# Ionis exceeds 2018 financial guidance

February 27, 2019

# Total revenue increased 17% compared to 2017 to \$600 million Commercial revenues more than doubled Conference call and webcast today, February 27, 2019, at 11:30 a.m. Eastern Time

CARLSBAD, Calif., Feb. 27, 2019 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) today reported financial results for the fourth quarter and full year 2018 and reviewed highlights of its successful year.



"We begin 2019 in the strongest position in our 30-year history. Building on this foundation, we believe we are positioned for continued growth," said Stanley T. Crooke, M.D., Ph.D., chairman of the board and chief executive officer of Ionis. "In 2018, we launched TEGSEDI globally through our affiliate, Akcea, adding revenue from TEGSEDI sales to our substantial commercial revenue from SPINRAZA. We also achieved many important milestones in our pipeline, particularly among the medicines within our late-stage pipeline. This week, Novartis exercised its option to license AKCEA-APO(a)-L<sub>Rx</sub> for which we earned \$150 million. Novartis plans to initiate a Phase 3 cardiovascular outcomes study and initiation activities are already underway. We and Akcea are finalizing Phase 3 study designs for the AKCEA-TTR-L<sub>Rx</sub> pivotal program that we plan to initiate in the second half of this year. In addition, our late-stage neurological disease programs recently achieved important milestones. Roche is now enrolling patients in the Phase 3 study of IONIS-HTT<sub>Rx</sub> for Huntington's disease and Biogen is planning to add an additional cohort to the ongoing study of IONIS-SOD1<sub>Rx</sub> for patients with SOD1-related ALS that has the potential to support marketing approval. We achieved these successes while growing revenues, investing in the commercialization of TEGSEDI, advancing our broad and diverse pipeline, and consistently leading our industry in innovation – demonstrating the success of our business model and robust technology platform."

#### 2018 Financial Results and Highlights

- Revenues increased by 17 percent compared to 2017
  - Total revenue was \$600 million compared to \$514 million in 2017.
  - o Commercial revenue from SPINRAZA for 2018 was \$238 million, more than double compared to 2017.
  - TEGSEDI sales were \$2.2 million in the fourth quarter of 2018, with commercial sales commencing in the EU in October and in the U.S. in December.
  - Commercial revenue was over 40 percent of total revenue in 2018 compared to less than 25 percent in 2017, reflecting Ionis' transition to a commercial-stage company.
- Achieved third consecutive year of non-GAAP operating profitability
  - GAAP operating income was \$11 million for the fourth quarter and an operating loss of \$61 million for the full year
     2018, compared to an operating loss of \$6 million and operating income of \$31 million for the same periods in
     2017
  - Non-GAAP operating income was \$45 million for the fourth quarter and \$70 million for the full year 2018, compared to operating income of \$16 million and \$117 million for the same periods in 2017.
  - o Operating expenses increased in 2018 primarily due to investment in the commercialization of TEGSEDI.
- Strong financial results trigger the recognition of a significant tax benefit
  - In 2018, Ionis reported GAAP net income attributable to Ionis common stockholders of \$274 million, primarily driven by a \$291 million one-time non-cash income tax benefit Ionis recorded in 2018 related to its income tax assets.
  - Because of Ionis' strong financial performance over the past few years and its outlook regarding the continued growth of its business, the Company believes it is more likely than not that it will be able to use the significant amount of income tax assets it has accumulated to offset future taxable income.
- Substantial cash position of over \$2 billion enables continued investment in commercial products and pipeline

#### 2019 Financial Guidance

The Company's full year 2019 financial guidance consists of the following components (on a non-GAAP basis):

Net income on a non-GAAP basis				
Total Revenue	>\$725 million			
R&D Expenses	~\$360 million to \$390 million			
SG&A Expenses	~\$260 million to \$290 million			
Operating Income	>\$100 million			
Cash and Short-Term Investments	s ~\$2 billion			

"Building upon our success in 2018, we expect to continue our momentum this year and beyond. We anticipate both commercial and R&D revenues to contribute to our overall earnings growth this year, potentially making 2019 our fourth consecutive year of non-GAAP operating profitability. And for the first time, due to our strong financial performance in recent years and strong confidence in our future, we are projecting to be profitable on the bottom line on a non-GAAP basis in 2019. We believe our ability to be profitable while investing in commercial activities, fully exploiting our pipeline and advancing our technology, clearly sets us apart from our peers," said Elizabeth L. Hougen, chief financial officer of lonis.

All non-GAAP amounts referred to in this press release exclude non-cash compensation expense related to equity awards. Please refer to the reconciliation of non-GAAP and GAAP measures, which is provided later in this release. In prior financial results releases, lonis referred to amounts that excluded non-cash compensation expense related to equity awards as "pro forma". Additionally, lonis has labeled its prior period financial statements "as revised" to reflect the revenue recognition accounting standard the Company adopted on January 1, 2018.

