Innorna Announces the U.S. FDA Orphan Drug Designation Granted to IN022 for the Treatment of Homocystinuria

UNITED STATES, July 23, 2024 /EINPresswire.com/ -- Innorna, a clinical-stage biotech company pioneering its proprietary lipid nanoparticle (LNP) technology to develop novel RNA therapeutics, today announced that the U.S. Food and Drug Administration (FDA) granted Orphan Drug Designation (ODD) to its investigational therapy, IN022, for the treatment of classic homocystinuria (HCU). IN022 was also granted by the US FDA a Rare Pediatric Disease Designation (RPDD) on July 5, 2024. Obtaining RPDD and ODD would significantly facilitate the IN022 clinical development and approval process and quickly bring this potential therapy to HCU patients.

> The RPDD and ODD designations granted by US FDA will significantly facilitate the clinical development and approval of IN022. We are excited to bring this potential therapy to HCU patients quickly."
>
> *Linxian Li, Ph.D., CEO and Founder of Innorna*

About HCU and IN022

HCU is a rare autosomal recessive inherited sulfur amino acid metabolism disorder primarily caused by a cystathionine beta-synthase deficiency (CBS). HCU patients have a broad spectrum of age (older than three years) on presentation, with multisystemic clinical complications, particularly skeletal and connective tissue defects, osteoporosis, dislocated optic lenses, learning difficulties, and developmental delay or thromboembolism. Currently, there is no approved therapeutic drug for this disease.

IN022, a mRNA therapy utilizing Innorna's proprietary mRNA-LNP technology, is designed to address the root cause of HCU resulting from a deficiency in CBS. It is expected to restore the function of the CBS enzyme, thereby normalizing homocysteine metabolism and potentially ameliorating symptoms in HCU patients.

About Innorna

Founded in 2019, Innorna focuses on developing best-in-class LNP delivery technology and

advancing innovative RNA therapies to address unmet medical needs globally. Innorna has built a diversity-oriented lipid library (DOLL) of over 5,000 ionizable lipids, which can be applied in various modalities or scenarios, including mRNA vaccines and therapeutics, cell therapies (CAR-T, CAR-NK, etc.), and genome editing therapies. Innorna's comprehensive R&D capability fully supports the end-to-end process of innovative treatments for internal development and external collaboration partners, from discovery to clinical development. Innorna has developed an extensive global patent portfolio and filed over 40 patent applications regarding the innovation of LNP and mRNA technology.

Based on its proprietary technology platform, Innorna has built extensive internal R&D pipelines for infectious and rare diseases. In addition, the company has established partnerships with pharma and biotech companies to explore the technology's potential in broader therapeutic areas. Since its establishment five years ago, Innorna has been widely recognized by the investment community and industry. It has won many awards, including MIT Technology Review's Global 50 Smartest Companies (2020 and 2022) and Fortune China's Most Socially Influential Startups.

At Innorna, we value INNOVATION, INTEGRITY, EFFICIENCY, and OPENNESS. We are committed to exploring the frontier of mRNA application based on platform technologies and leading the revolutionary step toward expanding the clinical application of mRNA in various therapeutic approaches to fulfill the unmet medical needs of patients worldwide.

Please visit the Innorna website at www.innorna.com for more information.

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