



Press Release

Biocon, Quark Announce Initiation of Pivotal Phase II/III Study of QPI-1007 in Rare Eye Disease in India First siRNA Drug Trial Approved By DCGI in India

Bengaluru, India, and Fremont, CA, June 23, 2016

Biocon Ltd. (BSE code: 532523, NSE: BIOCON), Asia's premier biopharmaceutical company, and Quark Pharmaceuticals, Inc., a leader in the discovery and development of novel RNA interference (RNAi)-based therapeutics, announced today the randomization of the first patient in India in the pivotal global Phase II/III study of QPI-1007, a novel siRNA (small interfering RNA) drug candidate for ocular neuroprotection.

Biocon and its partner, Quark Pharmaceuticals, have received approval from the Drug Controller General of India (DCGI) to proceed with the study, the first ever clinical trial of a siRNA therapy in India.

The QPK207 study will determine the effect of QPI-1007 on visual function in subjects with acute non-arteritic ischemic optic neuropathy (NAION), which is a rare ocular disorder with an unmet need globally. This is part of a global Phase II/III study run by Quark in collaboration with "Neuro-Ophthalmology Research Disease Investigator Consortium" (NORDIC) and is already enrolling in the US and a number of other countries.

Biocon Chairperson & Managing Director Kiran Mazumdar-Shaw said: *"Biocon is committed to develop innovative therapies that address unmet medical needs and the initiation of the Phase II/III study investigating QPI-1007 in NAION in India is an important step towards this goal. India has a significant NAION patient population and we are pleased to be the first biopharma company in the country to provide an siRNA-based therapy that is likely to benefit thousands of patients who either have no access to treatment or cannot afford it."*

"siRNA therapeutics is a new class of breakthrough medicines, with the potential to revolutionize drug development and offer therapeutic precision and widespread applicability and we are delighted to initiate QPK207 in India in this multi-national



study to seek a treatment for NAION, a debilitating condition," stated **Dr. Daniel Zurr, Chairman and CEO of Quark**. "QPI-1007 represents a novel therapeutic strategy for treating NAION and future plans are to develop it for additional optic neuropathies, including glaucoma, which, similar to NAION, are characterized by the death of retinal ganglion cells."

"It takes strong partnerships to translate novel science into commercial therapies to help people and we are extremely pleased to have partnered with Biocon, an innovative company best positioned to lead the siRNA based drug class development in India," added **Dr. Zurr**.

Biocon and Quark Pharma had entered into a licensing and collaboration agreement in 2013 to co-develop, manufacture and commercialize QPI-1007 in India and other key markets.

About RNAi

RNA interference (RNAi) is a universal mechanism within living cells that employs non-coding RNA to control which genes are active and how active they are. This natural mechanism, which was discovered in 1998 and was awarded the Nobel Prize in 2006, has already revolutionized experimental biology and currently holds the highest promise for therapeutic purposes. Various types of short double-stranded RNAs act as effector molecules of the RNAi mechanism. Some of them, targeting specific genes in a sequence-dependent manner and inhibiting their expression, are called short interfering RNAs (siRNAs). siRNAs can be designed based on the sequence information of virtually any gene, produced synthetically and used as drugs. siRNA drugs can cause inhibition of expression of any gene, regardless of its traditional attribution to potentially "druggable" or "non-druggable" targets. These drugs are highly specific and potentially safer than small molecule therapies which are known to exhibit some degree of promiscuity. Quark's siRNA platform includes proprietary siRNA compound structures and chemical modifications with improved pharmacological properties, while the Company's strong IP portfolio provides freedom to operate in the siRNA space.