#### **Business Highlights**

- SPINRAZA the worldwide standard-of-care for the treatment of all people with spinal muscular atrophy
  - In 2018, SPINRAZA sales nearly doubled to \$1.7 billion compared to 2017, driven by growth in the U.S. and outside the U.S., as reported by Biogen.
  - As of the fourth quarter of 2018, more than 6,600 SMA patients from over 40 countries were on SPINRAZA, including commercial patients and patients in the expanded access program and clinical trials.
  - o In the fourth quarter of 2018, the number of adult patients on therapy in the U.S. grew by over 20 percent compared to the third quarter, accounting for more than 50 percent of new patient starts in the U.S.
- TEGSEDI™ (inotersen) launch underway in multiple markets for the treatment of polyneuropathy of hereditary transthyretin amyloidosis (hATTR) in adult patients
  - TEGSEDI generated \$2.2 million in sales from the U.S. and EU in its first quarter of launch.
  - PTC Therapeutics, Ionis and Akcea's commercialization partner in Latin America, filed for marketing authorization for TEGSEDI in Brazil and was granted priority review.

#### **Key Upcoming Events**

- Roche plans to present data from the OLE study of IONIS-HTT<sub>Rx</sub> in patients with Huntington's disease in 2019.
- Biogen plans to present data from the completed portions of the Phase 1/2 study of IONIS-SOD1<sub>Rx</sub> in 2019.
- Ongoing regulatory discussions on WAYLIVRA in the EU, and if approved, launch.
- Ionis and its partners plan to report data from numerous Phase 2 studies including IONIS-FXI<sub>Rx</sub>, IONIS-HBV<sub>Rx</sub>, and IONIS-GHR-L<sub>Rx</sub>.

#### Revenue

Ionis' revenue in the three months and year ended December 31, 2018 was \$192 million and \$600 million, respectively, compared to \$168 million and \$514 million for the same periods in 2017 and was comprised of the following (amounts in millions):

	Three i	months ended,	Year ended		
	Dec	cember 31,	December 31,		
	2018	2017	2018	2017	
Revenue:		(as revised)		(as revised)	
Commercial revenue:					
SPINRAZA royalties	\$70	\$52	\$238	\$113	
TEGSEDI product sales, net	2	-	2	-	
Licensing and royalty revenue	1	2	15	7	
Total commercial revenue	73	54	255	120	
R&D Revenue:					
Amortization from upfront payments	33	28	125	98	
Milestone payments	38	17	83	152	
License fees	38	51	102	116	
Other services	10	18	35	28	
Total R&D revenue	119	114	345	394	
Total revenue	\$192	\$168	\$600	\$514	

The increase in revenue in 2018 compared to 2017 was primarily due to increasing commercial revenue from SPINRAZA royalties, which more than doubled. Additionally, Ionis earned more than \$2 million from TEGSEDI product sales in the fourth quarter of 2018.

lonis' R&D revenue demonstrates the Company's ability to generate sustainable revenue from its numerous partnerships. R&D revenue from the amortization of upfront payments increased over \$25 million in 2018 compared to 2017. The increase in amortization was primarily due to Ionis' 2018 strategic neurology collaboration with Biogen. Also, in 2018, Ionis added amortization revenue from its new collaboration with Roche to develop IONIS-FB-L<sub>Rx</sub>. Ionis' R&D revenue from milestone payments, license fees and other services for 2018 continued to make a significant contribution to Ionis' financial results.

Already in the first quarter of 2019, Ionis has earned \$185 million. The Company earned \$150 million from Novartis when it licensed AKCEA-APO(a)-L<sub>Rx</sub> and \$35 million from Roche when it enrolled the first patient in the Phase 3 study of IONIS-HTT<sub>Rx</sub> in patients with Huntington's disease.

# **Operating Expenses**

Operating expenses for the three months and year ended December 31, 2018 on a GAAP basis were \$181 million and \$661 million, respectively, and on a non-GAAP basis were \$147 million and \$530 million, respectively. These amounts compare to GAAP operating expenses for the three months and year ended December 31, 2017 of \$174 million and \$483 million, respectively, and non-GAAP operating expenses of \$152 million and \$397 million, respectively. The full year increase in operating expenses in 2018 compared to 2017 was principally due to Ionis' investments in the global launch of TEGSEDI. The Company's SG&A expenses also increased due to an increase in fees the Company owed under its in-licensing agreements related to SPINRAZA, due to increased SPINRAZA product sales.

#### **Income Tax Benefit**

lonis reported an income tax benefit of \$292 million and \$291 million for the three months and year ended December 31, 2018, respectively, compared to \$7 million and \$6 million for the same periods in 2017. Ionis' tax benefit increased significantly in 2018 primarily due to a one-time non-cash tax benefit related to its deferred income tax assets. In the fourth quarter of 2018, the Company released a large portion of the valuation allowance associated with its deferred tax assets. Because of Ionis' strong financial performance over the past few years and its outlook regarding the continued growth of its business, the Company determined that it is more likely than not that it will be able to utilize most of the deferred income tax assets it has accumulated to offset future taxable income.

#### Net Loss Attributable to Noncontrolling Interest in Akcea

At December 31, 2018, Ionis owned approximately 75 percent of Akcea. The shares of Akcea third parties own represent an interest in Akcea's equity that Ionis does not control. However, because Ionis continues to maintain overall control of Akcea through its voting interest, Ionis reflects the assets, liabilities and results of operations of Akcea in Ionis' consolidated financial statements. Ionis reflects the noncontrolling interest attributable to other owners of Akcea's common stock in a separate line called "Net Ioss attributable to noncontrolling interest in Akcea" on Ionis' statement of operations. Ionis' net Ioss attributable to noncontrolling interest in Akcea for the three months and year ended December 31, 2018, was \$17 million and \$59 million, respectively. Ionis' net Ioss attributable to noncontrolling interest in Akcea for the three months and year ended December 31, 2017, was \$6 million and \$11 million, respectively.

