

PRESS RELEASE

Basilea reports start of first-in-human phase 1 oncology study with oral panRAF kinase inhibitor

Basel, Switzerland, May 4, 2015 – Basilea Pharmaceutica Ltd. (SIX: BSLN) reported today the start of a clinical phase 1 study with the orally administered investigational drug BAL3833/CCT3833 in patients with advanced solid tumors. BAL3833/CCT3833 is the lead compound of a series of small molecule panRAF inhibitors recently in-licensed by Basilea.

The open-label dose-escalation phase 1 study is designed to investigate the safety and tolerability of once-daily oral doses of BAL3833/CCT3833 in adult patients with advanced solid tumors. The study aims to establish a safe maximum tolerated dose for future clinical studies.

“A significant range of cancers are associated with mutations in key factors involved in the transmission of growth signals, such as the RAF kinases, leading to uncontrolled tumor growth,” said Dr. Laurenz Kellenberger, Basilea’s Chief Scientific Officer. “The BRAF kinase, for example, is mutated in a range of cancers including 50% of melanomas. Preclinical data show that our novel panRAF kinase inhibitors are able to block growth of BRAF mutant melanoma cells *in vitro* and *in vivo*. This includes activity in tumor models resistant to currently marketed anti-cancer drugs targeting the BRAF pathway. Moreover, as these panRAF kinase inhibitors also target other growth modulators relied upon by drug-resistant tumor cells and non-melanoma cancers, it is anticipated that their spectrum of activity will extend beyond currently available BRAF inhibitors. This supports the potential for use of these panRAF inhibitors in the treatment of expanded melanoma patient populations as well as other cancer types.”

Prof. Achim Kaufhold, Basilea’s Chief Medical Officer, said: “The initiation of the first clinical study with this promising compound is a significant development milestone and we are very pleased to add BAL3833/CCT3833 as the second oncology drug in clinical stage to our expanding oncology development portfolio.”

The study is sponsored by The Institute of Cancer Research, London, and The Royal Marsden NHS Foundation Trust and is being carried out at The Royal Marsden, and The Christie NHS Foundation Trust in Manchester, with biomarker work by the Cancer Research UK Manchester Institute at The University of Manchester and Basilea. The study will be funded by the Wellcome Trust, the NIHR Biomedical Research Centre at The Royal Marsden and The Institute of Cancer Research, The Christie charity and the Cancer Research UK Manchester Institute. Basilea will assume full operational responsibility after phase 1.

About BAL3833

BAL3833 (also known as CCT3833) is an orally available small-molecule panRAF kinase inhibitor targeting certain cell proliferation signaling pathways that are associated with tumor growth. It is the lead compound of a series of panRAF inhibitors recently in-licensed by Basilea under an agreement with The Institute of Cancer Research, London, Cancer Research Technology, the Wellcome Trust, and The University of Manchester. The compound originates from research at The Institute of Cancer Research and the Cancer Research UK Manchester Institute, by scientists funded by Cancer Research UK and the Wellcome Trust. These panRAF kinase inhibitors target both BRAF and the growth pathways that tumor cells rely on when they become resistant to existing drugs targeting BRAF. BRAF is mutated in a range of cancers including melanomas, colorectal and serous ovarian cancer. Data from preclinical studies suggest that compounds

from this class are active in tumors derived from patients which have developed resistance to currently available RAF pathway inhibitors.¹

About Wellcome Trust

The Wellcome Trust is a global charitable foundation dedicated to improving health. It provides more than GBP 700 million a year to support bright minds in science, the humanities and the social sciences, as well as education, public engagement and the application of research to medicine. Its GBP 18 billion investment portfolio gives it the independence to support such transformative work as the sequencing and understanding of the human genome, research that established front-line drugs for malaria, and Wellcome Collection, its free venue for the incurably curious that explores medicine, life and art.

About The Institute of Cancer Research, London

The Institute of Cancer Research, London, is one of the world's most influential cancer research institutes. Scientists and clinicians at The Institute of Cancer Research (ICR) are working every day to make a real impact on cancer patients' lives. Through its unique partnership with The Royal Marsden NHS Foundation Trust and "bench-to-bedside" approach, the ICR is able to create and deliver results in a way that other institutions cannot. Together the two organisations are rated in the top four cancer centres globally. The ICR has an outstanding record of achievement dating back more than 100 years. It provided the first convincing evidence that DNA damage is the basic cause of cancer, laying the foundation for the now universally accepted idea that cancer is a genetic disease. Today it leads the world at isolating cancer-related genes and discovering new targeted drugs for personalized cancer treatment. As a college of the University of London, the ICR provides postgraduate higher education of international distinction. It has charitable status and relies on support from partner organisations, charities and the general public. The ICR's mission is to make the discoveries that defeat cancer. For more information visit www.icr.ac.uk

About The Royal Marsden NHS Foundation Trust

The Royal Marsden opened its doors in 1851 as the world's first hospital dedicated to cancer diagnosis, treatment, research and education. Today, together with its academic partner, The Institute of Cancer Research (ICR), it is the largest and most comprehensive cancer centre in Europe treating over 50,000 NHS and private patients every year. It is a centre of excellence with an international reputation for groundbreaking research and pioneering the very latest in cancer treatments and technologies. The Royal Marsden, with the ICR, is the only National Institute for Health Research Biomedical Research Centre for Cancer. First awarded the status in 2006, it was re-awarded in 2011. A total of GBP 62 million is being provided over five years, to support pioneering research work, and is being shared out over eight different cancer themes. Since 2004, the hospital's charity, The Royal Marsden Cancer Charity, has helped raise over GBP 100 million to build theatres, diagnostic centres, and drug development units. For more information visit www.royalmarsden.nhs.uk

