Kira Pharmaceuticals Receives FDA Clearance of IND Application for Phase 2 Evaluation of KP104 in Systemic Lupus Erythematosus Associated Thrombotic Microangiopathy (SLE-TMA)

Oct 03, 2022

Phase 1 supporting data indicated an encouraging profile and confirmed the dual mechanism of action achieved through targeting both the alternative and terminal complement pathways

CAMBRIDGE, MA (October 3, 2022) – Kira Pharmaceuticals, a global biotechnology company pioneering transformational complement therapies to treat immune-mediated diseases, announced today that the US Food and Drug Administration (FDA) has cleared the Investigational New Drug (IND) application for KP104, a first-in-class bifunctional biologic that selectively and synergistically targets the alternative and terminal complement pathways. The Phase 2 trial will evaluate the efficacy, safety, tolerability, pharmacokinetics (PK), and pharmacodynamics (PD) of KP104 in participants with systemic lupus erythematosus associated thrombotic microangiopathy (SLE-TMA) in the US, China, and Australia. The IND was supported by Phase 1 data (SYNERGY-1 Study), which demonstrated proof-of-mechanism for KP104 on both the terminal and alternative complement pathways and showed an encouraging profile for the Phase 2 study in SLE-TMA.

“We believe KP104, which uniquely modulates two targets, one in the proximal or alternative pathway and one in terminal pathway, offers immense potential to address complement-mediated diseases for which there are no currently approved treatments, such as SLE-TMA,” said Frederick Beddingfield, MD, PhD, CEO of Kira Pharmaceuticals. “This IND clearance is further testament to the dedication of the Kira team to advance KP104 and bring this next-generation therapeutic one step closer to patients awaiting improved treatment options.”

The complement system is a key component of innate immunity that operates via several activation pathways comprising more than 30 proteins. Dysregulation within this system can be a critical driver of numerous immunologic conditions including SLE-TMA. The complexity of the complement system has made development of selective and

effective treatments a challenge, amplifying the demand for next-generation complement-based therapeutics.

TMA is a serious complication that can occur in patients with SLE for which there are no currently approved treatments. Kira is pursuing several assets to address the unmet need for highly effective complement-targeted therapeutics, including treatments for SLE-TMA, with lead candidate KP104 showing significant promise in early clinical studies.

Kira has completed a Phase 1 first-in-human (FIH) study of KP104 demonstrating clinical proof-of-mechanism (POM) for the first-in-class bifunctional biologic. Furthermore, data in relevant animal models indicate a pharmacological profile optimal for continued development of KP104 for both intravenous and subcutaneous administration. The company will present safety, tolerability, PK, and PD data from the Phase 1 study at a medical conference later this year.

About SLE-TMA

Thrombotic microangiopathies (TMA) are associated with a number of diseases and are characterized by destruction of red blood cells, low platelet counts, and organ damage. TMA can occur as a severe symptom of systemic lupus erythematosus (SLE), leading to poorer patient outcomes as compared to patients living with SLE or lupus nephritis (LN) alone. Complications resulting from TMA in SLE are a cause of significant morbidity and mortality. There are currently no approved therapies for SLE-TMA and the current standard of care treatments provide poor long-term benefits.

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.

# # #

Media Contact

Sarah O'Connell
Verge Scientific Communications
soconnell@vergescientific.com

科越医药 KP104 治疗系统性红斑狼疮相关血栓性微血管病（SLE-TMA）的 II 期临床试验申请获得美国 FDA 批准

令人振奋的临床 I 期试验数据证实了 KP104 可同时选择性抑制补体旁路和末端双重途径的协同作用机制

美国马萨诸塞州剑桥和中国江苏苏州（2022 年 10 月 3 日）科越医药，一家致力于研发新一代补体靶向药 物治疗补体介导疾病的全球生物技术公司，今日宣布其 KP104 的 II 期临床试验申请已获得美国食品和药物 管理局（FDA）批准。KP104 是一种全球首创（First-in-Class）的补体双靶点生物制剂，它能特异性地抑制 补体旁路和末端途径，且对两个靶点的抑制具有协同作用。该项临床 II 期试验将在美国、中国、澳大利亚 开展，以评估 KP104 在系统性红斑狼疮 相关血栓性微血管病（SLE-TMA） 患者中的有效性、安全性、耐受性、药代动力学（PK）和药效学（PD）。支持此次临床申请递交的临床 I 期试验数据（SYNERGY-1 研究）证明了 KP104 能同时抑制补体旁路和末端途径的协同作用机制，并显示了 令人鼓舞的结果，为系统性红斑狼疮相关血栓性微血管病的临床 II 期试验奠定了基础。

科越医药首席执行官 Frederick Beddingfield 博士说：“KP104 是目前唯一能作用于补体双靶点的药物，一个 靶点在旁路途径的近端，另一个靶点在末端途径，我们认为 KP104 在治疗像系统性红斑狼疮相关血栓性微 血管病这样目前尚无批准药物的补体介导的疾病方面具有巨大潜力。科越团队一直致力于尽快推进 KP104 的研发，以使 KP104 这新一代的补体药物能更早惠及患者。FDA 对该临床 II 期试验申请的获批，是对我们 KP104 工作的肯定。”

补体系统是一个复杂的蛋白质通路，也是机体先天免疫系统的一个重要组成部分，它通过由 30 多种蛋白质 组成的多种激活途径发挥作用。补体系统的异常激活和失调可能是包括系统性红斑狼疮相关血栓性微血管 病在内的多种免疫疾病的重要驱动因素。补体系统的复杂性给补体药物的研发带来了很大挑战，也凸显了 对新一代补体药物的巨大研发需求。

血栓性微血管病（TMA）是系统性红斑狼疮（SLE）患者的一种严重并发症，目前尚无批准的治疗药物。科 越医药正致力于开发多款产品，以填补高效的补体靶向药物来解决

尚未满足的临床需求，包括对系统性红 斑狼疮相关血栓性微血管病 的治疗， 科越医药的主导产品 KP104 在早期临床研究中显示出巨大的前景。

科越医药已经完成了 KP104 的临床 I 期首次人体（FIH）研究，该研究证实了首创药物 KP104 双靶点同时选 择性抑制补体旁路和末端途径的协同作用机制。此外，相关动物模型中的数据表明， KP104 的药理学特性 支持其在下一步研发中采用静脉注射和皮下注射两种给药方式。公司将在今年晚些时候的一次医学会议上 展示临床 I 期研究的安全性、耐受性、PK 和 PD 数据。

关于系统性红斑狼疮相关血栓性微血管病

血栓性微血管病与许多疾病相关，其特征是红细胞破坏、血小板计数降低和器官损伤。血栓性微血管病可 作为系统性红斑狼疮（SLE）的严重症状出现，与仅患系统性红斑狼疮或狼疮性肾炎（LN）的患者相比，继 发于系统性红斑狼疮的血栓性微血管病患者预后更差。血栓性微血管病引起的并发症是导致系统性红斑狼 疮患者病情严重和死亡率升高的原因之一。目前尚无批准的继发于系统性红斑狼疮的血栓性微血管病的治 疗方法，现有的标准治疗的长期效果欠佳。

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 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（SLETMA）和阵发性睡眠性血红蛋白尿症 （PNH）。临床 II 期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。

媒体联系人 Sarah O’Connell Verge Scientific Communications
soconnell@vergescientific.com