#### Net Income (Loss) Attributable to Ionis Common Stockholders

lonis reported net income attributable to lonis' common stockholders of \$320 million and \$274 million for the three months and year ended December 31, 2018, respectively, compared to a net loss of \$3 million and net income of \$0.3 million for the same periods in 2017, all on a GAAP basis. On a non-GAAP basis, lonis reported net income attributable to lonis' common stockholders of \$351 million and \$394 million for the three months and year ended December 31, 2018, respectively, compared to \$18 million and \$85 million for the same periods in 2017. The increase in 2018 net income attributable to lonis' common stockholders was primarily due to increases in revenue and the income tax benefit lonis recognized in the fourth quarter of 2018.

For the three months ended December 31, 2018, basic and diluted net income per share were \$2.32 and \$2.21, respectively. For the year ended December 31, 2018, basic and diluted net income per share were \$2.09 and \$2.07, respectively. For the three months ended December 31, 2017, basic and diluted net loss per share were each \$0.03. For the year ended December 31, 2017, basic and diluted net income per share were each \$0.15. All amounts are on a GAAP basis.

#### **Balance Sheet**

As of December 31, 2018, Ionis had cash, cash equivalents and short-term investments of \$2.1 billion compared to \$1.0 billion at December 31, 2017. The increase in Ionis' cash, cash equivalents and short-term investments was primarily due to the \$1 billion Ionis received from Biogen for the 2018 strategic neurology collaboration.

#### **Webcast and Conference Call**

Today, at 11:30 a.m. Eastern Time, Ionis will conduct a live webcast conference call to discuss this earnings release and related activities. Interested parties may listen to the call by dialing 877-443-5662 or access the webcast at <a href="https://www.ionispharma.com">www.ionispharma.com</a>. A webcast replay will be available for a limited time.

# About Ionis Pharmaceuticals, Inc.

As the leader in RNA-targeted drug discovery and development, lonis has created an efficient, broadly applicable, proprietary antisense technology platform with the potential to treat diseases where no other therapeutic approaches have proven effective. Our drug discovery platform has served as a springboard for actionable promise and realized hope for patients with unmet needs – such as children and adults with spinal muscular atrophy (SMA). We created SPINRAZA® (nusinersen)\* and are proud to have brought new hope to the SMA community by developing the first and only approved treatment for this disease.

Our sights are set on all the patients we have yet to reach with a pipeline of more than 40 medicines with the potential to treat patients with cardiovascular disease, rare diseases, neurological diseases, infectious diseases and cancer. We created TEGSEDI™ (inotersen) the world's first RNA-targeted therapeutic approved for the treatment of polyneuropathy of hereditary transthyretin (TTR) amyloidosis (ATTR) in adult patients that our affiliate Akcea Therapeutics is commercializing. Together with Akcea, we are also bringing new medicines to patients with cardiometabolic lipid disorders.

To learn more about Ionis follow us on twitter @ionispharma or visit http://ir.ionispharma.com/.

\*Spinraza is marketed by Biogen.

# Ionis' Forward-looking Statement

This press release includes forward-looking statements regarding Ionis' business, financial guidance and the therapeutic and commercial potential of SPINRAZA (nusinersen) and TEGSEDI (inotersen) and Ionis' technologies and products in development, including the business of Akcea Therapeutics, Inc., Ionis' majority-owned affiliate. 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, 2017, and most recent Form 10-Q quarterly filing, which are on file with the SEC. 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.

lonis Pharmaceuticals <sup>™</sup> is a trademark of lonis Pharmaceuticals, Inc.Akcea Therapeutics <sup>™</sup> is a trademark of Akcea Therapeutics, Inc. TEGSEDI <sup>™</sup> is a trademark of Akcea Therapeutics, Inc. WAYLIVRA <sup>™</sup> is a trademark of Akcea Therapeutics, Inc. SPINRAZA<sup>®</sup> is a registered trademark of Biogen.

# IONIS PHARMACEUTICALS, INC. SELECTED FINANCIAL INFORMATION Condensed Consolidated Statements of Operations (In Thousands, Except Per Share Data)

