

Ionis partners with Metagenomi to add gene editing to its broad technology platform

November 14, 2022

- *Collaboration with a leader in gene editing systems is Ionis' latest move to expand and diversify its technology*
- *CRISPR-Cas gene editing is a natural extension of Ionis' innovative approach to delivering potentially transformative therapies*
- *Ionis to hold webcast Nov. 14 at 8 a.m. Eastern Time*

CARLSBAD, Calif. and EMERYVILLE, Calif., Nov. 14, 2022 /PRNewswire/ -- [Ionis Pharmaceuticals, Inc.](#) (Nasdaq: IONS) and [Metagenomi](#) today announced that the companies have entered a collaboration that will leverage Ionis' extensive expertise in RNA-targeted therapeutics and Metagenomi's versatile next-generation gene editing systems to pursue a mix of validated and novel genetic targets that have potential to expand therapeutic options for patients. The companies will jointly conduct research aimed initially at delivering investigational medicines for up to four genetic targets. Ionis has the right to add four more targets upon achievement of pre-determined development milestones.

"Ionis was founded over 30 years ago to discover and develop novel, highly personalized medicines using our powerful RNA-targeting technology platform. This partnership with Metagenomi supports Ionis' strategic objective to advance our technology and expand our capabilities to deliver precision genetic medicines. Together, we can broaden the application of gene editing by leveraging Ionis' vast experience in nucleic acid therapeutics to optimize and extend the reach of gene editing for liver targets and to new tissues," said Ionis Chief Executive Officer Brett P. Monia, Ph.D. "This alliance brings our two companies closer to delivering next-generation therapies with the potential to revolutionize the treatment of diseases."

"Gene editing has the potential to transform chronic therapies into potentially curative treatments for patients who currently have limited options," said Brian C. Thomas, Ph.D., chief executive officer and founder of Metagenomi. "This collaboration is a strategic step to combine Metagenomi's leading gene editing toolbox of diverse, specific and highly efficient nucleases, with Ionis' pioneering expertise of RNA-targeted therapeutics, to catalyze the next wave of in vivo gene editing applications. We continue to follow our strategy of partnering with the most experienced teams in the genetic medicines field that will allow us to transform the lives of patients."

Under the terms of the agreement, Ionis will pay \$80 million upfront to Metagenomi plus the potential for future milestone payments and royalties. Wells Fargo Securities acted as financial advisor to Ionis on the transaction.

Webcast

Ionis will conduct a webcast on Nov. 14 at 8 a.m. Eastern Time to discuss this announcement and related activities. Interested parties may access the webcast [here](#). A webcast replay will be available for a limited time at the same address.

CRISPR Gene Editing

CRISPR (clustered regularly interspaced short palindromic repeats) gene editing is a technology that modifies the sequence of DNA in cells utilizing a specific RNA-guided nuclease (Cas enzyme). Gene editing enzymes act as molecular word processors to correct the genetic code at the precise spot where it is malfunctioning.

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.

About Metagenomi

Metagenomi is a gene editing company committed to developing potentially curative therapeutics by leveraging a proprietary toolbox of next-generation gene editing systems to accurately edit DNA where current technologies cannot. Our metagenomics-powered discovery platform and analytical expertise reveal novel cellular machinery sourced from otherwise unknown organisms. We adapt and forge these naturally evolved systems into powerful gene editing systems that are ultra-small, extremely efficient, highly specific and have a decreased risk of immune response. These systems fuel our pipeline of novel medicines and can be leveraged by partners. Our goal is to revolutionize gene editing for the benefit of patients around the world. For more information, please visit <https://metagenomi.co/>.

Ionis' Forward-looking Statements

This press release includes forward-looking statements regarding our collaboration and license agreement with Metagenomi, Inc., the therapeutic and commercial potential of genetic targets using gene editing technologies, Ionis' technologies and Ionis' 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 our 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 at www.ionispharma.com

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-partners-with-metagenomi-to-add-gene-editing-to-its-broad-technology-platform-301676345.html](https://www.prnewswire.com/news-releases/ionis-partners-with-metagenomi-to-add-gene-editing-to-its-broad-technology-platform-301676345.html)

SOURCE Ionis Pharmaceuticals, Inc.

Ionis Investor Contact: info@ionisph.com, 760-603-2331; Ionis Media Contact: corporatecommunications@ionisph.com, 760-603-4679; Metagenomi Investor Contact: Simon Harnest, simon@metagenomi.co, +1 (917) 403-1051; Metagenomi Media Contact: Ashlye Hodge, ashlye@metagenomi.co, +1 (510) 734-4409