Innorna Announces U.S. FDA Rare Pediatric Disease Designation Granted to IN022 for the Treatment of Homocystinuria

UNITED STATES, July 9, 2024 /EINPresswire.com/ -- Innorna, a clinical-stage biotech company pioneering its proprietary lipid nanoparticle (LNP) technology to develop novel RNA therapeutics, announced a significant milestone today. The U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease Designation (RPDD) to its investigational mRNA therapy IN022 for the treatment of classic homocystinuria (HCU), a serious or life-threatening genetic disease. RPDD is granted by the FDA to drugs intended to treat serious or life-threatening diseases that primarily affect individuals aged from birth to 18 years and fewer than 200,000 people in the United States. This RPDD will greatly facilitate IN022 clinical development and bring this potential therapy to patients as quickly as possible. The RPDD also means that the FDA may award a Priority Review Voucher upon the approval of the IN022 product.

> The Rare Pediatric Disease Designation represents a significant step forward in the development of IN022 as a potential treatment of homocystinuria. We look forward to its advancement in the clinic."
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> *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, the first to use an 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 therapies 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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