Revenue:   Commercial revenue:   SPINRAZA royalties   S70,187   S52,073   S237,930   S112,540			oths ended, ober 31, 2017		ended, nber 31, 2017
Revenue:           Commercial revenue:           SPINRAZA royalties         \$70,187         \$52,073         \$23,930         \$112,540           Licensing and royalty revenue         523         1,835         14,755         7,474           Licensing and royalty revenue         523         1,835         344,755         394,165           Research and development revenue under collaborative agreements         119,166         113,833         344,752         394,165           Total revenue         72,947         167,792         599,674         514,179           Expenses:         2         181,331         167,792         599,674         514,179           Cost of products sold         777         7         1,820         514,179           Research, development and patent         113,451         128,285         414,604         374,644           Selling, general and administrative         67,103         45,707         24,622         108,488           Total operating expenses         181,331         145,092         61,046         483,132           Income (loss) from operations         10,782         (6,200)         (61,372)         31,047           Other income (expense):         11,488         673         30,		(	(as revised)		
Commercial revenue:         \$70,187         \$52,073         \$23,70         \$12,540           TEGSEDI product sales, net         2,237         2,237         7,47           Licensing and royalty revenue         523         1,835         14,755         7,474           Total commercial revenue         72,947         53,908         254,922         120,014           Research and development revenue under collaborative agreements         119,166         113,83         344,752         394,165           Total revenue         192,113         167,792         59,674         514,179           Expenses:         2         181,315         167,792         59,674         514,179           Expenses:         8         181,331         167,792         59,674         514,179           Research, development and patent         113,451         128,285         414,604         374,644           Selling, general and administrative         67,103         457,07         24,622         108,488           Total operating expenses         181,331         173,992         661,046         483,132           Income (loss) from operations         11,484         673         30,187         8,179           Other income (expenses)         (11,458)         (10,787)<	Revenue:				
TEGSEDI product sales, net         2,237         2,237         2,237         1,755         7,474           Licensing and royalty revenue         523         1,835         14,755         7,474           Total commercial revenue         72,947         53,908         254,922         120,014           Research and development revenue under collaborative agreements         1191,166         113,883         344,752         394,165           Total revenue         777         15,902         596,74         514,179           Expenses:         777         1,820         514,179           Research, development and patent         113,451         128,285         414,604         374,644           Selling, general and administrative         67,103         45,707         244,622         108,488           Total operating expenses         181,331         173,992         661,046         483,132           Income (loss) from operations         10,782         (6,00)         (61,372)         31,047           Other income (expenses):         11,484         673         30,187         8,179           Interest expense         (11,458)         (10,787)         (47,789)         (44,752)           Loss on debt financing liability for leased facility         2,620         2,					
Licensing and royalty revenue         523         1,835         14,755         7,474           Total commercial revenue         72,947         53,908         254,922         120,014           Research and development revenue under collaborative agreements         119,166         113,863         344,752         394,165           Total revenue         192,113         167,792         50,672         514,179           Expenses:         70         152,113         128,285         14,804         374,644           Research, development and patent         113,451         128,285         414,604         374,644           Selling, general and administrative         67,103         45,707         246,222         108,488           Selling, speneral and administrative         67,103         45,707         246,222         108,488           Total operating expenses         181,331         173,99         661,046         483,132           Income (loss) from operations         10,782         (6,200)         (6,372         31,047           University in income (expenses)         111,484         673         30,187         8,179           Income (loss) before income tax benefit         11,458         10,789         44,789         44,789           Other expenses	SPINRAZA royalties	\$70,187	\$52,073	\$237,930	\$112,540
Total commercial revenue   72,947   53,908   254,922   120,014   Research and development revenue under collaborative agreements   119,166   113,833   344,752   394,165   Total revenue   192,113   167,792   599,674   514,179   Expenses:     192,113   167,792   599,674   514,179   Expenses:     192,113   167,792   599,674   514,179   Expenses:   132,451   128,285   414,604   374,644   Selling, general and administrative   67,103   45,707   244,622   108,488   Total operating expenses   181,331   173,992   661,046   483,132   Income (loss) from operations   10,782   (6,200)   (61,372)   31,047   Other income (expense):   11,484   673   30,187   8,179   Interest expense   11,484   673   30,187   44,789   (44,782)   Loss on debt financing liability for leased facility   1,458   (10,787)   (16,333)   (76,156)   (16,763)   Income (loss) before income tax benefit   10,771   (16,333)   (76,156)   (16,763)   Income tax benefit   291,964   7,164   291,141   5,980   Net income (loss) attributable to noncontrolling interest in Akcea Therapeutics, Inc.   330,785   \$(9,169)   \$214,985   \$(10,783)   Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc.   330,078   \$(2,922)   \$(273,741)   \$(346)   \$(375)   (37	TEGSEDI product sales, net	2,237	-	2,237	-
Research and development revenue under collaborative agreements         119,166         113,883         344,752         394,165           Total revenue         192,113         167,792         599,674         514,179           Expenses:         Cost of products sold         777         -         1,820         -           Research, development and patent         113,451         128,285         414,604         374,644           Selling, general and administrative         67,103         45,707         244,622         108,488           Total operating expenses         181,331         173,992         661,046         483,132           Income (loss) from operations         10,782         (6,200)         (61,372)         31,047           Other income (expenses):         Investment income         11,484         673         30,187         8,179           Interest expense         (11,458)         (10,787)         (44,789)         (44,752)           Loss on debt financing liability for leased facility         -         -         -         (7,689)           Other expenses         (37)         (19)         (182)         (3,548)           Income (loss) before income tax benefit         291,964         7,164         291,141         5,980 <tr< td=""><td>Licensing and royalty revenue</td><td>523</td><td>1,835</td><td>14,755</td><td>7,474</td></tr<>	Licensing and royalty revenue	523	1,835	14,755	7,474
Total revenue         192,113         167,792         599,674         514,179           Expenses:         Cost of products sold         777         - 1,820         - 1,820         - 1,820         - 1,820         - 1,820         - 1,820         - 1,820         - 1,820         - 1,820         - 1,820         - 1,820         - 1,820         - 1,820         - 1,820         - 1,820         - 1,820         - 1,820         - 2,828         414,604         374,644         821,619,913         - 1,820         - 1,820         - 2,828         18,488         570,70         244,622         108,488         - 10,782         66,7103         45,707         244,622         108,488         100,600         10,782         (6,200)         (61,372)         31,047         010,448         10,772         24,622         108,488         100,772         10,782         61,046         483,132         10,000         (61,072)         31,047         010,448         10,772         010,782         01,047	Total commercial revenue	72,947	53,908	254,922	120,014
