Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 1 of 122 PageID #:600

# **UNITED STATES DISTRICT COURT** NORTHERN DISTRICT OF ILLINOIS

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MARK COLWELL, Individually and On Behalf ) of All Others Similarly Situated,

Plaintiff,

v.

EXICURE, INC., DAVID A. GILJOHANN, BRIAN C. BOCK, and GRANT T. CORBETT,

Defendants.

Civil Action No. 1:21-cv-06637 Judge John F. Kness Magistrate Judge Jeffrey T. Gilbert

CLASS ACTION

SECOND AMENDED CLASS FOR ACTION COMPLAINT **VIOLATION OF THE FEDERAL SECURITIES LAWS** 

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Counsel for Lead Plaintiff James Mathew and Lead Counsel for the Class

# TABLE OF CONTENTS

I.	NAT	JRE OF THE ACTION 1		
II.	JURI	DICTION AND VENUE		
III.	PAR	ΓΙΕS		
	A.	Plaintiffs		
	B.	Defendants		
IV.	DEFENDANTS' FRAUDULENT SCHEME TO TOUT FALSE DATA ON XCUR-FXN'S EFFICACY			
	A.	Contrary to FDA Requirements, Exicure Lacked Basic Controls on Data Integrity		
	В.	Despite Internal Warnings, Exicure Pursues XCUR-FXN Based on Unreliable Data		
	C.	Defendant Corbett Falsifies the Results of <i>In Vitro</i> Experiments and, Due to Exicure's Lack of Controls, No One Checks the Validity of His Purported Results		
	D.	Defendants Disseminate the False <i>In Vitro</i> Data to Investors, Starting at Exicure's January 7, 2021 R&D Day		
	E.	Corbett Fabricates <i>In Vivo</i> Data and Defendants Publicly Release It Starting in April 2021		
	F.	Defendants Present the False <i>In Vivo</i> Data at Exicure's July 15, 2021 R&D Day		
	G.	By September 2021, CEO Giljohann Is Aware That Corbett's Data Was False, Yet Continues to Disseminate It		
	H.	The Truth Emerges: Defendants Admit That Over Seven Months of Exicure's SEC Filings and Public Presentations Were False		
	I.	Post-Class Period Events		
V.	DEFI	ENDANTS' FALSE AND MISLEADING STATEMENTS		
	A.	False and Misleading Statements Made Orally and in Investor Presentations 43		
		1. January 7, 2021 R&D Day Presentation		

# Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 3 of 122 PageID #:602

		2.	January 7, 2021, February 2, 2021, and March 15, 2021 Investor Presentations	. 51
		3.	January 11, 2021 H.C. Wainwright BioConnect Conference; March 9, 2021 H.C. Wainwright Global Life Science Conference	. 54
		4.	April 12, 2021 Investor Presentation	. 55
		5.	May 27, 2021 Investor Presentation	. 58
		6.	July 15, 2021 R&D Day Presentation	. 61
		7.	Exicure's July 16, 2021 Investor Presentation	. 65
		8.	August 2, 2021 and August 5, 2021 Investor Presentations	. 67
		9.	September 8, 2021 Investor Presentation	. 70
		10.	September 13, 2021 H.C. Wainwright 23rd Annual Global Investment Conference; September 30, 2021 Benzinga Healthcare Small Cap Conference; October 5, 2021 Chardan Genetic Medicines Conference	. 73
		11.	October 13, 2021 Investor Presentation	. 78
	В.	False a	and Misleading Statements in Exicure's SEC Filings	. 82
	C.	False a	and Misleading Certifications of Effective Controls	. 86
VI.	ADDI	FIONAI	L ALLEGATIONS OF SCIENTER	. 89
	A.	Defen	dant Corbett's Scienter Is Admitted	. 89
	B.	Forme	r Employee Allegations	. 90
	C.	Defendants Had Knowledge and Continuous Access to Reports Regarding the False XCUR-FXN Data90		
	D.	Core (	Operations Supports Scienter	. 98
	E.		icure Burned Cash, Defendants Were Motivated to Lie ure New Funding	. 98
	F.		dants Giljohann and Corbett Spoke Extensively about the XCUR-FXN Data and Analysts Focused on It	100
	G.		re's Grossly Deficient Controls and Defendants' False Certifications They Were "Effective" Support Scienter	101
	H.	Execu	tive Terminations Support Scienter	102

# Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 4 of 122 PageID #:603

	I.	The Company's Self-Serving Statements Do Not Defeat the Strong Inference of Scienter
	J.	Corporate Scienter
VII.	LOSS	CAUSATION
	A.	Exicure First Reveals "Alleged Improprieties" in XCUR-FXN Research and Its Stock Plummets 38.5%
	В.	Exicure States That It Is "Unable to Determine" the False Data's Impact on R&D for XCUR-FXN; the Stock Drops 30%
	C.	Exicure Finally Admits That It Reported False Data from "at Least Three Different Experiments" for Over Seven Months and Abandons XCUR-FXN; the Stock Plunges 46%
VIII.	PRESU	JMPTION OF RELIANCE
IX.	CLASS	S ACTION ALLEGATIONS
X.	INAPP	LICABILITY OF STATUTORY SAFE HARBOR 112
XI.	CLAIN	IS FOR RELIEF
	COUN	IT I
	COUN	IT II
	COUN	IT III
XII.	JURY	DEMAND
XIII.	PRAY	ER FOR RELIEF

## Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 5 of 122 PageID #:604

Lead Plaintiff James Mathew ("Plaintiff") alleges claims under Sections 10(b) and 20(a) of the Securities Exchange Act of 1934 for a class period of January 7, 2021 to December 10, 2021, both inclusive (the "Class Period"). Plaintiff, by and through his undersigned counsel, alleges the following upon personal knowledge as to Plaintiff and Plaintiff's own acts, and upon information and belief as to all other matters based on, among other things, the independent investigation conducted by and through Lead Counsel. This investigation includes, but is not limited to, a review and analysis of public filings by Exicure, Inc. ("Exicure" or "the Company") with the Securities and Exchange Commission ("SEC"); transcripts of Exicure conferences with investors and analysts; press releases and media reports concerning the Company; analyst reports concerning Exicure; and other public information and data regarding the Company; and interviews with former employees of Exicure ("FEs") conducted in the investigation by and through Lead Counsel.<sup>1</sup>

# I. NATURE OF THE ACTION

1. This securities class action arises from Defendants' *admitted false statements* about XCUR-FXN, Exicure's purported breakthrough drug for Friedreich's Ataxia, a rare, fatal neurological disease. Defendants fabricated—that is, made up—the results of multiple experiments on XCUR-FXN in 2020 and 2021 and reported the false data and results to investors.

2. This was securities fraud. At the end of the Class Period, on December 10, 2021, Exicure admitted that "misreported data" on XCUR-FXN's "efficacy" from "at least three different experiments" had been "included in various public presentations and SEC filings from as early as January 7, 2021 through as late as August 12, 2021"—the bulk of the Class Period.

<sup>&</sup>lt;sup>1</sup> Throughout this Second Amended Complaint, emphasis is added unless otherwise noted.

# Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 6 of 122 PageID #:605

Further, Exicure stated that Defendant Grant Corbett, Ph.D., Exicure's former Group Head, Neuroscience, admitted that he had "intentionally misreported" the XCUR-FXN data.

3. By 2019, with a low share price and rapidly diminishing cash, Exicure needed a miracle drug to survive.

4. To boost Exicure's sagging stock price and attract new funding, Defendants embarked on a fraudulent scheme to tout XCUR-FXN based on fabricated and manipulated data.

5. Exicure hired Defendant Corbett in February 2019 and seized on Friedreich's Ataxia ("FA") as its first neurology drug. FA is caused by an inherited genetic mutation that leads to a deficiency in frataxin protein. The frataxin deficiency renders patients unable to walk, confines them to wheelchairs, and ultimately causes their premature death—typically by age 40.

