Kira Pharmaceuticals Announces First Cohort of Patients Dosed in Phase 2 Study of KP104 in Paroxysmal Nocturnal Hemoglobinuria (PNH)

Nov 30, 2022

CAMBRIDGE, MA (November 30, 2022) – Kira Pharmaceuticals, a global biotechnology company pioneering transformational complement therapies to treat immune-mediated diseases, today announced that the first cohort has been dosed in a Phase 2 trial evaluating KP104 for the treatment of paroxysmal nocturnal hemoglobinuria (PNH) in patients in China. KP104 is a first-in-class bifunctional biologic designed to modulate complement activity through selective targeting of key intervention points in the alternative and terminal complement pathways.

“Progression of KP104 into Phase 2 represents an important milestone for Kira in our pursuit of more effective treatments for complement-mediated diseases,” said Frederick Beddingfield, MD, PhD, CEO of Kira Pharmaceuticals. “This advancement is supported by key Phase 1 biomarker and safety data that confirm KP104’s dual mechanism of action, demonstrate dose-dependent inhibition of complement activity, support development of KP104 for subcutaneous (SC) and intravenous (IV) administration, and indicate a favorable safety profile. We’re confident in the clinical translation of these results to patients and look forward to continued advancement of KP104 as a differentiated and effective treatment across a range of complement-mediated diseases with few treatment options.”

The Phase 2 study aims to evaluate the safety, tolerability, immunogenicity, pharmacokinetics, pharmacodynamics, and efficacy of KP104 in patients with PNH that have not previously been treated with complement inhibitor therapies. The study is designed in two parts; the first is to assess escalating doses and varied dose intervals of KP104, while the second is to assess clinical proof of concept for treatment of PNH with KP104. More information about the Phase 2 trial is available on clinicaltrials.gov (NCT05476887).

Kira is also initiating two additional Phase 2 trials evaluating KP104 in a renal basket study including IgA nephropathy (IgAN) and complement 3 glomerulopathy (C3G) and

in thrombotic microangiopathies secondary to systemic lupus erythematosus (SLE-TMA).

About Paroxysmal Nocturnal Hemoglobinuria (PNH)

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. Current therapies include C5 inhibitors, which do not address extravascular hemolysis (EVH) related to the alternative pathway or a C3 inhibitor, which may address EVH but may not adequately block C5 downstream, leading to life-threatening breakthrough hemolysis (Breakthrough Hemolysis in PNH with Proximal or Terminal Complement Inhibition, N Engl J Med, July 14, 2022).

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, safety, 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 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, and Australia. 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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Media Contact

Sarah O'Connell
Verge Scientific Communications

soconnell@vergescientific.com

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 首个队列的患者已完成第一次给药

美国马萨诸塞州剑桥市和中国江苏苏州市（2022 年 11 月 30 日）——科越医药，一家致力于研发创新型补体药物治疗免疫介导疾病的全球化生物技术公司，今日宣布，公司在中国开展的 KP104 用于治疗阵发性睡眠性血红蛋白尿症（PNH）的 II 期临床研究，首个队列的患者已完成第一次给药。KP104 是一种全球首创的双功能生物制剂，旨在通过选择性 靶向作用于旁路和终末补体途径中的关键靶点来调节补体活性。

科越医药首席执行官 Frederick Beddingfield 博士表示：“KP104 进入 II 期临床是科越寻求 补体介导疾病更有效治疗手段过程中的重要里程碑。这一进展得到了关键的 I 期生物标志 物和安全性数据的支持，这些数据证实了 KP104 的双重作用机制，证明了呈剂量相关的 补体活性抑制，支持 KP104 通过皮下（SC）和静脉（IV）给药的开发，同时又展现了良好 的安全性。我们有信心将这些结果向患者进行临床转化，期待 KP104 作为一种区别于其 它治疗手段继续开发，并针对一系列缺乏治疗手段的补体介导性疾病提供有效的治疗方法。

II 期临床研究旨在评估 KP104 在既往未接受补体抑制剂治疗的 PNH 患者中的安全性、耐 受性、免疫原性、药代动力学、药效学和疗效。本研究分为两部分：第一个是评估 KP104 的递增剂量和不同给药间期，而第二个是评估使用 KP104 治疗 PNH 的临床概念验证。有 关 II 期研究的更多信息可在药物临床试验登记与信息公示平台（clinicaltrials.gov; NCT05476887）获取。

科越还启动了另外两项 II 期临床研究来评估 KP104，其一是针对补体介导的肾病，包括 IgA 肾病（IgAN）和 C3 肾小球病（C3G），另一项则是针对继发于系统性红斑狼疮的血栓 性微血管病（SLE-TMA）。

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，其特征是红细胞 破坏、血栓形成和骨髓功能受损。PNH 的患病人数在百万分之 1-5，几乎都是由于基

因突 变导致产生异常造血干细胞引起。这些干细胞产生不规则的红细胞，这些红细胞很容易通 过补体激活而被破坏。目前的治疗包括 C5 抑制剂，但无法解决与替代途径相关的血管外 溶血（EVH）。或是 C3 抑制剂，可能解决 EVH，但有存在不能充分阻断下游 C5 的问题， 导致危及生命的突破性溶血（Breakthrough Hemolysis in PNH with Proximal or Terminal Complement Inhibition, N Engl J Med, July 14, 2022）。 由于补体生物学的复杂性，结合多个靶点影响 PNH 病理学等种种因素，在疗效、安全性 和给药便利性各方面都优于现有治疗手段的下一代药物的医疗需求仍然没有得到满足。

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的血栓性微血管病（SLE-TMA）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。KP104 是一种尚未获得任何卫 生当局批准用于任何适应症的研究药物。

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，也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，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 的患者推进改变生活的疗法科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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媒体联系人

Sarah O’Connell Verge Scientific Communications

soconnell@vergescientific.com