Ionis partner licenses rare kidney disease treatment and will advance into Phase 3 clinical study

July 11, 2022

- Positive data from a Phase 2 study of IONIS-FB-L$_{Rx}$ support further development for treatment of patients with IgA nephropathy

CARLSBAD, Calif., July 11, 2022 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS), today announced that its long-standing partner, Roche, will license and advance IONIS-FB-L$_{Rx}$, an investigational antisense medicine, into a Phase 3 clinical study in patients with immunoglobulin A nephropathy (IgAN). IgAN is a rare and serious condition that often leads to chronic kidney disease and renal failure. Roche's decision to advance the program comes after positive data from a Phase 2 clinical study in which IONIS-FB-L$_{Rx}$ met its primary endpoint of change in 24-hour urinary protein at 29 weeks compared to baseline.

In the Phase 2 study (NCT04014335), IONIS-FB-L$_{Rx}$ demonstrated a favorable safety and tolerability profile. The study data are consistent with the clinical profile seen across Ionis' other LICA programs, further validating how advancements in the company's **LI**gand-**C**onjugated **A**ntisense technology platform position Ionis to deliver potentially transformative treatments for a range of unmet medical needs. Data from the Phase 2 study of IONIS-FB-L$_{Rx}$ in patients with IgAN has been submitted for presentation at an upcoming medical meeting.

IgAN occurs when too much IgA protein accumulates in the kidneys, causing inflammation and tissue damage, which is the root cause of the disease. IONIS-FB-L$_{Rx}$ was designed by Ionis to reduce the production of complement factor B (FB), which is associated with the development of several complement-mediated diseases, including IgAN.

"Roche's decision to advance the program reaffirms our shared confidence in the ability of Ionis' antisense medicines to effectively target the root cause of difficult to treat diseases like immunoglobulin A nephropathy," said Michael McCaleb, Ph.D., vice president, clinical development at Ionis. "The results of the Phase 2 study provide initial clinical evidence that IONIS-FB-L$_{Rx}$ reduces complement and protein levels in the urine of patients with IgAN."

Roche will lead and be responsible for the Phase 3 study of IONIS-FB-L$_{Rx}$ in patients with IgAN and for future global development, regulatory and commercialization activities.

IONIS-FB-L$_{Rx}$ is also being evaluated in GOLDEN (NCT03815825), a Phase 2 clinical study to determine whether the medicine can slow or halt the progression of geographic atrophy due to age-related macular degeneration, or AMD. Ionis will receive $55 million from Roche for licensing IONIS-FB-L$_{Rx}$ for IgAN and achieving a development milestone in the GOLDEN study.

About IgA Nephropathy (IgAN)

Immunoglobulin A nephropathy (IgAN) is an important cause of chronic kidney disease and renal failure. Also known as Berger's disease, IgAN is characterized by deposition of IgA and Complement 3 (C3) activation products in the glomerular mesangium of the kidneys, resulting in inflammation and tissue damage. Although IgAN may occur at any age, it generally presents in the second or third decade of life. The clinical presentation, disease progression and histologic findings are highly variable among affected individuals. Current therapies are aimed at reduction of protein levels in the urine with administration of angiotensin inhibitors and control of blood pressure. Sometimes immunosuppressive therapies are given; however, this practice is not universally accepted.

About Ionis Pharmaceuticals, Inc.

For more than 30 years, Ionis has been the leader in RNA-targeted therapy, pioneering new markets and changing standards of care with its novel antisense technology. Ionis currently has three marketed medicines and a premier late-stage pipeline highlighted by industry-leading cardiovascular and neurological franchises. Our scientific innovation began and continues with the knowledge that sick people depend on us, which fuels our vision of becoming a leading, fully integrated biotechnology company.

To learn more about Ionis, visit www.ionispharma.com and follow us on Twitter @ionispharma.

Ionis' Forward-looking Statements

This press release includes forward-looking statements regarding Ionis' business and the therapeutic and commercial potential of Ionis' technologies, IONIS-FB-L$_{Rx}$ and other products in development. Any statement describing Ionis' goals, expectations, financial or other projections, intentions or beliefs is a forward-looking statement and should be considered an at-risk statement. Such statements are subject to certain risks and uncertainties, including those related to the impact COVID-19 could have on our business, and including but not limited to, those related to our commercial products and the medicines in our pipeline, and particularly those inherent in the process of discovering, developing and commercializing medicines that are safe and effective for use as human therapeutics, and in the endeavor of building a business around such medicines. Ionis' forward-looking statements also involve assumptions that, if they never materialize or prove correct, could cause its results to differ materially from those expressed or implied by such forward-looking statements.

Although Ionis' forward-looking statements reflect the good faith judgment of its management, these statements are based only on facts and factors currently known by Ionis. As a result, you are cautioned not to rely on these forward-looking statements. These and other risks concerning Ionis' programs are described in additional detail in Ionis' annual report on Form 10-K for the year ended Dec. 31, 2021, and the most recent Form 10-Q quarterly filing, which are on file with the Securities and Exchange Commission. Copies of these and other documents are available from the Company.

In this press release, unless the context requires otherwise, "Ionis," "Company," "we," "our," and "us" refers to Ionis Pharmaceuticals and its subsidiaries.

Ionis Pharmaceuticals® is a trademark of Ionis Pharmaceuticals, Inc.

View original content to download multimedia:https://www.prnewswire.com/news-releases/ionis-partner-licenses-rare-kidney-disease-treatment-and-will-advance-into-phase-3-clinical-study-301583315.html

SOURCE Ionis Pharmaceuticals, Inc.

Ionis Pharmaceuticals Investor Contact: 760-603-2331; Ionis Pharmaceuticals Media Contact: 760-603-4679