



## Exicure, Inc. Reports Full Year 2019 Financial Results and Corporate Progress

March 3, 2020

- *Friedreich's ataxia chosen as first neurological indication*
- *Reported Phase 1b data for lead immuno-oncology candidate in solid tumors*
- *Development partnership with Allergan in hair loss disorders*
- *Cash and short-term investments on Dec. 31, 2019 of \$110.8 million*

CHICAGO & CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Exicure, Inc. (NASDAQ:XCUR), the pioneer in gene regulatory and immunotherapeutic drugs utilizing spherical nucleic acid (SNA™) technology, today reported full year financial results for year ended December 31, 2019 and provided an update on corporate progress.

"2019 was a pivotal year for Exicure as we joined strong scientific and clinical progress with the capital resources and distinguished investors necessary to expand our pipeline and build our organization," said Dr. David Giljohann, Chief Executive Officer of Exicure. "Looking ahead, we intend to invest in growing a pipeline of drug candidates targeting neurological indications, as well as advance our Merkel cell and cutaneous squamous cell carcinoma programs into Phase 2 trials. We believe our ongoing clinical progress in immuno-oncology and dermatology has influenced our ability to expand our SNA platform in additional therapeutic areas through collaborative partnerships. Our collaboration with Allergan in hair loss disorders is just one of what we hope will be a number of such partnerships expanding our SNA platform into new therapeutic areas," concluded Dr. Giljohann.

### Corporate Progress

Key achievements for Exicure during 2019 include:

- **Established Friedreich's ataxia as Exicure's first neurological indication, being developed in collaboration with the Friedreich's Ataxia Research Alliance**
- **Announced preliminary data in the ongoing Phase 1b/2 trial of lead immuno-oncology candidate AST-008 in patients with solid tumors**
- **Entered a collaboration agreement with Allergan for two discovery programs in hair loss disorders with \$25 million upfront and up to \$725 million in potential milestones**
- **Expanded scientific advisory board to include neurology experts Dr. Susan Perlman and Dr. Hank Paulson**
- **Expanded Board of Directors with the addition of Jeffrey L. Cleland of Orpheris, Bali Muralidhar of Abingworth LLP, Bosun Hau of Tybourne Capital Management, and Tim Walbert of Horizon Therapeutics**
- **Raised approximately \$90.8 million in gross proceeds from the sale of common stock in two public offerings and up-listed to the NASDAQ Global Market**

### Pipeline Updates

#### Neurology

- In December of 2019, Exicure announced the development of XCUR-FXN, an SNA-based therapeutic candidate, for the treatment of Friedreich's ataxia (FA). FA is driven by triplet repeats in the frataxin gene which compromises the patient's ability to generate adequate levels of frataxin protein. Exicure believes its SNA technology has the potential to address this genetic challenge and that its therapeutic strategy may lead to increases in the frataxin protein. Exicure will be developing XCUR-FXN with guidance from, and in collaboration with, the Friedreich's Ataxia Research Alliance. Preclinical research is ongoing and IND-enabling studies for XCUR-FXN are expected in late 2020.
- In 2020, Exicure expects to continue pre-clinical research on the application of its SNA technology in neurological conditions, building on its early proof-of-concept work with nusinersen and its new therapeutic candidate, XCUR-FXN. Exicure is currently exploring additional neurological conditions, including spinocerebellar ataxia, Batten disease, amyotrophic lateral sclerosis (ALS), and Huntington's disease.

#### Immuno-oncology; AST-008

- In December of 2019, Exicure announced preliminary results from the Phase 1b study of AST-008 in patients with solid tumors. AST-008 is an investigational SNA consisting of toll-like receptor 9 (TLR9) agonists designed for immuno-oncology applications, and is being evaluated in combination with pembrolizumab in patients with solid tumors. At that time the study had enrolled fourteen patients including five melanoma patients, four Merkel cell carcinoma (MCC) patients, two cutaneous squamous cell carcinoma patients, two head and neck squamous cell carcinoma patients, and one mucosal melanoma

patient. Prior to enrolling, most patients had progressive disease on anti-PD-1 or anti-PD-L1 antibodies.