6. There is no approved treatment for FA. Capitalizing on this tragic disease, Exicure sought to create a new drug, XCUR-FXN, to sell at sky-high prices. XCUR-FXN would purportedly increase frataxin levels to mitigate or reverse FA—the functional equivalent of a cure.

7. From inception, however, XCUR-FXN had no effect on frataxin levels, and the XCUR-FXN program was based on fabricated data enabled by Exicure's deficient controls.

8. Defendants' fraudulent scheme was simple. Defendant Corbett created false data showing a large increase in frataxin levels and used it to prepare false graphs with software called Prism. The software allowed Corbett to view the results of his fabrication in real time; Corbett simply fabricated data until the graph showed the desired visual result. Defendant David Giljohann—a Ph.D. scientist who became Exicure's CEO in 2013—approved the false graphs for inclusion in Exicure's SEC filings and dissemination to investors.

9. The scheme started in summer 2020 when Corbett used an unreliable assay to evaluate XCUR-FXN during the screening process. The assay was not capable of reliably

## Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 7 of 122 PageID #:606

determining whether XCUR-FXN had the desired impact on genetic expression of FXN genes, according to Exicure's Research and Development Manager, FE-3. At the time, Exicure's Senior Director of R&D, Bart Anderson, and Corbett were both expressly told by FE-3 that the assay was unreliable and should not be used, but they ignored the warning.

By summer 2020, Corbett still had no viable drug candidate. In summer 2020,
Corbett admitted to FE-3 that he had no viable results: "He said, I don't think we have anything.
I don't think we are seeing anything that is worth (pushing forward). There is nothing there,"
FE-3 said. FE-3 added that Corbett "seemed very stressed" at the time.

11. With no viable results, Defendants resorted to fraud: Corbett manipulated initial screening data to show that XCUR-FXN dramatically increased FXN mRNA levels in cells from FA patients. Shortly thereafter, in a Company-wide meeting, CEO Giljohann praised Corbett and rewarded him with a Lego set for delivering results.

12. Corbett proceeded to fabricate data in three more experiments from autumn 2020 through early 2021:

13. <u>First</u>, Corbett fabricated the results of an experiment on human neurons: He created false graphs purportedly showing that XCUR-FXN raised frataxin levels in the neurons when it had no such effect.

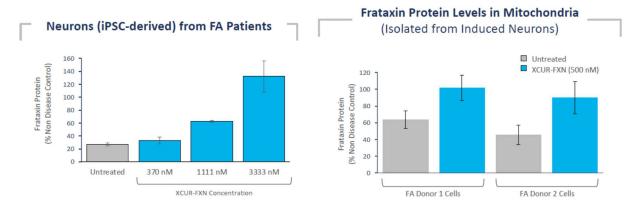
14. <u>Second</u>, Corbett fabricated the results of a similar experiment on human mitochondria. Again, he created false graphs purportedly showing that XCUR-FXN raised frataxin levels in the mitochondria when it did not.

15. <u>Third</u>, Corbett fabricated the results of an *in vivo* experiment that tested XCUR-FXN on living mice. This experiment again showed that XCUR-FXN did not work: it had no

### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 8 of 122 PageID #:607

impact on frataxin levels and killed multiple mice. Instead of reporting this disastrous result, Corbett created a false graph that claimed XCUR-FXN doubled or tripled frataxin levels in mice.

16. Starting on January 7, 2021, Defendants repeatedly touted the false data and results to investors. For example, Exicure's January 7, 2021 investor presentation purported to show that doses of XCUR-FXN dramatically increased frataxin levels in neurons and mitochondria, with the blue bars (treatment with XCUR-FXN) showing frataxin levels significantly higher than the grey bars (untreated neurons and mitochondria):



17. This was an outright falsehood because XCUR-FXN had no impact on frataxin levels, and the graphs above were fabricated by Corbett based on false data.

