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Genkyotex Announces Investigator-Initiated Phase 2 Clinical Trial to Evaluate 48-week Treatment with GKT831 in Patients with Type 1 Diabetes and Kidney Disease

Genkyotex (Euronext Paris & Brussels: FR00011790542 – GKTX), a biopharmaceutical company and the leader in NOX therapies, announced today that world-renowned diabetes experts Professor Mark Cooper, Head of the Department of Diabetes at Monash University, and Professor Jonathan Shaw, Deputy Director (Clinical and Population Health) at the Baker Heart and Diabetes Institute in Melbourne, Australia, will lead the conduct of a phase 2 clinical trial to evaluate the efficacy and safety of the Company's lead product candidate, GKT831, in patients with type 1 diabetes and kidney disease (diabetic kidney disease).

This investigator-initiated study will be based at the Baker Institute and will include multiple study sites across Australia. This research is financially supported by JDRF Australia, the recipient of the Australian Research Council Special Research Initiative in Type 1 Juvenile Diabetes funding, with additional financial support by the Baker Institute. Genkyotex shall provide GKT831 Good Manufacturing Practice (GMP) material for the trial. The trial is expected to begin patient enrollment during the second half of 2017.

Diabetic kidney disease is a fibrotic disorder where progressive glomerulosclerosis and interstitial fibrosis leads to end stage renal disease. GKT831 is a NOX 1 and 4 enzyme inhibitor that has shown potent anti-fibrotic activity in a broad range of preclinical models including several DKD models [1-4]. In a previous, short-term phase 2 trial in patients with type 2 diabetes and kidney disease, GKT831 demonstrated an excellent safety profile and achieved statistically significant reductions in several secondary efficacy endpoints. However, improvements in albuminuria, the study's primary efficacy endpoint, was not achieved after 12 weeks of treatment.

The Baker Institute study will be a placebo-controlled, double blind, randomized, parallel group phase 2 trial to evaluate the effect of oral GKT831 on the urine albumin-to-creatinine ratio (UACR) in patients with type 1 diabetes and persistent albuminuria despite treatment with optimal standard of care. The primary endpoint of the study will be UACR difference between means at the end of treatment period of 48 weeks, adjusted for baseline. A key secondary endpoint of the study will be the effect of GKT831 on renal function, as defined by changes in estimated glomerular filtration rate. Patients will receive 200mg of oral GKT831 or placebo twice a day for 48 weeks. A total of 142 patients are planned to be enrolled into the study at up to 15 investigational centers in Australia.

Professor Cooper has stated that *"We are very excited to be commencing this study which arises in part from original research performed in our laboratories and which was initially supported by JDRF. This work is a classic example of bench to bedside clinical translation. We appreciate JDRF greatly assisting us in providing us with an opportunity to bring this new treatment forward for what is a major burden of T1D kidney disease."*

“We are delighted to be working with Professor Cooper and his team to pursue the clinical evaluation of GKT831 in this severe diabetic complication,” said Dr. Philippe Wiesel, chief medical officer of Genkyotex. *“The design of this phase 2 trial was informed by previous phase 2 results in patients with type 2 diabetes and kidney disease performed by Genkyotex, in particular the extended 48-week treatment duration, a more homogenous and earlier stage patient population, and a higher dose throughout the dosing period. We also wish to thank JDRF for supporting this study, as well as previous preclinical studies, which has enabled a number of investigators to evaluate GKT831’s impact on ophthalmic, vascular, and renal complications caused by type 1 diabetes.”*

References:

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2. Gray SP et al. Combined NOX1/4 inhibition with GKT137831 in mice provides dose-dependent reno- and atheroprotection even in established micro- and macrovascular disease. *Diabetologia.* 2017 May;60(5):927-937. doi: 10.1007/s00125-017-4215-5.
3. Jha JC et al. Genetic targeting or pharmacologic inhibition of NADPH oxidase nox4 provides renoprotection in long-term diabetic nephropathy. *J Am Soc Nephrol.* 2014 Jun;25(6):1237-54. doi: 10.1681/ASN.2013070810. Epub 2014 Feb 7.
4. You YH. Metabolomics Reveals a Key Role for Fumarate in Mediating the Effects of NADPH Oxidase 4 in Diabetic Kidney Disease. *J Am Soc Nephrol.* 2016 Feb;27(2):466-81. doi: 10.1681/ASN.2015030302.