Expenses:         Cost of products sold         777         1,820         -           Research, development and patent         113,451         128,285         414,604         374,644           Selling, general and administrative         67,103         45,707         244,622         108,488           Total operating expenses         181,331         173,992         661,046         483,132           Income (loss) from operations         10,782         (6,200)         (61,372)         31,047           Other income (expense):         11,484         673         30,187         8,179           Interest expense         (11,458)         (10,787)         (44,789)         (44,752)           Loss on debt financing liability for leased facility         11,484         673         30,187         8,179           Income (loss) before income tax benefit         10,771         (16,333)         (76,156)         (16,763)           Income (loss) before income tax benefit         291,964         7,164         291,141         5,980           Net income (loss)         \$302,735         \$(9,169)         \$214,985         \$(10,783)           Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc.         17,343         6,247         58,756         11,129           Net	Research and development revenue under collaborative agreements	119,166	113,883	344,752	394,165
Cost of products sold         777         - 1,820         - 1,820         - 1,820         - 1,820         - 1,820         - 1,820         - 374,644         - 374,644         - 374,644         - 374,644         - 374,644         - 374,644         - 374,644         - 374,644         - 374,644         - 374,644         - 374,644         - 374,644         - 374,042         - 374,644         - 374,644         - 374,644         - 374,042         - 374,644         - 374,644         - 374,644         - 374,644         - 374,644         - 374,644         - 374,644         - 374,644         - 374,645         - 374,645         - 374,645         - 374,645         - 374,645         - 374,645         - 374,645         - 374,645         - 374,645         - 374,647         - 37	Total revenue	192,113	167,792	599,674	514,179
Research, development and patent         113,451         128,285         414,604         374,644           Selling, general and administrative         67,103         45,707         244,622         108,488           Total operating expenses         181,331         173,992         661,046         483,132           Income (loss) from operations         10,782         (6,200)         (61,372)         31,047           Other income (expenses):         11,484         673         30,187         8,179           Investment income         (11,458)         (10,787)         (44,789)         (44,752)           Loss on debt financing liability for leased facility         -         -         -         -         -         (7,689)           Other expenses         (37)         (19)         (182)         (3,548)           Income (loss) before income tax benefit         10,771         (16,33)         (76,156)         (16,763)           Net income (loss)         \$291,964         7,164         291,141         5,980           Net income (loss)         \$214,985         \$(10,783)           Net income (loss)         \$214,985         \$(10,783)           Net income (loss) attributable to lonis Pharmaceuticals, Inc. common stockholders         \$20,078         \$(2,922)         \$27	Expenses:				
Selling, general and administrative         67,103         45,707         244,622         100,488           Total operating expenses         181,331         173,992         661,046         483,132           Income (loss) from operations         10,782         (6,200)         (61,372)         31,047           Other income (expense):         11,484         673         30,187         8,179           Investment income         11,458         (10,787)         (44,789)         (44,752)           Loss on debt financing liability for leased facility         (11,458)         (10,787)         (44,789)         (44,752)           Other expenses         (37)         (19)         (182)         (3,548)           Income (loss) before income tax benefit         10,771         (16,333)         (76,156)         (16,763)           Net income (loss)         \$302,735         \$(9,169)         \$214,985         \$(10,783)           Net income (loss)         \$302,735         \$(9,169)         \$214,985         \$(10,783)           Net income (loss) attributable to lonis Pharmaceuticals, Inc. common stockholders         \$320,078         \$(2,922)         \$273,741         \$346           Basic net income (loss) per share         \$2.21         \$(0.03)         \$2.09         \$0.15           Dilut	Cost of products sold	777	-	1,820	-
Total operating expenses         181,331         173,992         661,046         483,132           Income (loss) from operations         10,782         (6,200)         (61,372)         31,047           Other income (expense):         Investment income         11,484         673         30,187         8,179           Interest expense         (11,458)         (10,787)         (44,789)         (44,752)           Loss on debt financing liability for leased facility         -         -         -         -         (7,689)           Other expenses         (37)         (19)         (182)         (3,548)           Income (loss) before income tax benefit         10,771         (16,333)         (76,156)         (16,763)           Income tax benefit         291,964         7,164         291,141         5,980           Net income (loss)         \$302,735         \$(9,169)         \$214,985         \$(10,783)           Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc.         17,343         6,247         58,756         11,129           Net income (loss) attributable to lonis Pharmaceuticals, Inc. common stockholders         \$320,078         \$(2,922)         \$273,741         \$346           Basic net income (loss) per share         \$2.32         \$(0.03)	Research, development and patent	113,451	128,285	414,604	374,644
Income (loss) from operations         10,782         (6,200)         (61,372)         31,047           Other income (expense):         Investment income         11,484         673         30,187         8,179           Interest expense         (11,458)         (10,787)         (44,789)         (44,752)           Loss on debt financing liability for leased facility         -         -         -         -         -         (7,689)           Other expenses         (37)         (19)         (182)         (3,548)           Income (loss) before income tax benefit         10,771         (16,333)         (76,156)         (16,763)           Income tax benefit         291,964         7,164         291,141         5,980           Net income (loss)         \$302,735         \$(9,169)         \$214,985         \$(10,783)           Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc.         17,343         6,247         58,756         11,129           Net income (loss) attributable to lonis Pharmaceuticals, Inc. common stockholders         \$320,078         \$(2,922)         \$273,741         \$346           Basic net income (loss) per share         \$2.32         \$(0.03)         \$2.09         \$0.15           Shares used in computing basic net income (loss) per share         137,699<	Selling, general and administrative	67,103	45,707	244,622	108,488
Other income (expense):         Investment income       11,484       673       30,187       8,179         Interest expense       (11,458)       (10,787)       (44,789)       (44,752)         Loss on debt financing liability for leased facility       -       -       -       -       (7,689)         Other expenses       (37)       (19)       (182)       (3,548)         Income (loss) before income tax benefit       10,771       (16,333)       (76,156)       (16,763)         Income tax benefit       291,964       7,164       291,141       5,980         Net income (loss)       \$302,735       \$(9,169)       \$214,985       \$(10,783)         Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc.       17,343       6,247       58,756       11,129         Net income (loss) attributable to lonis Pharmaceuticals, Inc. common stockholders       \$320,078       \$(2,922)       \$273,741       \$346         Basic net income (loss) per share       \$2.32       \$(0.03)       \$2.09       \$0.15         Diluted net income (loss) per share       \$2.21       \$(0.03)       \$2.07       \$0.15         Shares used in computing basic net income (loss) per share       137,699       124,818       132,320       124,016	Total operating expenses	181,331	173,992	661,046	483,132