18. After January 7, 2021, Exicure continued to "misreport the results" of the experiments in its "public presentations and SEC filings," as the Company admitted at the end of the Class Period. Further, Giljohann personally touted the false data and false charts in oral statements to investors. For example, Giljohann falsely claimed that XCUR-FXN was "able to dose dependently and consistently upregulate frataxin protein and frataxin mRNA," and that the drug caused frataxin increases in mice of "two to three-fold above the baseline."

## Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 9 of 122 PageID #:608

19. Defendants' fraud was enabled by the fact that Exicure—in violation of federal law—lacked controls on data integrity and the accuracy of its public disclosures, creating conditions that were ripe for the fabrication and falsification of data and results.

20. In particular, contrary to FDA regulations, Exicure had no "separate" and "independent" person to "assure" that "the reported results accurately reflect the raw data" and that there were "no deviations from approved protocols or standard operating procedures" in its experiments. 21 C.F.R. § 58.35(a)-(b). Three former Exicure scientists (FE-1, 2, and 3) confirmed that from 2019 through 2021, Exicure had no second, independent person to check that its publicly reported results matched the original data. Despite Exicure's failure to implement any (much less effective) controls and its continuing violations of FDA requirements, Defendants Giljohann and Bock (Exicure's CFO) falsely certified that Exicure had "effective" disclosure controls and procedures throughout the Class Period.

21. By September 2021, Giljohann and other senior executives had proof that XCUR-FXN did not work—and, to make matters worse, was toxic. As a former Exicure scientist (FE-1) explained, a third-party laboratory working for Exicure performed a second *in vivo* mice experiment in summer 2021—and "it was very clear that [XCUR-FXN] didn't work." FE-1 further explained that the laboratory used "the same methodology, the same type of animal" as Corbett. But the results directly contradicted Corbett's false data: they showed that XCUR-FXN had no impact on frataxin levels, contrary to Corbett's claim of a dramatic increase. Further, XCUR-FXN was alarmingly toxic. Both cohorts of mice that received XCUR-FXN had deaths—and over half of one cohort died. FE-1 said this "alarming" result was a potential safety issue and "raised flags."

22. Defendant Giljohann was aware of these alarming results at the time: in September 2021, he personally participated in two conference calls with the laboratory and learned

#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 10 of 122 PageID #:609

about the results. Despite these findings, Giljohann recklessly disregarded that Corbett's data purporting to show that XCUR-FXN safely raised frataxin levels in mice—was false.

23. Desperate to maintain the illusion of an effective new drug, Defendants concealed the results of the second mice experiment and continued to tout Corbett's false data:

- On September 30, 2021, Giljohann claimed that XCUR-FXN "works in the animals";
- Exicure's October 13, 2021 investor presentation stated that XCUR-FXN had shown "*In vivo* frataxin upregulation of ~3x in the CNS of mice";

24. Exicure's share price plummeted when the truth was revealed in three partial corrective disclosures. On November 15, 2021, Exicure announced that "a former Company senior researcher" had admitted to "alleged improprieties" as to XCUR-FXN, and that outside counsel was conducting an "investigation." This news caused a precipitous 38.5% stock drop.

25. On November 19, 2021, Exicure signaled that the "alleged improprieties" might affect the "timing of completion" of R&D on XCUR-FXN and Exicure's broader "research and development activities," causing an additional 30% stock drop.

26. Finally, on December 10, 2021, Exicure revealed that over seven months of the Company's SEC filings and public statements contained false data; the false data was from "at least three different experiments" and concerned XCUR-FXN's "efficacy"; and Corbett admitted that he "intentionally misreported" the data. Exicure's stock plunged another 46% on this news.

27. While Exicure has claimed that Corbett had acted "alone," its actions told the real story: on December 10, 2021, when Exicure released the investigation results, Giljohann was immediately removed as CEO and a member of Exicure's Board, and without the false data, XCUR-FXN was worthless and immediately abandoned.