- o Available data, as of December 11, 2019, showed that AST-008 administration, alone or in combination with pembrolizumab, produced cytokine and chemokine expression and immune cell activation in patient blood indicative of desired immune activation. Of the 4 MCC patients, one patient, who had previously progressed on anti-PD-1 antibody therapy, had confirmed stable disease with decreased target lesion diameters for a period in excess of twelve weeks, while a second MCC patient experienced a target lesion complete response and a confirmed overall partial response longer than 24 weeks. No treatment-related serious adverse events or dose-limiting toxicities have been observed. The most common reported adverse event was injection site reactions.
- o Exicure is now completing the Phase 1b study and preparing to begin a Phase 2 study in both Merkel cell carcinoma and in cutaneous squamous cell carcinoma. Exicure currently has seven trial sites open and seeks to expand to about fifteen sites.

## **Collaborations**

Exicure entered into a collaboration with Allergan Pharmaceuticals International Limited in late 2019 and is now actively engaged in preclinical research and discovery in two clinical programs related to the treatment of hair loss disorders. Under the terms of the collaboration, Exicure received a \$25 million upfront payment and is eligible to receive up to \$725 million in potential milestones. In early 2019, Exicure also entered into a collaboration agreement with Dermelix Biotherapeutics under which Dermelix will develop a targeted therapy for the treatment of Netherton Syndrome (NS).

## **2019 Financial Results and Financial Guidance**

*Cash Position:* As of December 31, 2019, Exicure had cash and cash equivalents of \$48.5 million and short-term investments of \$62.3 million for a total of \$110.8 million compared to \$26.3 million of cash and cash equivalents and no short-term investments as of December 31, 2018. In 2019, Exicure raised approximately \$90.8 million in gross proceeds from the sale of common stock and received a \$25 million upfront payment in connection with the Collaboration Agreement with Allergan.

*Research and Development Expense:* Research and development expense was \$19.3 million for the year ended December 31, 2019 compared to \$14.1 million for the year ended December 31, 2018. The increase in research and development expense of \$5.2 million was primarily due to higher platform and discovery-related expenses of \$4.7 million, higher employee-related expense of \$0.9 million and higher facilities, depreciation, and other expenses of \$0.2 million, partially offset by a net decrease of \$0.6 million in costs related to our clinical development programs. The increase in platform and discovery-related expenses is mostly due to a license fee of \$3.8 million paid to Northwestern University in connection with the \$25.0 million upfront payment received from Allergan.

*General and Administrative Expense:* General and administrative expense was \$8.6 million for the year ended December 31, 2019 and \$7.8 million for the year ended December 31, 2018, an increase of \$0.8 million. This increase was primarily due to higher compensation and related expenses, recruiting fees in connection with adding two new board members, higher D&O insurance premiums, Nasdaq listing costs and lease costs associated with our Cambridge, MA office. These increased costs were partially offset by reductions in legal and other transaction costs.

*Net Loss:* Net loss was \$26.3 million for the year ended December 31, 2019, compared to net loss of \$22.4 million for the year ended December 31, 2018, an increase in loss of \$3.9 million. Net loss reflects the changes in expenses discussed above and is offset by an increase in revenue of \$1.2 million for the year ended December 31, 2019 compared to revenue for the year ended 2018. Revenue in 2019 was primarily related to the Dermelix Collaboration.

*Cash Runway Guidance:* Exicure believes that, based on its current operating plans and as of the date of this press release, its existing cash and cash equivalents as of December 31, 2019 is sufficient to meet its anticipated cash requirements into early 2022.

## **About Friedreich's Ataxia (FA)**

FA is a rare, degenerative, life-shortening neuro-muscular disorder that affects children and adults, and involves the loss of strength and coordination usually leading to wheelchair use, diminished vision, hearing and speech, scoliosis, increased risk of diabetes, and a life-threatening heart condition. There are no FDA-approved treatments. An estimated 5,000 patients in the US and 15,000 patients worldwide are affected by FA.

## **About FARA**

The Friedreich's Ataxia Research Alliance (FARA) is a 501(c)(3), non-profit, charitable organization dedicated to accelerating research leading to treatments and a cure for Friedreich's ataxia. [www.CureFA.org](http://www.CureFA.org).

## **About Exicure, Inc.**

Exicure, Inc. is a clinical-stage biotechnology company developing therapeutics for neurology, immuno-oncology, inflammatory diseases and other genetic disorders based on our proprietary Spherical Nucleic Acid, or SNA technology. Exicure believes that its proprietary SNA architecture has distinct chemical and biological properties that may provide advantages over other nucleic acid therapeutics and may have therapeutic potential to target diseases not typically addressed with other nucleic acid therapeutics. Exicure is in preclinical development of XCUR-FXN an SNA-based therapeutic candidate, for the treatment of Friedreich's ataxia (FA). Exicure's drug candidate AST-008 is in a Phase 1b/2 trial in patients with advanced solid tumors. Exicure is based outside of Chicago, IL and in Cambridge, MA.

