some of the hardest to treat leukemias and lymphomas including, acute myeloid leukemia (AML), acute lymphoblastic leukemia (ALL), chronic myeloid leukemia (CML) and mantle cell lymphoma (MCL).

Indication for BESPONSA® (Inotuzumab Ozogamicin) in the EU

BESPONSA is approved as monotherapy for the treatment of adults with relapsed or refractory CD22-positive B-cell precursor ALL in the EU. Adult patients with Ph+ relapsed or refractory B-cell precursor ALL should have failed treatment with at least one TKI.

Important Safety Information for BESPONSA® (Inotuzumab Ozogamicin) in the EU

The most common (? 20%) adverse reactions associated with BESPONSA were thrombocytopenia (51%), neutropenia (49%), infection (48%), anaemia (36%), leukopenia (35%), fatigue (35%), haemorrhage (33%), pyrexia (32%), nausea (31%), headache (28%), febrile neutropenia (26%), increased transaminases (26%), abdominal pain (23%), increased gamma-glutamyltransferase (21%), and hyperbilirubinaemia (21%).

The most common (? 2%) serious adverse reactions associated with BESPONSA were infection (23%), febrile neutropenia (11%), haemorrhage (5%), abdominal pain (3%), pyrexia (3%), veno-occlusive disease/sinusoidal obstruction syndrome (VOD/SOS) (2%), and fatigue (2%).

In the Phase 3 INO-VATE ALL trial (N=164 patients treated with BESPONSA), VOD/SOS was reported in 22 (13%) patients including 5 (3%) patients during study therapy or in follow-up without an intervening hematopoietic stem cell transplant (HSCT). Among the 77 patients who proceeded to a subsequent HSCT (6 of whom received additional salvage therapy after treatment with BESPONSA before proceeding to HSCT), VOD/SOS was reported in 17 (22%) patients. Five of the 17 VOD/SOS events that occurred post-HSCT were fatal.

VOD/SOS was reported up to 56 days after the last dose of BESPONSA without an intervening HSCT. The median time from HSCT to onset of VOD/SOS was 15 days (range: 3-57 days). Of the 5 patients who experienced VOD/SOS during treatment with BESPONSA but without an intervening HSCT, 2 patients had also received an HSCT before BESPONSA treatment.

Among patients who proceeded to HSCT after BESPONSA treatment, VOD/SOS was reported in 5/11 (46%) patients who received an HSCT both prior to and after BESPONSA treatment and 12/66 (18%) patients who only received an HSCT after BESPONSA treatment.

The EU Summary of Product Characteristics (SmPC) is available at http://www.ema.europa.eu.

About BESPONSA® (Inotuzumab Ozogamicin)

BESPONSA is an antibody-drug conjugate (ADC) comprised of a monoclonal antibody (mAb) targeting CD22, a cell surface antigen expressed on cancer cells in almost all B-ALL patients, linked to a cytotoxic agent.8 When BESPONSA binds to the CD22 antigen on B-cells, it is internalized into the cell, where the cytotoxic agent calicheamicin is released to destroy the cell.9

BESPONSA originates from a collaboration between Pfizer and Celltech, now UCB. Under the terms of this agreement, Pfizer has sole responsibility for all manufacturing and clinical development activities for this molecule. Pfizer also collaborated with SFJ Pharmaceuticals Group (SFJ) on the registrational program (INO-VATE ALL) for BESPONSA.

About Pfizer Oncology

Pfizer Oncology is committed to pursuing innovative treatments that have a meaningful impact on those living with cancer. As a leader in oncology speeding cures and accessible breakthrough medicines to patients, Pfizer Oncology is helping to redefine life with cancer. Our strong pipeline of biologics, small molecules and immunotherapies, one of the most robust in the industry, is studied with precise focus on identifying and translating the best scientific breakthroughs into clinical application for patients across a wide range of cancers. By working collaboratively with academic institutions, individual researchers, cooperative research groups, governments and licensing partners, Pfizer Oncology strives to cure or control cancer with its breakthrough medicines. Because Pfizer Oncology knows that success in oncology is not measured solely by the medicines you manufacture, but rather by the meaningful partnerships you make to have a more positive impact on people’s lives.

DISCLOSURE NOTICE: The information contained in this release is as of June 30, 2017. 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 BESPONSA (inotuzumab ozogamicin), and an approval by the European Commission as monotherapy for the treatment of adults with relapsed or refractory CD22-positive B-cell precursor acute lymphoblastic leukemia, including its 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, uncertainties regarding the commercial success of BESPONSA; 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; whether and when applications for BESPONSA may be filed in any other jurisdictions; whether and when the BLA pending in the United States and any other such applications that may be pending or filed for BESPONSA may be approved by the FDA or other regulatory authorities, respectively, which will depend on the assessment by such regulatory authorities of the benefit-risk profile suggested by the totality of the efficacy and safety information submitted; decisions by regulatory authorities regarding labeling and other matters that could affect the availability or commercial potential of BESPONSA; 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, 2016 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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