28. The Company is now an empty shell with only 13 employees and no ongoing research.

# **II. JURISDICTION AND VENUE**

29. This Court has jurisdiction over the subject matter of this action pursuant to Section 27 of the Exchange Act of 1934 (15 U.S.C. § 78aa). In addition, because this is a civil action arising under the laws of the United States, this Court has jurisdiction pursuant to 28 U.S.C. § 1331.

30. Venue is proper in this District pursuant to Section 27 of the Exchange Act (15 U.S.C. § 78aa). In addition, venue is proper pursuant to 28 U.S.C. § 1391(b) because the acts and transactions giving rise to the violations of law complained of occurred in part in this District, including the falsification of XCUR-FXN data in this District, the preparation of false and misleading statements in this District, and the dissemination of such statements into this District.

31. In connection with the acts alleged in this complaint, Defendants, directly or indirectly, used the means and instrumentalities of interstate commerce, including, but not limited to, the mails, interstate telephone communications, and the facilities of the national securities markets.

# **III. PARTIES**

# A. Plaintiffs

32. Plaintiff James Mathew purchased or otherwise acquired Exicure common stock at artificially inflated prices during the Class Period, as set forth in the Certification previously filed with the Court (ECF 21-5), and suffered damages as a result of Defendants' violations of the federal securities laws alleged herein.

# B. Defendants

33. Defendant Exicure is a Delaware corporation with its corporate headquarters in Chicago, Illinois. Exicure's common stock trades on NASDAQ under the ticker symbol "XCUR."

### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 12 of 122 PageID #:611

34. Defendant David Giljohann, Ph.D., is a co-founder of Exicure and was the Company's CEO from November 2013 to December 10, 2021, and had previously served as Exicure's founding scientist, principal scientist, and Chief Operating Officer. He was also a member of Exicure's Board from March 2014 to December 10, 2021. Defendant Giljohann signed certain Exicure SEC filings that contained false and misleading statements, and made false and misleading statements on conference calls with investors and analysts, as alleged specifically herein. Defendant Giljohann had the power and authority to, and in fact did, approve and control the contents of the Company's SEC filings and investor presentations alleged herein to be false and misleading. In addition, Defendant Giljohann participated in a scheme to create and disseminate false data and results to investors through the Company's SEC filings and investor presentations, as alleged herein, and—regardless of whether he directly made the resulting misstatements—Defendant Giljohann is personally liable for that scheme under Section 10(b) of the Exchange Act and Rule 10b-5(a) and (c) thereunder.

35. Defendant Brian Bock was Exicure's CFO from May 13, 2021 to February 4, 2022. During his tenure, Defendant Bock signed certain Exicure SEC filings that contained false and misleading statements, as alleged specifically herein, and had the power and authority to, and in fact did, approve and control the contents of the Company's SEC filings alleged herein to be false and misleading.

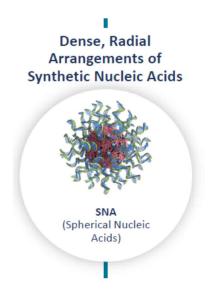
36. Defendant Grant T. Corbett, Ph.D., was Exicure's Group Head, Neuroscience, from February 2019 to November 2021. Defendant Corbett made false and misleading statements on conference calls with investors and analysts, as alleged specifically herein. In addition, Defendant Corbett participated in a scheme to create and disseminate false data and results to investors through the Company's SEC filings and investor presentations, as alleged herein, and—regardless

Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 13 of 122 PageID #:612

of whether he directly made the resulting misstatements—Defendant Corbett is personally liable for that scheme under Section 10(b) of the Exchange Act and Rule 10b-5(a) and (c) thereunder.

# IV. DEFENDANTS' FRAUDULENT SCHEME TO TOUT FALSE DATA ON XCUR-FXN'S EFFICACY

37. Exicure claimed to use proprietary spherical nucleic acid ("SNA") technology to develop new drugs. SNAs are oligonucleotides (short pieces of DNA or RNA) arranged around the outside of a spherical nanoparticle, as shown in Exicure's illustration below:



38. Exicure claimed that its SNAs could affect the genetic drivers of disease at the cellular level by delivering nucleic acid therapeutics into patients' cells more effectively than other companies' products. However, Exicure has yet to deliver a successful clinical drug.