About Genkyotex

Genkyotex is the leading biopharmaceutical company in NOX therapies. Listed on the Euronext Paris and Euronext Brussels markets, Genkyotex is established in France and, via its GenKyoTex Suisse SA subsidiary, in Switzerland. A leader in NOX therapies, its unique therapeutic approach is based on a selective inhibition of NOX enzymes that amplify multiple disease processes such as fibrosis, inflammation, pain processing, cancer development, and neurodegeneration.

Genkyotex’s platform enables the identification of orally available small-molecules that selectively inhibit specific NOX enzymes. Genkyotex is developing a pipeline of first-in-class product candidates targeting one or multiple NOX enzymes. The lead product candidate, GKT831, a NOX1 and NOX4 inhibitor is expected to enter a phase II clinical trial in primary biliary cholangitis (PBC, a fibrotic orphan disease) during the first half of 2017. This product candidate may also be active in other fibrotic indications. Its second product candidate, GKT771, is a NOX1 inhibitor targeting multiple pathways in angiogenesis, pain processing, and inflammation, and should enter a phase I clinical study at the end of the second half of 2017.

Genkyotex also has a versatile platform, Vaxiclase, that is particularly well-suited to the development of various immunotherapies. A partnership covering the use of Vaxiclase as an antigen per se (GTL003) has been established with Serum Institute of India Ltd (Serum Institute), the world’s largest producer of vaccine doses. This agreement covers territories outside the United States and Europe, and could generate up to \$57 million in revenues for Genkyotex, before royalties on sales. It will enable Serum Institute to develop acellular multivalent combination vaccines against a variety of infectious diseases, including whooping cough. The last preclinical milestone foreseen in the agreement was reached in November 2016, opening the path to formal preclinical testing prior to potential clinical development and subsequent commercialization.

For further information, please go to www.genkyotex.com.

About JDRF and type 1 diabetes

JDRF is the leading global organisation funding type 1 diabetes (T1D) research. JDRF Australia is built on a grassroots model of people connecting in their local communities, collaborating regionally for efficiency and broader fundraising impact, and uniting on an international stage to pool resources, passion and energy. Our mission is to accelerate life-changing breakthroughs to cure, prevent and treat T1D and its complications. To accomplish this, JDRF has invested nearly \$2 billion since our inception. We collaborate with academic institutions, policymakers, and corporate and industry partners to develop and deliver a pipeline of

innovative therapies to people living with T1D. Our staff and volunteers in seven countries are dedicated to advocacy, community engagement and our vision of a world without T1D. For more information, please visit jdrf.org.au.

About the Australian Type 1 Diabetes Clinical Research Network (T1DCRN)

The Type 1 Diabetes Clinical Research Network (T1DCRN) is an innovative clinical research program led by JDRF Australia and funded by the Australian Government through the Australian Research Council (ARC) Special Research Initiatives scheme. The T1DCRN's goal is to accelerate patient benefit through supporting the most promising research projects, promoting and retaining outstanding scientists and attracting new researchers to the field of type 1 diabetes research. For more information, please visit t1dcrn.org.au.



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This press release may contain forward-looking statements by the company with respect to its objectives. Such statements are based upon the current beliefs, estimates and expectations of Genkyotex's management and are subject to risks and uncertainties such as the company's ability to implement its chosen strategy, customer market trends, changes in technologies and in the company's competitive environment, changes in regulations, clinical or industrial risks and all risks linked to the company's growth. These factors as well as other risks and uncertainties may prevent the company from achieving the objectives outlined in the press release and actual results may differ from those set forth in the forward-looking statements, due to various factors. Without being exhaustive, such factors include uncertainties involved in the development of Genkyotex's products, which may not succeed, or in the delivery of Genkyotex's products marketing authorizations by the relevant regulatory authorities and, in general, any factor that could affect Genkyotex's capacity to commercialize the products it develops. No guarantee is given on forward-looking statements which are subject to a number of risks, notably those described in the registration document filed with the French Markets Authority (the AMF) on 1 April 2015 under number R.15-015, as updated in the Document E filed with the AMF on 31 January 2017 under number E.17-004 and in the Annual Financial Report of the company on 27 February 2017, and those linked to changes in economic conditions, the financial markets, or the markets on which Genkyotex is present. Genkyotex products are currently used for clinical trials only and are not otherwise available for distribution or sale.

INVESTORS	MEDIA	US
NewCap Dušan Orešanský, Tristan Roquet Montégon and Emmanuel Huynh +33 1 44 71 94 92 genkyotex@newcap.eu	ALIZE RP Caroline Carmagnol and Margaux Pronost +33 6 64 18 99 59 +33 1 44 54 36 65 genkyotex@alizerp.com	LifeSci Advisors, LLC Brian Ritchie +1-212-915-2578 britchie@lifesciadvisors.com