

Basel, 28 July 2017

## **FDA grants breakthrough therapy designation for Venclexta in acute myeloid leukaemia**

- **First in class BCL2-specific oral inhibitor represents a potential new way of treating acute myeloid leukaemia (AML), the most common type of aggressive leukaemia in adults<sup>1</sup>**
- **17<sup>th</sup> breakthrough therapy designation for Roche's portfolio of medicines and 4<sup>th</sup> for Venclexta**

Roche (SIX: RO, ROG; OTCQX: RHHBY) today announced that the US Food and Drug Administration (FDA) has granted breakthrough therapy designation for Venclexta® (venetoclax) in combination with low dose cytarabine (LDAC) for elderly patients with previously untreated AML who are ineligible for intensive chemotherapy. FDA breakthrough therapy designation is intended to expedite the development and review of medicines with early evidence of potential clinical benefit in serious or life-threatening diseases and to help ensure that patients receive access to medicines as soon as possible. This is the seventeenth breakthrough therapy designation for Roche's portfolio of medicines, and the fourth for Venclexta.

Venclexta is being developed by AbbVie and Roche. It is jointly commercialised by AbbVie and Genentech, a member of the Roche Group, in the United States and commercialised by AbbVie outside of the United States.

Breakthrough therapy designation was granted based on data from an ongoing open-label phase Ib study of Venclexta in combination with LDAC in previously untreated elderly patients (over 65 years) with AML, an aggressive form of leukaemia, who are ineligible for intensive chemotherapy. Preliminary data from this study (M14-387) presented at the 22<sup>nd</sup> European Hematology Association (EHA) Annual Congress, 22-25 June, in Madrid 2017 (Abstract E911) showed durable efficacy with an acceptable safety profile for Venclexta in combination with LDAC in this patient group.

### **About AML**

AML is an aggressive form of leukaemia that starts in immature forms of blood-forming cells, known as myeloid cells, found in the bone marrow.<sup>2</sup> AML is the most common type of aggressive leukaemia in adults.<sup>1</sup>

It has one of the lowest survival rates of all types of leukaemia<sup>3</sup>. Even with the best available therapies, older patients aged 65 and over have survival rates comparable to patients with advanced lung cancer, with a five year overall survival rate of <5%.<sup>4,5</sup> Approximately 20,000 people in the United States and 18,000 in Europe are diagnosed with AML each year.<sup>6,7</sup>

### **About Venclexta**

Venclexta is a small molecule designed to selectively bind and inhibit the BCL-2 protein, which plays an important role in a process called apoptosis (programmed cell death). It is believed that blocking BCL-2 may restore the signalling system that tells cells, including cancer cells, to self-destruct.

Venclexta is being co-developed by AbbVie and Roche. Together, the companies are committed to research with Venclexta, which is currently being evaluated in phase III clinical trials for the treatment of relapsed, refractory and previously untreated chronic lymphocytic leukaemia, along with studies in several other cancers including AML. Venclexta is commercialised jointly by AbbVie and Genentech, a member of the Roche Group, in the United States and commercialised by AbbVie outside of the United States.

### **About Roche in haematology**

For more than 20 years, Roche has been developing medicines that redefine treatment in haematology. Today, we are investing more than ever in our effort to bring innovative treatment options to people with diseases of the blood. In addition to approved medicines MabThera®/Rituxan® (rituximab), Gazyva®/Gazyvaro® (obinutuzumab), and Venclexta®/Venclyxto® (venetoclax) in collaboration with AbbVie, Roche's pipeline of investigational haematology medicines includes TECENTRIQ® (atezolizumab), an anti-CD79b antibody drug conjugate (polatuzumab vedotin/RG7596) and a small molecule antagonist of MDM2 (idasanutlin/RG7388). Roche's dedication to developing novel molecules in haematology expands beyond malignancy, with the development of the investigational haemophilia A treatment emicizumab.

### **About Roche**

Roche is a global pioneer in pharmaceuticals and diagnostics focused on advancing science to improve people's lives. The combined strengths of pharmaceuticals and diagnostics under one roof have made Roche the leader in personalised healthcare – a strategy that aims to fit the right treatment to each patient in the best way possible.

Roche is the world's largest biotech company, with truly differentiated medicines in oncology, immunology, infectious diseases, ophthalmology and diseases of the central nervous system. Roche is also the world leader in in vitro diagnostics and tissue-based cancer diagnostics, and a frontrunner in diabetes management. Founded in 1896, Roche continues to search for better ways to prevent, diagnose and treat diseases and make a sustainable contribution to society. The company also aims to improve patient access to medical innovations by working with all relevant stakeholders. Thirty medicines developed by Roche are included in the World Health Organization Model Lists of Essential Medicines, among them life-saving antibiotics, antimalarials and cancer medicines. Roche has been recognised as the Group Leader in sustainability within the Pharmaceuticals, Biotechnology & Life Sciences Industry eight years in a row by the Dow Jones Sustainability Indices (DJSI).

The Roche Group, headquartered in Basel, Switzerland, is active in over 100 countries and in 2016 employed more than 94,000 people worldwide. In 2016, Roche invested CHF 9.9 billion in R&D and posted sales of CHF 50.6 billion. Genentech, in the United States, is a wholly owned member of the Roche Group. Roche is the majority shareholder in Chugai Pharmaceutical, Japan. For more information, please visit [www.roche.com](http://www.roche.com).

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## References

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<sup>1</sup> National Cancer Institute: Adult Acute Myeloid Leukemia Treatment (PDQ)<sup>®</sup>–Patient Version. Available at: [https://www.cancer.gov/types/leukemia/patient/adult-aml-treatment-pdq#link/\\_1](https://www.cancer.gov/types/leukemia/patient/adult-aml-treatment-pdq#link/_1). Last accessed: July 2017.

<sup>2</sup> American Cancer Society: What is acute myeloid leukemia? Available at: <http://www.cancer.org/cancer/leukemia-acute/myeloidaml/detailedguide/leukemia-acute-myeloid-myelogenous-what-is-aml>. Last accessed: July 2017.

<sup>3</sup> Leukemia & Lymphoma Society: Facts and statistics overview – Leukemia. Available at: <http://www.lls.org/http%3A//llsorg.prod.acquia-sites.com/facts-and-statistics/facts-and-statistics-overview/facts-and-statistics#Leukemia>. Last accessed: July 2017.

<sup>4</sup> Sekeres MA. Treatment Of Older Adults With Acute Myeloid Leukemia: State Of The Art And Current Perspectives. *Haematologica* 2008; 93: 1769-1772

<sup>5</sup> Cancer Research UK: Survival statistics for acute myeloid leukaemia (AML). Available at: [http://www.cancerresearchuk.org/about-cancer/acute-myeloid-leukaemia-aml/survival?\\_ga=2.239561667.1384102361.1500450925-993068745.1500450925](http://www.cancerresearchuk.org/about-cancer/acute-myeloid-leukaemia-aml/survival?_ga=2.239561667.1384102361.1500450925-993068745.1500450925). Last accessed: July 2017.

<sup>6</sup> AML Global Portal. Disease Overview. Available at: <http://www.amlglobalportal.com/disease-overview>. Last accessed: July 2017.

<sup>7</sup> Adult acute myeloid leukemia treatment - for health professionals (PDQ<sup>®</sup>), 2015. Available at: <http://www.cancer.gov/types/leukemia/hp/adult-aml-treatment-pdq>. Last accessed: July 2017.