39. Defendant Giljohann, a Ph.D. scientist, was one of Exicure's first scientists and joined the Company immediately after graduate school. In a speech on October 4, 2018, Giljohann touted the "credibility that I bring as a scientist to the leadership role" and declared that "we're a science-based company" and "the science starts at the top."<sup>2</sup>

<sup>&</sup>lt;sup>2</sup> https://www.youtube.com/watch?v=US\_H10OrD5s

### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 14 of 122 PageID #:613

40. In reality, as detailed below, Defendants embarked on a fraudulent scheme to boost Exicure's sagging stock price by touting XCUR-FXN based on fabricated and manipulated data.

41. The starting point for the scheme was Exicure's lack of basic controls on data integrity. Contrary to FDA requirements, Exicure had no controls governing raw data or the accurate reporting of results. In particular, there was no "second body" to check Corbett's data, and no one checked that Exicure's public disclosures matched the original raw data. This violated the FDA's requirement that an independent "quality assurance unit" at Exicure "assure . . . that the reported results accurately reflect the raw data." 21 C.F.R. § 58.35(b)(6). The absence of this required control allowed Corbett to manipulate and fabricate data to create false data and graphs that were included in Exicure's SEC filings and investor presentations for nearly a year.

42. To effectuate the scheme, Corbett manipulated data and fabricated the results of four experiments on XCUR-FXN. Corbett created the false data and prepared false graphs using Prism software.<sup>3</sup> Giljohann approved the false graphs, which were disseminated to investors through Exicure's SEC filings, investor presentations publicly posted on Exicure's website, and oral statements. These material misstatements artificially distorted the market price of Exicure stock until the truth emerged in piecemeal corrective disclosures at the end of the Class Period.

43. The four experiments are summarized below. Exicure has admitted that the results of the latter three were misreported, stating that "Dr. Corbett misreported the results of at least three different experiments that were conducted through at least February 2021"; Corbett also manipulated the screening experiment in 2020.

<sup>&</sup>lt;sup>3</sup> The company that makes Prism describes it as the "preferred analysis and graphing solution purpose-built for scientific research." https://www.graphpad.com/features

Experiment	Actual Result	Claimed Result
r · · ·		
Screening candidate SNAs on	No effect on FXN	"2-3x increase in FXN mRNA
fibroblast cells from FA patient	mRNA	levels" (Jan. 7, 2021)
( <i>in vitro</i> , 2020)		
Testing XCUR-FXN at	No effect on frataxin	Frataxin protein exceeds 100% of
increasing doses on neurons and	protein or FXN	non-disease control; FXN mRNA
fibroblasts from FA patients (in	mRNA levels	reaches up to 70% of non-disease
vitro, 2020-21)		control (Jan. 7, 2021)
Testing XCUR-FXN on	No effect on frataxin	"XCUR-FXN returns frataxin levels
mitochondria from FA patients	protein or	to non-diseased level in
( <i>in vitro</i> , 2020-21)	mitochondrial	mitochondria" and "increases
	activity	mitochondrial energy metabolism
		(SDH activity), indicating improved
		mitochondrial health" (Jan. 7, 2021)
Testing XCUR-FXN on	No effect on frataxin	"~3x frataxin upregulation achieved
Pook800J mice ( <i>in vivo</i> , 2020-21)	protein levels;	in CNS of FA mouse model"; no
	multiple deaths	deaths reported (April 12, 2021)

# A. Contrary to FDA Requirements, Exicure Lacked Basic Controls on Data Integrity

44. Federal regulations require drug companies like Exicure to establish and maintain stringent controls on laboratory studies and scientific data. In particular, the FDA's Good Laboratory Practice for Nonclinical Laboratory Studies (21 C.F.R. Part 58) applies to "nonclinical laboratory studies that support or are intended to support applications for research or marketing permits for products regulated by the Food and Drug Administration." 21 C.F.R. § 58.1(a).<sup>4</sup>

45. These FDA regulations required Exicure to designate a "study director," who "shall assure that" (among other things) "[a]ll experimental data, including observations of unanticipated responses of the test system are accurately recorded and verified," "[a]ll applicable good laboratory practice regulations are followed," and "[a]ll raw data, documentation, protocols, specimens, and final reports are transferred to the archives during or at the close of the study." 21 C.F.R. § 58.33.