About QPI-1007

QPI-1007 is a double stranded RNA molecule chemically modified by Quark's proprietary technology. The drug is designed to temporarily inhibit the expression of caspase 2 and thereby block the apoptotic death of retinal ganglion cells. QPI-1007 is being developed as a neuroprotectant for the treatment of NAION and in the future other optic neuropathies such as glaucoma that result in the death of retinal ganglion cells (RGCs). QPI-1007 has been evaluated in a human, dose escalation, Phase I/IIa Study (Protocol QRK007), delivered by



single intravitreal injection to Optic Nerve Atrophy patients with low visual acuity and thereafter in acute NAION patients. This study was conducted at 22 sites in the US and 6 sites in Israel. This study showed that a single intravitreal injection of QPI-1007 was well tolerated in subjects with long-standing low vision or acute NAION. The drug has also demonstrated protective activity compared to historical data. QPI-1007 was also studied in a Phase IIa clinical trial in acute angle closure glaucoma patients in the United States, Vietnam and Singapore.

About the Study

This study is a pivotal Phase II/III, randomized, double masked, sham-controlled trial of QPI-1007 delivered by multi-dose intravitreal injections to subjects with acute non-arteritic anterior ischemic optic neuropathy (NAION) to compare the safety and efficacy of two doses of QPI-1007 along with the sham group. This multi-national, multi-centric trial is being conducted across 95 hospitals in several countries including India, US, Israel, Germany, Australia, Italy and China. Approximately 465 patients will be enrolled globally. The treatment for each patient will be 12 months.

About Quark Pharmaceuticals

Quark Pharmaceuticals, Inc., the world leader in novel therapeutic RNAi discovery and development, has the largest clinical-stage siRNA pipeline in the industry. The Company's fully integrated drug development platform spans therapeutic target identification to drug development. Quark's approach to delivery allows targeting of tissues and organs including the eye, kidney, ear, lung, skin, spinal cord and brain. Quark has three siRNA product candidates in clinical development in five different disease indications of which two are in pivotal Phase III studies. Quark's Joint Venture in China, Kunshan Ribo-Quark Pharmaceutical Inc, and its strategic partner in India, Biocon Limited, are part of Quark's worldwide clinical studies network. Quark is headquartered in Fremont, California and operates research and development facilities in Ness-Ziona, Israel. For additional information please visit: www.quarkpharma.com.

About Biocon

Biocon Limited, publicly listed in 2004, (BSE code: 532523, NSE Id: BIOCON, ISIN Id: INE376G01013) is India's largest and fully-integrated, innovation-led biopharmaceutical company. As an emerging global biopharmaceutical enterprise serving customers in over 100 countries, it is committed to reduce therapy costs of chronic diseases like autoimmune, diabetes, and cancer. Through innovative products and research services it is enabling access to affordable healthcare for patients, partners and healthcare systems across the globe. It has successfully developed and taken a range of Novel Biologics, Biosimilars, differentiated Small Molecules and affordable Recombinant Human Insulin and Analogs from 'Lab to Market'. Some of its key brands are INSUGEN® (rh-insulin), BASALOG®



(Glargine), CANMAb™ (Trastuzumab), BIOMAb-EGFR™ (Nimotuzumab) and ALZUMAb™ (Itolizumab), a 'first in class' anti-CD6 monoclonal antibody. It has a rich pipeline of Biosimilars and Novel Biologics at various stages of development including Insulin Tregopil, a high potential oral insulin analog. Visit: www.biocon.com

Forward Looking Statement

Certain statements in this release concerning our future growth prospects are forward-looking statements, which are subject to a number of risks, uncertainties and assumptions that could cause actual results to differ materially from those contemplated in such forward-looking statements. Important factors that could cause actual results to differ materially from our expectations include, amongst others general economic and business conditions in India, our ability to successfully implement our strategy, our research and development efforts, our growth and expansion plans and technological changes, changes in the value of the Rupee and other currency changes, changes in the Indian and international interest rates, change in laws and regulations that apply to the Indian and global biotechnology and pharmaceuticals industries, increasing competition in and the conditions of the Indian biotechnology and pharmaceuticals industries, changes in political conditions in India and changes in the foreign exchange control regulations in India. Neither our company, our directors, nor any of our affiliates, have any obligation to update or otherwise revise any statements reflecting circumstances arising after this date or to reflect the occurrence of underlying events, even if the underlying assumptions do not come to fruition.

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