Kira Pharmaceuticals Announces IND Approval from Chinese NMPA for Phase 2 Evaluation of KP104 in Paroxysmal Nocturnal Hemoglobinuria (PNH)

CAMBRIDGE, MA, USA and SUZHOU, JIANGSU, CHINA (October 17, 2022) – Kira Pharmaceuticals, a global biotechnology company pioneering transformational complement therapies to treat immune-mediated diseases, announced today that the Chinese National Medical Products Administration (NMPA) has cleared the Investigational New Drug (IND) application for evaluation of KP104, a first-in-class bifunctional biologic that selectively targets the alternative and terminal complement pathways, in paroxysmal nocturnal hemoglobinuria (PNH). This approval enables Phase 2 study of the efficacy, safety, tolerability, pharmacokinetics (PK), and pharmacodynamics (PD) of KP104 in participants with PNH in China.

“This clearance represents an important milestone for Kira in our advancement of lead asset, KP104, as a next-generation treatment for complement-mediated diseases like PNH. KP104 has been engineered to simultaneously block two key complement targets, an approach we believe will make it a groundbreaking therapeutic option for patients awaiting more effective treatments to address their conditions,” said Angela Yan, President for China & Asia Development and Operations of Kira Pharmaceuticals.

PNH is a rare, life-threatening blood disease in which aberrant red blood cells are produced and recognized as foreign entities within the body, resulting in hyperactivation of the complement system and destruction of red blood cells. Patients with PNH may also experience anemia, blood clots, and impairments to bone marrow function. Current complement targeted treatments for PNH operate via inhibition of single complement proteins. While these drugs have resulted in reductions in hemolysis, recent studies suggest that treatments that inhibit both the alternative and terminal complement pathways may offer further improvements in patient outcomes.

KP104 is a biologic that simultaneously blocks the alternative and terminal complement pathways by virtue of its bifunctional design. Phase 1 data from the SYNERGY-1 first-in-human (FIH) study of KP104 has demonstrated clinical proof-of-mechanism (POM) for the biologic, which received Orphan Drug Designation for treatment PNH from the

US Food and Drug Administration (FDA) earlier this year. Kira will present full data from the completed Phase 1 trial at 2022 American Society of Nephrology annual meeting later this year.

About Paroxysmal Nocturnal Hemoglobinuria

Paroxysmal Nocturnal Hemoglobinuria (PNH) is a rare, life-threatening blood disease that is characterized by the destruction of red blood cells, formation of blood clots, and impairment of bone marrow function. PNH affects between 1 and 5 people per million and is almost always caused by a genetic mutation that results in production of aberrant hematopoietic stem cells. These stem cells produce irregular red blood cells that are highly susceptible to destruction via complement activation. Due to the complexity of complement biology and multiple pathways driving PNH pathology, there remains a significant unmet medical need for next-generation drugs with better efficacy and convenience of administration than offered by current therapies.

About KP104

KP104 is a first-in-class bifunctional biologic designed to simultaneously and selectively block both the alternative and terminal complement pathways, providing a powerful and synergistic method of targeting validated drivers of complement-mediated disease. This dual-target mechanism of action uniquely positions KP104 to address complement-mediated diseases and potentially provide greater benefits than single-target complement agents. Engineered to have an extended half-life and potency, KP104 has a formulation suitable for both intravenous and subcutaneous administrations. KP104 is entering Phase 2 POC trials across multiple renal disease and hematologic indications and has been granted Orphan Drug Designation by the FDA for the treatment of paroxysmal nocturnal hemoglobinuria (PNH). Phase 2 trials will be conducted globally, including in the U.S., China, Australia, and South Korea. KP104 is an investigational agent not yet approved for any indication by any health authority.

About Kira Pharmaceuticals

Kira Pharmaceuticals is a clinical-stage biotechnology company pioneering complement-targeted therapies to treat immune-mediated diseases. Enabled by its

LOGIC platform, the company has developed a robust pipeline of novel assets against validated complement targets. Headquartered in Cambridge, Massachusetts and with facilities in China and Australia, Kira Pharmaceuticals has established a global team committed to advancing life-changing therapies to patients around the world. More information on Kira can be found at www.kirapharma.com and on LinkedIn.