<sup>&</sup>lt;sup>4</sup> Exicure was subject to these FDA requirements because it conducted studies that "support[ed] or [were] intended to support" an investigational new drug (IND) application for XCUR-FXN. *Id.* INDs are specifically included within the regulation. 21 C.F.R. § 58(e) (an "[a]pplication for research or marketing permit includes . . . [a]n investigational new drug application.").

# Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 16 of 122 PageID #:615

46. Further, the regulations expressly required Exicure to provide a second, independent person to assure that reported results accurately reflect raw data. Specifically, Exicure was required to provide a "quality assurance unit which shall be responsible for monitoring each study to assure management that the facilities, equipment, personnel, methods, practices, records, and controls are in conformance with the regulations in this part." 21 C.F.R. § 58.35(a); *see also* 21 C.F.R. § 58.31(a) ("management" must "assure that there is a quality assurance unit as described in § 58.35"). Pursuant to 21 C.F.R. § 58.35(b), this "quality assurance unit" was responsible for assuring the integrity of the study and assuring that the reported results accurately reflect the raw data. Specifically, 21 C.F.R. § 58.35(b) provides:

The quality assurance unit shall:

(1) Maintain a copy of a master schedule sheet of all nonclinical laboratory studies conducted at the testing facility indexed by test article and containing the test system, nature of study, date study was initiated, current status of each study, identity of the sponsor, and name of the study director.

(2) Maintain copies of all protocols pertaining to all nonclinical laboratory studies for which the unit is responsible.

(3) Inspect each nonclinical laboratory study at intervals adequate to assure the *integrity of the study* and maintain written and properly signed records of each periodic inspection showing the date of the inspection, the study inspected, the phase or segment of the study inspected, the person performing the inspection, findings and problems, action recommended and taken to resolve existing problems, and any scheduled date for reinspection. Any problems found during the course of an inspection which are likely to affect study integrity shall be brought to the attention of the study director and management immediately.

(4) *Periodically submit to management and the study director written status reports on each study*, noting any problems and the corrective actions taken.

(5) *Determine that no deviations from approved protocols or standard operating procedures were made* without proper authorization and documentation.

(6) Review the final study report to assure that such report accurately describes the methods and standard operating procedures, and that the reported results accurately reflect the raw data of the nonclinical laboratory study.

# Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 17 of 122 PageID #:616

(7) Prepare and sign a statement to be included with the final study report which shall specify the dates inspections were made and findings reported to management and to the study director.

47. Thus, the quality assurance unit was required to "[d]etermine" that there were "no deviations from approved protocols or standard operating procedures"; required "*to assure*" that the "*reported results accurately reflect the raw data*"; and required to bring "[a]ny problems . . . which are likely to affect study integrity . . . to the attention of the study director and management immediately." *Id.* Crucially, the "quality assurance unit shall be entirely separate from and independent of the personnel engaged in the direction and conduct of that study." 21 C.F.R. § 58.35(a).

48. In addition, Exicure was required to "have standard operating procedures in writing setting forth nonclinical laboratory study methods that management is satisfied are adequate to insure the quality and integrity of the data generated in the course of a study," 21 C.F.R. § 58.81(a), and the "final report" on each study was required to include a "description of all circumstances that may have affected the quality or integrity of the data." 21 C.F.R. § 58.185(a)(9).