Investment income   11,484   673   30,187   8,179     Interest expense   (11,458)   (10,787)   (44,789)   (44,752)     Loss on debt financing liability for leased facility   (7,689)     Other expenses   (37)   (19)   (182)   (3,548)     Income (loss) before income tax benefit   10,771   (16,333)   (76,156)   (16,763)     Income tax benefit   291,964   7,164   291,141   5,980     Net income (loss)   \$302,735   \$(9,169)   \$214,985   \$(10,783)     Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc.   17,343   6,247   58,756   11,129     Net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders   \$320,078   \$(2,922)   \$273,741   \$346     Basic net income (loss) per share   \$2.32   \$(0.03)   \$2.09   \$0.15     Diluted net income (loss) per share   \$2.21   \$(0.03)   \$2.07   \$0.15     Shares used in computing basic net income (loss) per share   \$137,699   124,818   313,320   124,816   132,320   124	Income (loss) from operations	10,782	(6,200)	(61,372)	31,047
Interest expense	Other income (expense):				
Loss on debt financing liability for leased facility       -        -       -       -       -       -       -       -       -       -       -       -       -       -       -       -        -       -       -       -       -       -       -       -       -       -       -       -       -       -       -        -       -       -       -       -       -       -       -       -       -       -       -       -       -       -        -       -       -       -       -       -       -       -       -       -       -       -       -       -       -        -       -       -       -       -       -       -       -       -       -       -       -       -       -       -        -       -       -       -       -       -       -       - </td <td>Investment income</td> <td>11,484</td> <td>673</td> <td>30,187</td> <td>8,179</td>	Investment income	11,484	673	30,187	8,179
Other expenses         (37)         (19)         (182)         (3,548)           Income (loss) before income tax benefit         10,771         (16,333)         (76,156)         (16,763)           Income tax benefit         291,964         7,164         291,141         5,980           Net income (loss)         \$302,735         \$(9,169)         \$214,985         \$(10,783)           Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc.         17,343         6,247         58,756         11,129           Net income (loss) attributable to lonis Pharmaceuticals, Inc. common stockholders         \$320,078         \$(2,922)         \$273,741         \$346           Basic net income (loss) per share         \$2.32         \$(0.03)         \$2.09         \$0.15           Diluted net income (loss) per share         \$2.21         \$(0.03)         \$2.07         \$0.15           Shares used in computing basic net income (loss) per share         137,699         124,818         132,320         124,016	•	(11,458)	(10,787)	(44,789)	, ,
Income (loss) before income tax benefit         10,771         (16,333)         (76,156)         (16,763)           Income tax benefit         291,964         7,164         291,141         5,980           Net income (loss)         \$302,735         \$(9,169)         \$214,985         \$(10,783)           Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc.         17,343         6,247         58,756         11,129           Net income (loss) attributable to lonis Pharmaceuticals, Inc. common stockholders         \$320,078         \$(2,922)         \$273,741         \$346           Basic net income (loss) per share         \$2.32         \$(0.03)         \$2.09         \$0.15           Diluted net income (loss) per share         \$2.21         \$(0.03)         \$2.07         \$0.15           Shares used in computing basic net income (loss) per share         137,699         124,818         132,320         124,016	,	-	-	-	, ,
Income tax benefit         291,964         7,164         291,141         5,980           Net income (loss)         \$302,735         \$(9,169)         \$214,985         \$(10,783)           Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc.         17,343         6,247         58,756         11,129           Net income (loss) attributable to lonis Pharmaceuticals, Inc. common stockholders         \$320,078         \$(2,922)         \$273,741         \$346           Basic net income (loss) per share         \$2.32         \$(0.03)         \$2.09         \$0.15           Diluted net income (loss) per share         \$2.21         \$(0.03)         \$2.07         \$0.15           Shares used in computing basic net income (loss) per share         137,699         124,818         132,320         124,016	·		<u> </u>		
Net income (loss)         \$302,735         \$(9,169)         \$214,985         \$(10,783)           Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc.         17,343         6,247         58,756         11,129           Net income (loss) attributable to lonis Pharmaceuticals, Inc. common stockholders         \$320,078         \$(2,922)         \$273,741         \$346           Basic net income (loss) per share         \$2.32         \$(0.03)         \$2.09         \$0.15           Diluted net income (loss) per share         \$2.21         \$(0.03)         \$2.07         \$0.15           Shares used in computing basic net income (loss) per share         137,699         124,818         132,320         124,016	Income (loss) before income tax benefit	10,771	(16,333)	(76,156)	(16,763)
Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc. $17,343$ $6,247$ $58,756$ $11,129$ Net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders $320,078$ $(2,922)$ $273,741$ $346$ Basic net income (loss) per share $2.32$ $(0.03)$ $2.09$ $2.09$ $30.15$ Diluted net income (loss) per share $32.21$ $(0.03)$ $20.07$ $20.15$ Shares used in computing basic net income (loss) per share $32.21$ $30.09$ $30.15$ $30.15$ Shares used in computing basic net income (loss) per share	Income tax benefit	291,964	7,164	291,141	5,980
Net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders         \$320,078         \$(2,922)         \$273,741         \$346           Basic net income (loss) per share         \$2.32         \$(0.03)         \$2.09         \$0.15           Diluted net income (loss) per share         \$2.21         \$(0.03)         \$2.07         \$0.15           Shares used in computing basic net income (loss) per share         137,699         124,818         132,320         124,016	Net income (loss)	\$302,735	\$(9,169)	\$214,985	\$(10,783)
Net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders         \$320,078         \$(2,922)         \$273,741         \$346           Basic net income (loss) per share         \$2.32         \$(0.03)         \$2.09         \$0.15           Diluted net income (loss) per share         \$2.21         \$(0.03)         \$2.07         \$0.15           Shares used in computing basic net income (loss) per share         137,699         124,818         132,320         124,016	Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc.	17,343	6,247	58,756	11,129
Diluted net income (loss) per share         \$2.21         \$(0.03)         \$2.07         \$0.15           Shares used in computing basic net income (loss) per share         137,699         124,818         132,320         124,016		\$320,078	\$(2,922)		\$346
Diluted net income (loss) per share         \$2.21         \$(0.03)         \$2.07         \$0.15           Shares used in computing basic net income (loss) per share         137,699         124,818         132,320         124,016					
Shares used in computing basic net income (loss) per share 137,699 124,818 132,320 124,016	Basic net income (loss) per share	\$2.32	\$(0.03)	\$2.09	<u>\$0.15</u>
Shares used in computing basic net income (loss) per share 137,699 124,818 132,320 124,016	Diluted net income (loss) per share	\$2.21	\$(0.03)	\$2.07	\$0.15
enales assume the means (1999) per share	· / /	137,699	124,818	132,320	124,016

