KIRA PHARMACEUTICALS RECEIVES FDA ORPHAN DRUG DESIGNATION FOR KP104, A BIFUNCTIONAL ANTIBODY FUSION PROTEIN, FOR THE TREATMENT OF PAROXYSMAL NOCTURNAL HEMOGLOBINURIA

CAMBRIDGE, MA, USA and SUZHOU, JIANGSU, CHINA (July 28, 2022) – Kira Pharmaceuticals, a global clinical-stage biotechnology company pioneering transformational complement therapies to treat immune-mediated diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to KP104 for the treatment of Paroxysmal Nocturnal Hemoglobinuria (PNH).

PNH is a rare, life-threatening blood disease that arises due to hyperactivity of the complement system. An intricate constellation of protein pathways, the complement system is a key component of innate immunity. Aberrant activity within this system can be a driver of diseases such as autoimmune and inflammatory conditions. KP104 is a bifunctional, first-in-class biologic with a unique dual-approach mechanism of action. Designed to selectively block the alternative and terminal pathways, KP104 provides a powerful and synergistic method of targeting validated drivers of disease in the complement system. KP104 has also been engineered to have an extended half-life and potency, with a formulation that can be used for both IV and subcutaneous administration.

“Receiving Orphan Drug Designation is a key milestone for Kira, validating KP104’s differentiated potential to provide a safe and effective treatment for PNH patients,” said Frederick Beddingfield, MD, PhD, CEO at Kira Pharmaceuticals. “As a bifunctional complement therapy targeting both the alternative and terminal pathways, KP104 has the potential to treat complement-mediated diseases where single-target therapies are not adequate. As we enter Phase 2 across multiple indications, we look forward to continued clinical evaluation of KP104 for patients in need.”

The FDA’s Orphan Drug Designation program provides orphan status to drugs defined as those intended for the treatment, diagnosis, or prevention of rare diseases that affect fewer than 200,000 people in the United States.

KP104 has completed a Phase 1 first-in-human (FIH) study and is entering Phase 2 trials across multiple indications. The Phase 1 clinical trial was a randomized, double blind, placebo-controlled study designed to evaluate the safety, tolerability, pharmacokinetics (PK), and pharmacodynamics (PD) of escalating single and multiple doses of KP104 in

healthy volunteers. Kira plans to present this Phase 1 data at a medical conference later this year.

About KP104

KP104 is a bifunctional, first-in-class biologic with a unique dual-approach mechanism of action. Designed to selectively block the alternative and terminal pathways, KP104 provides a powerful and synergistic method of targeting validated drivers of disease in the complement system. KP104 has also been engineered to have an extended half-life and potency and has a formulation suitable for both IV and subcutaneous administrations. KP104 is entering Phase 2 trials across multiple indications, including IgA nephropathy (IgAN), C3 Glomerulopathy (C3G), Thrombotic microangiopathies secondary to systemic lupus erythematosus (SLE-TMA) and Paroxysmal Nocturnal Hemoglobinuria (PNH). Phase 2 trials will be conducted globally including in the U.S., China, Australia, and South Korea.

About Paroxysmal Nocturnal Hemoglobinuria

Paroxysmal Nocturnal Hemoglobinuria (PNH) is a rare, life-threatening blood disease that arises due to hyperactivity of the complement system, part of the innate immune system. 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. 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 Kira Pharmaceuticals

Kira Pharmaceuticals is a global clinical-stage biotechnology company pioneering transformational complement therapies to treat immune-mediated diseases. Enabled by its LOGIC platform, the company has developed a pipeline of nine (9) novel assets spanning targets across the complement cascade. Kira is committed to advancing life-changing therapies that will transform the lives of patients globally with complement-driven diseases. Kira Pharmaceuticals is headquartered in Cambridge, Massachusetts with additional offices in Suzhou and Shanghai, China. More information on Kira can be found at www.kirapharma.com and on LinkedIn.

科越医药双功能抗体融合蛋白获 FDA 孤儿药认定

美国马萨诸塞州剑桥和中国苏州， 2022 年 7 月 28 日 /PRNewswire/-- 科越医药（Kira Pharmaceuticals），一家致力于研发新一代补体靶向药物来治疗补体介导疾病的全球临床阶段生 物技术公司，今天宣布，美国食品和药品管理局（FDA）已批准 KP104 孤儿药认定，用于治疗阵发性 睡眠性血红蛋白尿症（PNH）。

PNH 是一种罕见的、危及生命的血液性疾病，由补体系统的过度活动引起。补体系统是一组复杂的蛋 白质通路群，是先天性免疫的关键组成部分。这一系统内的异常活动可能是导致自身免疫性和炎症性 疾病的诱因。KP104 是一款具有独特作用机制的全球首创的双功能补体类生物药，能够选择性同时 抑制旁路和终端补体途径，通过调节对疾病发展至关重要的补体活化级联中的两个单独的限速步 骤，为抑制补体提供一种强有力且可能更加有选择性的精准治疗方法。KP104 还设计有延长的半 衰期和效力，以及皮下注射的给药方式，从而让患者有可能在家中实现自我给药。

科越医药首席执行官、医学博士 Frederick Beddingfield 表示：“获得孤儿药认定是科越的一个关 键里程碑，证实了 KP104 差异化潜力，能够为 PNH 患者提供安全有效的治疗。作为一种靶向旁路途径 和终端途径的双功能补体疗法，KP104 具有潜力可治疗单靶点疗法不足以治疗的补体介导的疾病。随 着我们多个适应症的 2 期临床试验的推进，我们期待对 KP104 对一系列补体介导疾病的有未被满足临 床需求的患者进行临床评估。”

FDA 的孤儿药认定项目为用于治疗、诊断或预防罕见疾病（在美国的影响人数低于 20 万人）的药物提 供孤儿药身份。

KP104 已完成 1 期临床首次人体（FIH）研究，并即将开始多个适应症的 2 期临床试验。1 期临床试验 是一项随机、双盲、安慰剂对照研究，旨在评估 KP104 在健康志愿者中单剂量递增和多剂量给药的安 全性、耐受性、药代动力学(PK)和药效学(PD)。科越计划在今年晚些时候的国际学术会议上分享这些 1 期临床数据。

关于 KP104

KP104 是一种具有独特作用机制的全球首创双功能补体生物药。KP104 旨在同时选择性抑制补体旁 路和终端途径，提供一种强大的协同机制且可能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力，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 2 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（SLE TMA）的血栓性微血管病和 PNH。2 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。

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（PNH）是一种罕见的、危及生命的血液疾病，由属于先天免疫系统的补 体系统的过度活动引起。PNH 以红细胞破坏、血栓形成和骨髓功能受损为特征，每百万人中有 1 到 5 人受此病影响。由于补体生物学的复杂性和 PNH 致病病理学的多种途径，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。

关于科越医药

科越医药是一家临床研发阶段的全球生物技术公司，是补体靶向疗法治疗免疫介导疾病的先驱。公司 凭借自己的 LOGIC 药物发现平台，以克服补体药物开发过程中的各种挑战，致力于推进首创疗法 （FIC）及同类最佳疗法 （BIC），公司已经开发出针对补体靶点的九种创新管线。 科越医药致力于开发针对补体介导疾病的突破性免疫疗法，以此彻底改善补体驱动型疾病患者的 生活。科越医药总部位于马萨诸塞州剑桥，并且在中国苏州和上海设有研发中心。如需了解有关科 越的更多信息，请访问 www.kirapharma.com 和关注 LinkedIn。