

PRESS RELEASE

Basilea in-licenses targeted cancer therapy

Basel, Switzerland, April 1, 2015 – Basilea Pharmaceutica Ltd. (SIX: BSLN) reports today that it has entered into a license agreement for novel panRAF kinase inhibitors with a consortium of organizations including The Institute of Cancer Research, London, Cancer Research Technology, the Wellcome Trust and The University of Manchester.

The agreement grants Basilea exclusive worldwide rights to develop, manufacture and commercialize novel panRAF kinase inhibitors which originate from research at The Institute of Cancer Research by scientists funded by Cancer Research UK and the Wellcome Trust. RAF kinases play an important role in tumor cell proliferation. The oral, small molecule panRAF inhibitors target BRAF and other growth pathways relied upon by resistant tumor cells. These properties allow anti-cancer activity in a range of tumor models including tumors resistant to anti-BRAF therapy associated with a number of marketed anti-cancer drugs.¹ The lead compound is anticipated to start clinical phase 1 testing in 2015.

Under the terms of the agreement, the consortium will lead clinical phase 1 development for the lead compound. Basilea will assume full operational responsibility thereafter. The consortium receives an upfront payment and is eligible to potential milestone payments on achievement of pre-specified clinical, regulatory and commercial milestones, as well as tiered royalties on future net sales.

Professor Caroline Springer, Professor of Biological Chemistry at The Institute of Cancer Research, London, said: "I'm delighted by today's announcement, which is excellent news for research into treatments for drug-resistant cancers. The agreement provides the foundation for the clinical development of this exciting new drug class. It is an important milestone in efforts to tackle resistance to existing cancer therapies and provide new options for cancer patients."

"We are excited about complementing our growing and maturing oncology pipeline with this novel program including a lead compound expected to enter clinical testing in 2015. The available data show that this novel class of panRAF inhibitors are active in tumors which have developed resistance to currently available RAF kinase inhibitors and have the potential to offer new treatment options for melanoma as well as additional cancer indications," said Dr. Laurenz Kellenberger, Basilea's Chief Scientific Officer.

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 <http://www.icr.ac.uk>

About Cancer Research Technology

Cancer Research Technology (CRT) is a specialist commercialization 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 wholly owned by 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 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 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 UK'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. www.manchester.ac.uk

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.

<http://www.manchester.ac.uk/research/beacons/cancer/>

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 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 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 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