# IONIS PHARMACEUTICALS, INC. SELECTED FINANCIAL INFORMATION Condensed Consolidating Statement of Operations (In Thousands)

		Year Ended,			
		December 31, 2018			
	lonis	Akcea	Eliminations	Ionis Consolidated	
Revenue:					
Commercial revenue:					
SPINRAZA royalties	\$237,930	\$	- \$-	\$237,930	

TEGSEDI product sales, net	-	2,237	-	2,237
Licensing and royalty revenue	2,755	12,000		14,755
Total commercial revenue	240,685	14,237	-	254,922
Research and development revenue under collaborative agreements	294,122	50,630	-	344,752
Intercompany revenue	107,137	<u> </u>	(107,137)	
Total revenue	641,944	64,867	(107,137)	599,674
Expenses:				
Cost of products sold	-	11,733	(9,913)	1,820
Research, development and patent expenses	289,650	130,340	(5,386)	414,604
Selling, general and administrative	90,562	153,610	450	244,622
Total operating expenses	380,212	295,683	(14,849)	661,046
Income (loss) from operations	261,732	(230,816)	(92,288)	(61,372)
Other income (expense):				
Investment income	24,563	5,624	-	30,187
Interest expense	(44,789)	-	-	(44,789)
Other expenses		(182)		(182)
Income (loss) before income tax expense	241,506	(225,374)	(92,288)	(76,156)
Income tax (expense) benefit	291,588	(447)		291,141
Net income (loss)	\$533,094	\$(225,821)	\$(92,288)	\$214,985
Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc.	\$-	\$-	\$58,756	\$58,756
Net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders	\$533,094	\$(225,821)	\$(33,532)	\$273,741

# IONIS PHARMACEUTICALS, INC. Reconciliation of GAAP to Non-GAAP Basis: Condensed Consolidated Operating Expenses, Income (Loss) From Operations, and Net Income (Loss) (In Thousands)