For more information, visit Exicure's website at [www.exicuretx.com](http://www.exicuretx.com).

## **Exicure Forward-Looking Statements**

This press release contains forward-looking statements (including within the meaning of Section 21E of the United States Securities Exchange Act of 1934, as amended, and Section 27A of the United States Securities Act of 1933, as amended) concerning the Company, the Company's technology, potential therapies and other matters. Forward-looking statements generally include statements that are predictive in nature and depend upon or refer

to future events or conditions, and include words such as “may,” “will,” “should,” “would,” “expect,” “plan,” “believe,” “intend,” “look forward,” and other similar expressions among others. Statements that are not historical facts are forward-looking statements. Forward-looking statements are based on current beliefs and assumptions that are subject to risks and uncertainties and are not guarantees of future performance. Actual results could differ materially from those contained in any forward-looking statement as a result of various factors, including, without limitation: unexpected costs, charges or expenses that reduce cash runway; that Exicure’s pre-clinical or clinical programs do not advance or result in approved products on a timely or cost effective basis or at all; the cost, timing and results of clinical trials; that many drug candidates do not become approved drugs on a timely or cost effective basis or at all; the ability to enroll patients in clinical trials; possible safety and efficacy concerns; regulatory developments; and the ability of Exicure to protect its intellectual property rights. Furthermore, data from preclinical studies often fails to be indicative of outcomes in human trials. Risks facing the Company and its programs are set forth in the Company’s filings with the SEC. Except as required by applicable law, the Company undertakes no obligation to revise or update any forward-looking statement (including without limitation its cash runway guidance) or to make any other forward-looking statements, whether as a result of new information, future events or otherwise.

**EXICURE, INC.**  
**UNAUDITED CONSOLIDATED BALANCE SHEETS**  
(in thousands, except share and per share data)

	<b>December 31,</b>	
	<b>2019</b>	<b>2018</b>
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$ 48,460	\$26,268
Short-term investments	62,326	—
Accounts receivable	16	—
Unbilled revenue receivable	19	3
Prepaid expenses and other assets	1,955	1,392
Total current assets	112,776	27,663
Property and equipment, net	2,099	1,061
Other noncurrent assets	388	32
Total assets	<b>\$115,263</b>	<b>\$28,756</b>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Current portion of long-term debt	\$ 4,965	\$ —
Accounts payable	1,814	500
Accrued expenses and other current liabilities	2,435	1,543
Current portion of deferred revenue	21,873	—
Total current liabilities	31,087	2,043
Long-term debt, net	—	4,925
Common stock warrant liability	414	797
Deferred revenue non-current	2,956	—
Other noncurrent liabilities	59	39
Total liabilities	<b>\$ 34,516</b>	<b>\$ 7,804</b>
Stockholders' equity:		
Common stock, \$0.0001 par value per share; 200,000,000 shares authorized, 86,069,263 issued and outstanding, December 31, 2019; 44,358,000 shares issued and outstanding, December 31, 2018	9	4
Additional paid-in capital	162,062	75,942
Accumulated other comprehensive loss	(27)	—
Accumulated deficit	(81,297)	(54,994)
Total stockholders' equity	80,747	20,952
Total liabilities and stockholders' equity	<b>\$115,263</b>	<b>\$28,756</b>

**EXICURE, INC.**  
**UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS**  
(in thousands, except share and per share data)

	<b>Year Ended</b>	
	<b>December 31,</b>	
	<b>2019</b>	<b>2018</b>
Revenue:		
Collaboration revenue	\$ 1,296	\$ 118
Total revenue	1,296	118
Operating expenses:		

Research and development expense	19,340	14,119
General and administrative expense	8,573	7,818
Total operating expenses	<u>27,913</u>	<u>21,937</u>
Operating loss	(26,617)	(21,819)
Other income (expense), net:		
Dividend income	543	323
Interest income	178	4
Interest expense	(786)	(672)
Other income (loss), net	379	(249)
Total other income (loss), net	<u>314</u>	<u>(594)</u>
Net loss	<u>\$ (26,303)</u>	<u>\$ (22,413)</u>

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Source: Exicure, Inc.