Ionis presents positive Phase 2 data in patients with IgA Nephropathy at American Society of Nephrology's Kidney Week 2022

November 7, 2022

– IONIS-FB-L_{Rx} achieved a 44% mean reduction in proteinuria in patients treated for 6 months

– Roche to advance IONIS-FB-L_{Rx} into Phase 3 development in H1 2023

CARLSBAD, Calif., Nov. 7, 2022 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) today presented positive results from a Phase 2 clinical study of IONIS-FB-L_{Rx} in patients with immunoglobulin A nephropathy (IgAN). Results from the study were presented in a poster session at the American Society of Nephrology's (ASN) Kidney Week 2022.

In the Phase 2 study, IONIS-FB-L_{Rx} met its primary endpoint of change in 24-hour urinary protein, demonstrating a 44% mean reduction in proteinuria from baseline to week 29. Kidney function, as measured by estimated glomerular filtration rate (eGFR), was maintained in all patients in the study. IONIS-FB-L_{Rx} achieved robust and sustained reductions in plasma complement Factor B (CFB), Alternative Pathway Activity (AH50), and urinary complement fragment Ba (Factor Ba). IONIS-FB-L_{Rx} also demonstrated a favorable safety and tolerability profile in this study.

"We are extremely pleased with the results from this proof-of-concept Phase 2 study of IONIS-FB-L_{Rx}, which demonstrated the potential to treat IgAN by inhibiting complement Factor B," said Richard Geary, Ph.D., executive vice president, drug development, at Ionis. "IgAN can lead to chronic kidney disease and kidney failure and has few treatment options. Given the significant unmet medical need, we are particularly pleased that Roche is moving expeditiously to advance IONIS-FB-L_{Rx} into Phase 3 development for IgAN in the first half of next year."

About the IONIS-FB-L_{Rx} Study

IONIS-FB-L_{Rx} is being evaluated in an ongoing open-label, single arm, Phase 2 clinical study in up to 25 participants with IgAN in two dose cohorts treated sequentially (NCT04014335). The first dose cohort included 10 patients treated for 29 weeks. The primary endpoint in the study is the change in 24-hour urine protein excretion from baseline to week 29. The study is also evaluating reduction in plasma complement Factor B, measures of kidney function and safety and tolerability of monthly dosing of IONIS-FB-L_{Rx}.

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 December 31, 2021, and the most recent Form 10-Q quarterly filing, which is 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.

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