	Three months				
	ended, Year e			ended,	
	Decem	nber 31,	Decem	December 31,	
	2018	2017	2018	2017	
		(as		(as	
		revised)		revised)	
		(unau	dited)		
As reported research, development and patent expenses according to GAAP	\$113,451	\$128,285	\$414,604	\$374,644	
Excluding compensation expense related to equity awards	(18,860)	(16,078)	(76,557)	(64,521)	
Non-GAAP research, development and patent expenses	\$94,591	\$112,207	\$338,047	\$310,123	
As reported selling, general and administrative expenses according to GAAP	\$67,103	\$45,707	\$244,622	\$108,488	
Excluding compensation expense related to equity awards	(15,083)	(6,255)	(54,595)	(21,454)	
		_			
Non-GAAP selling, general and administrative expenses	\$52,020	\$39,452	\$190,027	\$87,034	
As reported operating expenses according to GAAP	\$181,331	\$173,992	\$661,046	\$483,132	
Excluding compensation expense related to equity awards	(34,103)	(22,333)	(131,312)	(85,975)	
Non-GAAP operating expenses	\$147,228	\$151,659	\$529,734	\$397,157	
As reported income (loss) from operations according to GAAP	\$10,782	\$(6,200)	\$(61,372)	\$31,047	
Excluding compensation expense related to equity awards	(34,103)	(22,333)	(131,312)	(85,975)	
Non-GAAP income from operations	\$44,885	\$16,133	\$69,940	\$117,022	
As reported net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders	<del></del>				
according to GAAP	\$320,078	\$(2,922)	\$273,741	\$346	
Excluding compensation expense related to equity awards attributable to Ionis Pharmaceuticals, Inc.		, ,			
common stockholders	(30,897)	(20,518)	(119,790)	(84,369)	
Non-GAAP net income attributable to Ionis Pharmaceuticals, Inc. common stockholders accordin	g				
to GAAP	\$350,975	\$17,596	\$393,531	\$84,715	

### Reconciliation of GAAP to Non-GAAP Basis

As illustrated in the Selected Financial Information in this press release, non-GAAP operating expenses, non-GAAP income (loss) from operations, and non-GAAP net income (loss) were adjusted from GAAP to exclude compensation expense related to equity awards, which are non-cash. Ionis has regularly reported non-GAAP measures for operating results as non-GAAP results. These measures are provided as supplementary information and are not a substitute for financial measures calculated in accordance with GAAP. Ionis reports these non-GAAP results to better enable financial statement users to assess and compare its historical performance and project its future operating results and cash flows. Further, the presentation of Ionis' non-GAAP results is consistent with how Ionis' management internally evaluates the performance of its operations. In prior financial results releases, Ionis referred to amounts that excluded non-cash compensation expense related to equity awards as "pro forma".

# IONIS PHARMACEUTICALS, INC. Condensed Consolidated Balance Sheets (In Thousands)

	December 31, 2018	December 31, 2017
		(as revised)
Assets:		,
Cash, cash equivalents and short-term investments	\$2,084,072	\$1,022,715
Contracts receivable	12,759	62,955
Other current assets	111,055	83,064
Property, plant and equipment, net	132,160	121,907
Other assets	327,738	32,133
Total assets	\$2,667,784	\$1,322,774
Liabilities and stockholders' equity:		
Other current liabilities	\$120,038	\$118,276
Current portion of deferred contract revenue	160,256	125,336
1% convertible senior notes	568,215	533,111
Long-term obligations, less current portion	64,756	72,745
Long-term deferred contract revenue	567,359	108,026
Total Ionis stockholders' equity	1,048,079	281,013
Noncontrolling interest in Akcea Therapeutics, Inc.	139,081	84,267
Total stockholders' equity	1,187,160	365,280
Total liabilities and stockholders' equity	\$2,667,784	\$1,322,774

# IONIS PHARMACEUTICALS, INC. Condensed Consolidating Balance Sheet (In Thousands)

December 31, 2018

				Ionis
	Ionis	Akcea	Eliminations	Consolidated
Assets:				
Cash, cash equivalents and short-term investments	\$1,831,463	\$252,609	\$-	\$2,084,072
Contracts receivable	10,763	1,996	-	12,759
Receivable from Akcea Therapeutics, Inc.	18,901	-	(18,901)	-
Other current assets	102,613	12,630	(4,188)	111,055
Property, plant and equipment, net	126,464	5,696	-	132,160
Other assets	885,288	92,330	(649,880)	327,738
Total assets	\$2,975,492	\$365,261	\$(672,969)	\$2,667,784
Liabilities and stockholders' equity:				
Other current liabilities	\$87,820	\$55,307	\$(23,089)	\$120,038
Current portion of deferred contract revenue	134,902	25,354	-	160,256
1% convertible senior notes	568,215	-	-	568,215
Long-term obligations, less current portion	60,314	4,442	-	64,756
Long-term deferred contract revenue	565,624	3,434	(1,699)	567,359
Total stockholders' equity before noncontrolling interest	1,558,617	276,724	(787,262)	1,048,079
Noncontrolling interest in Akcea Therapeutics, Inc.			139,081	139,081
Total stockholders' equity	1,558,617	276,724	(648,181)	1,187,160
Total liabilities and stockholders' equity	\$2,975,492	\$365,261	\$(672,969)	\$2,667,784

SOURCE Ionis Pharmaceuticals, Inc.

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