49. That Exicure used a contract research organization (CRO), Charles River, to conduct certain experiments (discussed below) did not excuse Exicure from the FDA data management and integrity requirements. As Exicure's 2020 Form 10-K, filed on March 11, 2021, explained:

The FDA requires preclinical studies to be conducted in accordance with applicable GLPs, and clinical trials to be conducted in accordance with applicable FDA regulations and GCPs, including *requirements for conducting, recording and reporting the results of preclinical studies and clinical trials to assure that data and reported results are credible and accurate* and that the rights, integrity and confidentiality of clinical trial participants are protected. *Our reliance on third parties that we do not control does not relieve us of these responsibilities and requirements.* 

#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 18 of 122 PageID #:617

50. Contrary to FDA requirements, Exicure had *no* controls governing raw data or the accurate reporting of results: no one checked that Exicure's public disclosures matched the actual data from experiments or "that the reported results accurately reflect the raw data." 21 C.F.R. § 58.35(b)(6). This allowed Corbett to fabricate and falsify data from multiple experiments on XCUR-FXN.

51. Three former Exicure scientists confirmed the absence of any controls—much less effective ones—over the data from Corbett's experiments.

52. FE-2, who joined Exicure in 2019, and from April 2020 to December 2021 was Research Associate II on the Assay Development Team, spoke to the lack of controls from 2019 through late 2021. "There wasn't anybody whose job it was to check data across different files and make sure they were correct," FE-2 said. "Thinking back now, it definitely seems like there should have been something in place." FE-2 stated that there was no second set of eyes checking for accuracy on any data uploaded to the Google Drive (discussed below); no one at Exicure checked to see if the data on protein concentrations that FE-2 uploaded to the Google Drive matched what the plate reader said. Nor, according to FE-2, did anyone check the data entered into Exicure's Prism graphing software (also discussed below) against the Excel spreadsheet data or the plate reader data to ensure it all matched.

53. Similarly, FE-1, an Exicure Project Manager from February 2020 to October 2022, confirmed that Exicure had no "second body" in place to check Corbett's data, which FE-1 described as "unusual" in the pharmaceutical industry. Based on working at other drug companies, FE-1 explained: "Usually, they have that second person who was quality control for data." In particular, FE-1 pointed to a key deficiency in Exicure's processes and QC checks: not having someone check whether Corbett's data matched the original data from Charles River.

54. FE-3 worked at Exicure from September 2016 to December 2021; from June 2018 to December 2021, FE-3 was Research and Development Manager, reporting to Bart Anderson, Senior Director of R&D. FE-3 similarly recalled that there was no one at Exicure who checked Corbett's reports, presentations, and claims for accuracy against the raw data.

# B. Despite Internal Warnings, Exicure Pursues XCUR-FXN Based on Unreliable Data

55. By 2019, Exicure began to focus on developing treatments for rare neurological diseases. Corbett joined Exicure in February 2019 as Group Head, Neuroscience.

56. On July 31, 2019, Exicure stock (which had previously traded over the counter) started trading on NASDAQ.

57. Exicure quickly set its sights on FA—a rare, fatal neurological condition caused by an inherited mutation in a specific gene called FXN, which causes reduced levels of frataxin. The lower frataxin levels cause loss of coordination (ataxia) in patients' arms and legs, hearing and vision loss, and heart disease, among other health issues. FA symptoms typically appear when patients are between age 10 and 15; patients become wheelchair-bound within two decades and typically die by age 40.

58. There is no approved treatment for FA. Thus, a safe, effective FA drug would be FA patients' only treatment option, allowing Exicure to dominate the market and charge high prices for its new drug. Exicure alluded to this profit motive in a January 7, 2021 investor presentation that touted the "[s]ubstantial commercial opportunity" of this fatal disease.

59. Exicure's FA drug candidate was called XCUR-FXN. Exicure claimed that XCUR-FXN would increase FXN mRNA (messenger RNA, involved in protein synthesis), and thereby increase the production of frataxin protein in mitochondria (the organelle inside cells that

## Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 20 of 122 PageID #:619

generates chemical energy). The higher frataxin levels, in turn, would mitigate or slow the progression of FA.

60. On December 16, 2019, Exicure announced its first neurological development program in FA; in its press release, Giljohann claimed that "[o]ver the past year," