

## **Ionis Pharmaceuticals Revises 2015 Financial Guidance and Provides Pipeline Update in Conjunction with J.P. Morgan Conference**

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### **Significantly improved net operating loss and increased cash projected**

CARLSBAD, Calif., Jan. 11, 2016 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS) today announced that the Company expects to significantly improve upon its financial guidance for 2015. The Company expects to end 2015 with a pro forma net operating loss (NOL) in the low \$20 million range and more than \$775 million in cash.



"Over the past several years, we have created a strong and consistent financial foundation. Our corporate partnerships have produced increasing levels of revenue as our partnered programs have matured and expanded. We have been able to support the advancement of our very large pipeline with modest expense growth due to the efficiency of our technology platform as well as the significant in-kind contributions of our partners. With three potentially transformational medicines close to commercialization, we look forward to adding product revenues and royalties to our revenue base over the next few years," said Elizabeth L. Hougen, chief financial officer of Ionis Pharmaceuticals. "We expect to end 2015 with a pro forma NOL in the low \$20 million range, which represents a 60% improvement over our original guidance. In addition, we expect to end 2015 with more than \$775 million in cash, substantially exceeding our 2014 year-end cash balance and our 2015 cash guidance. Importantly, our significant increase in revenue over the last several years reflects the successes in all of our collaborations as the drugs in those collaborations advanced."

The Company intends to provide financial guidance for 2016 in connection with its year-end 2015 financial results in February.

### **Pipeline Update**

Ionis management will present an update today on its drug development pipeline at the J.P. Morgan conference, including an update on its ongoing open-label Phase 2 clinical study of nusinersen in infants with Type I spinal muscular atrophy (SMA). Previously the company reported data from this study on event-free survival, measures of muscle function and assessments of developmental milestones. The data reported today show continued increases in median event-free survival and muscle function scores. The safety and tolerability profile of nusinersen to date continues to support further development.

In 2015, Ionis added nine new drugs to its pipeline. Already in 2016, the company has added two new drugs to its pipeline: IONIS-BIIB5<sub>Rx</sub> and IONIS-BIIB6<sub>Rx</sub>. Both new drugs are being developed together with Biogen and are designed to address undisclosed targets for the treatment of patients with neurodegenerative diseases. Ionis currently has a total of 38 drugs in its pipeline.

"We believe we are just beginning to realize the value we have created. We have three potentially groundbreaking medicines for which we have completed target enrollment in the respective pivotal Phase 3 trials and preparations are underway for each of these drugs for the necessary regulatory filings for marketing authorization. In addition, we and our partners are well along in preparing to commercialize these medicines. As our pipeline continues to mature, we have many other medicines coming along behind these three late-stage drugs," said B. Lynne Parshall, chief operating officer at Ionis. "We have also demonstrated in 2015 that the advances we are making in our technology continue to make better and better medicines, which allows us to expand the application of our drugs to new tissues, new targets and new diseases. All of

this sets us up for an exciting and event-filled 2016."

In January 2016, Ionis terminated its license agreement with Sanofi Genzyme for KYNAMRO<sup>®</sup> (mipomersen sodium).

"We are disappointed that we have had to terminate our contract with Genzyme to commercialize KYNAMRO," said Ms. Parshall. "We know that KYNAMRO is an important drug for patients with homozygous FH. Genzyme is continuing to support patients and physicians as we explore other potential commercialization options."

In addition, Ionis announced that results from the Phase 1/2 study with IONIS-AR-2.5<sub>Rx</sub> in heavily pretreated, late-stage prostate cancer patients demonstrated encouraging data, including several durable reductions in PSA levels. The drug also exhibited a good safety and tolerability profile supportive of continued development. Data from this study will be presented later in the year. Ionis plans to continue the development of IONIS-AR-2.5<sub>Rx</sub> independent of AstraZeneca.

#### **ABOUT IONIS PHARMACEUTICALS, INC.**

Ionis is the leading company in RNA-targeted drug discovery and development focused on developing drugs for patients who have the highest unmet medical needs, such as those patients with severe and rare diseases. Using its proprietary antisense technology, Ionis has created a large pipeline of first-in-class or best-in-class drugs, with over a dozen drugs in mid- to late-stage development. Drugs currently in Phase 3 development include volanesorsen, a drug Ionis is developing and plans to commercialize through its wholly owned subsidiary, Akcea Therapeutics, to treat patients with familial chylomicronemia syndrome and familial partial lipodystrophy; IONIS-TTR<sub>Rx</sub>, a drug Ionis is developing with GSK to treat patients with all forms of TTR amyloidosis; and nusinersen, a drug Ionis is developing with Biogen to treat infants and children with spinal muscular atrophy. Ionis' patents provide strong and extensive protection for its drugs and technology. Additional information about Ionis is available at [www.ionispharma.com](http://www.ionispharma.com).

#### **IONIS' FORWARD-LOOKING STATEMENT**

This press release includes forward-looking statements regarding Ionis Pharmaceuticals' business, financial guidance and the therapeutic and commercial potential of Ionis' technologies and products in development, including nusinersen, IONIS-TTR<sub>Rx</sub> and volanesorsen. 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, particularly those inherent in the process of discovering, developing and commercializing drugs that are safe and effective for use as human therapeutics, and in the endeavor of building a business around such drugs. 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, 2014, and its most recent quarterly report on Form 10-Q, which are on file with the SEC. Copies of these and other documents are available from the Company.

In December 2015, the Company changed its name from Isis Pharmaceuticals, Inc. to Ionis Pharmaceuticals, Inc. In this press release, unless the context requires otherwise, "Ionis," "Company," "we," "our," and "us" refers to Ionis Pharmaceuticals and its subsidiaries.

Ionis Pharmaceuticals<sup>™</sup> is a trademark of Ionis Pharmaceuticals, Inc. Akcea Therapeutics<sup>™</sup> is a trademark of Ionis Pharmaceuticals, Inc.

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