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科越医药宣布 KP104 用于治疗阵发性睡眠性血红蛋白尿症（PNH）的 II 期临床试验申请 已获得中国国家药品监督管理局批准

美国马萨诸塞州剑桥市和中国江苏苏州市（2022 年 10 月 17 日）——科越医药，一家致力于研发新一代补体药物治疗免疫介导疾病的全球生物技术公司，今日宣布，中国国家药品监督管理局（NMPA）已批准其 KP104 用于治疗阵发性睡眠性血红蛋白尿症（PNH）II 期临床试验的新药研究申请 (CTA)。KP104 是一种全球首创的双靶点补体生物制剂，它能特异性地同时抑制补体旁路和末端途径，且对两个靶点的抑制具有协同作用。该 II 期临床 试验的目的是在中国 PNH 患者中评估 KP104 的有效性、安全性、耐受性、药代动力学 （PK）和药效学（PD）。

科越医药中国和亚洲研发与运营总裁闫慧女士说 : “此 CTA 在中国首次获批，是科越加速推动 KP104 这新一代补体药物用于治疗像 PNH 这样的补体介导疾病道路上的一个重要里程碑。KP104 被设计用于同时阻断两个关键的补体靶点，对于那些需要更有效治疗手段的 PNH 患者来说，我们相信 KP104 将是一个突破性的治疗选择。”

阵发性睡眠性血红蛋白尿症是一种罕见的危及生命的血液系统疾病，它的特征是体内产生异常红细胞，并被机体认定为“异物”，从而导致补体系统过度激活和红细胞破坏，PNH 患 者可能出现贫血、血栓和骨髓功能受损。目前针对 PNH 的补体靶向治疗是通过抑制单个 补体靶点来实现的。虽然这些药物可减轻溶血，但最近的研究表明，同时抑制补体旁路途 径和末端途径的治疗可能会更有效地改善患者的病情。

KP104 是一种双靶点补体药物，它能同时阻断补体旁路途径和末端途径。来自 KP104 临床 I 期首次人体试验（FIH）SYNERGY-1 的数据证明了该生物制剂的双靶点作用机制，并 在今年早些时候获得了美国食品药品监督管理局（FDA）批准成为治疗 PNH 的孤儿药资 格认定。科越医药将在今年晚些时候的 2022 年美国肾脏学会年会上公布已经完成的临床 I 期试验的完整数据。

关于阵发性睡眠性血红蛋白尿症

阵发性睡眠性血红蛋白尿症是一种罕见的危及生命的血液系统疾病，由属于先天免疫系统的补体系统的过度活动引起，其特征是红细胞破坏、血栓形成和骨髓功能受损。每百万人

中有 1 至 5 人患有 PNH，几乎都是由基因突变引起，导致产生异常造血干细胞。这些干细胞产生的异常红细胞，极易通过补体激活而被破坏。由于补体生物学的复杂性以及 PNH 疾病的发生与补体通路多靶点相关，目前仍然存在巨大未被满足的临床需求，急需比当前疗法更好的疗效和给药便利性的下一代药物。

关于 KP104

KP104 是一种具有独特作用机制的全球首创双靶点补体药物。它可特异性地同时作用于补 体旁路途径和末端途径，从而有效地、协同性地抑制补体，以更加有选择性的精准治疗补 体介导的疾病。KP104 还被设计成具有延长的半衰期和效能，其配方可用于静脉注射和皮 下给药。KP104 正进入多个适应症的 II 期临床试验，包括 IgA 肾病（IgAN）、C3 肾小球病 （C3G）、继发于系统性红斑狼疮的血栓性微血管病（SLE-TMA）和阵发性睡眠性血红蛋 白尿症 。II 期临床试验将在全球范围内进行，包括美国、中国、澳大利亚和韩国。KP104 是一种尚未获得任何监管当局批准用于任何适应症治疗的研究药物。

关于科越医药

科越医药是一家处于临床研发阶段的全球化生物技术公司，致力于研发补体靶向疗法治疗免疫介导疾病。公司凭借自己的 LOGIC 药物发现平台，致力于推进首创疗法 （FIC）及同 类最佳疗法 （BIC），以改变患者的生活。科越医药总部位于马萨诸塞州剑桥，并通过在 中国苏州和上海以及澳大利亚建设研发中心和办公室，致力于建立全球足迹并为世界各地 的患者提供先进的治疗药物。如需了解有关科越医药的更多信息，请访问公司官网 www.kirapharma.com 和关注 LinkedIn。

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