About The University of Manchester

The University of Manchester, a member of the prestigious Russell Group of British universities, is the largest and most popular university in the UK. It has 20 academic schools and hundreds of specialist research groups undertaking pioneering multi-disciplinary teaching and research of worldwide significance. The University of Manchester is one of the country's major research institutions, rated fifth in the UK in terms of 'research power', and has had no fewer than 25 Nobel laureates either work or study there. The University had an annual income of GBP 886 million in 2013/14. Cancer is one of The University of Manchester's research beacons - examples of pioneering discoveries, interdisciplinary collaboration and cross-sector partnerships that are

tackling some of the biggest questions facing the planet. For more information visit www.manchester.ac.uk/research/beacons/cancer/

About Cancer Research UK Manchester Institute

Cancer Research UK Manchester Institute is a world leading cancer research institute within The University of Manchester and is core-funded by Cancer Research UK, the largest independent cancer research organisation in the world. It is at the heart of the Manchester Cancer Research Centre, a dynamic partnership between CRUK, The University of Manchester and The Christie Hospital NHS Foundation Trust that integrates cancer research across Manchester. Research at CRUK MI spans the whole spectrum of cancer research, from programmes investigating the molecular and cellular basis of cancer, to those focused on translational research and the development of novel therapeutics. Strong links with The Christie Hospital NHS Foundation Trust, Western Europe's largest specialist cancer hospital, provide exceptional opportunities for interactions between basic and clinical research teams, and facilitating rapid translation of fundamental research findings into patient benefit.

About Cancer Research UK

Cancer Research UK is the world's leading cancer charity dedicated to saving lives through research. Cancer Research UK's pioneering work into the prevention, diagnosis and treatment of cancer has helped save millions of lives. Cancer Research UK receives no government funding for its life-saving research. Every step it makes towards beating cancer relies on every pound donated. Cancer Research UK has been at the heart of the progress that has already seen survival rates in the UK double in the last forty years. Today, 2 in 4 people survive cancer. Cancer Research UK's ambition is to accelerate progress so that 3 in 4 people will survive cancer within the next 20 years. Cancer Research UK supports research into all aspects of cancer through the work of over 4,000 scientists, doctors and nurses. Together with its partners and supporters, Cancer Research UK's vision is to bring forward the day when all cancers are cured. For further information about Cancer Research UK's work or to find out how to support the charity, please call +44 (0)300 123 1022 or visit www.cancerresearchuk.org. Follow Cancer Research UK on Twitter and Facebook.

About Cancer Research Technology

Cancer Research Technology (CRT) is a specialist commercialisation and development company, which aims to develop new discoveries in cancer research for the benefit of cancer patients. CRT works closely with leading international cancer scientists and their institutes to protect intellectual property arising from their research and to establish links with commercial partners. CRT facilitates the discovery, development and marketing of new cancer therapeutics, vaccines, diagnostics and enabling technologies. CRT is a wholly owned subsidiary of Cancer Research UK, the world's leading cancer charity dedicated to saving lives through research. Further information about CRT can be found at www.cancertechnology.com.

About The Christie NHS Foundation Trust

The Christie specialise in cancer treatment, research and education. It is the first UK centre to be accredited as a comprehensive cancer centre. It is the largest single site cancer centre in Europe treating more than 40,000 patients a year. It serves an immediate population of 3.2 million in the Greater Manchester and Cheshire area and also delivers a number of regional and national services from the main Christie site. Its patients are at the heart of everything that The Christie does. As a centre of excellence The Christie focuses solely on improving outcomes for oncology patients. It is able to provide services based on expert staff and a specialised infrastructure dedicated to the delivery of cancer treatment, care, research and education. Its focus and size enables it to uniquely deliver effective and efficient specialist care offering

patients the best possible outcomes from its research programme. Its charity, which is one of the largest in the UK, provides enhanced services over and above what the NHS funds. It has over 30,000 supporters, who helped raise a record breaking GBP 14.8m last year, with 83p in every pound going directly to patients.

About Basilea

Basilea Pharmaceutica Ltd. is a biopharmaceutical company developing products that address increasing resistance and non-response to current treatment options in the therapeutic areas of bacterial infections, fungal infections and cancer. The company uses the integrated research, development and commercial operations of its subsidiary Basilea Pharmaceutica International Ltd. to develop and commercialize innovative pharmaceutical products to meet the medical needs of patients with serious and potentially life-threatening conditions. Basilea Pharmaceutica Ltd. is headquartered in Basel, Switzerland and listed on the SIX Swiss Exchange (SIX: BSLN). Additional information can be found at Basilea's website www.basilea.com.

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This communication expressly or implicitly contains certain forward-looking statements concerning Basilea Pharmaceutica Ltd. and its business. Such statements involve certain known and unknown risks, uncertainties and other factors, which could cause the actual results, financial condition, performance or achievements of Basilea Pharmaceutica Ltd. to be materially different from any future results, performance or achievements expressed or implied by such forward-looking statements. Basilea Pharmaceutica Ltd. is providing this communication as of this date and does not undertake to update any forward-looking statements contained herein as a result of new information, future events or otherwise.

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This press release can be downloaded from www.basilea.com.

References

- 1 M. R. Girotti et al., Paradox-breaking RAF inhibitors that also target SRC are effective in drug-resistant BRAF mutant melanoma, *Cancer Cell* 2015 (27), 85-96