

Innorna Announces U.S. FDA RPDD Granted to IN016 for the Treatment of Progressive Familial Intrahepatic Cholestasis

UNITED STATES, July 13, 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 (the

"FDA") has granted Rare Pediatric Disease Designation ("RPDD") to [IN016](#), one of the company's lead mRNA product candidates for rare diseases. IN016 is to treat Progressive Familial Intrahepatic Cholestasis (PFIC), a group of rare genetic disorders that cause progressive liver disease and can lead to cirrhosis and end-stage liver disease in infants and children. Following



“

The RPDD designation represents a significant step in developing IN022 and IN016 as potential treatments for HCU and PFIC, respectively. We are excited to continue its advancement toward the clinic.”

*Linxian Li, Ph.D., CEO and
Founder of Innorna*

IN022, a potential mRNA therapy for treating Homocystinuria (HCU), IN016 marks the second Innorna product candidate to receive the RPDD from the FDA. The RPDD will greatly facilitate IN016 and IN022 clinical development, quickly bringing these novel mRNA therapies to PFIC and classic HCU patients.

Innorna's advancements in rare diseases are driven by its technological breakthroughs in the LNP delivery field. Unlike mRNA infectious disease vaccines, the delivery technology used for mRNA rare disease therapies has many challenges, including tissue targeting, delivery efficiency, and safety. To overcome these, Innorna has, through extensive research, discovered and developed the

optimal LNP delivery technology for its mRNA-based rare disease therapies. This breakthrough has rapidly advanced Innorna's rare disease pipeline, addressing many patients' high unmet medical needs. In addition, significant progress has also been made in extra-hepatic-targeted delivery technology, which lays the groundwork for future extra-hepatic disease pipeline development.

Progressive familial intrahepatic cholestasis (PFIC) is a heterogeneous group of rare genetic disorders associated with defects in bile acid secretion or transport, resulting in unwanted bile accumulation within the liver. Typical symptoms include jaundice, hepatomegaly, pruritus, splenomegaly, and diarrhea. Over time, this disease can evolve into severe liver damage, fibrosis, and cirrhosis and is associated with high mortality rates. While symptoms can be managed to some extent, more effective treatment is in great need for PFIC. IN016 is designed to address the root cause of PFIC resulting from gene mutations, potentially restoring defective proteins, normalizing bile excretion, and possibly improving symptoms in PFIC 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.

bd@innorna.com

Innorna

[email us here](#)

This press release can be viewed online at: <https://www.einpresswire.com/article/727290136>

EIN Presswire's priority is source transparency. We do not allow opaque clients, and our editors

try to be careful about weeding out false and misleading content. As a user, if you see something we have missed, please do bring it to our attention. Your help is welcome. EIN Presswire, Everyone's Internet News Presswire™, tries to define some of the boundaries that are reasonable in today's world. Please see our Editorial Guidelines for more information.

© 1995-2024 Newsmatics Inc. All Right Reserved.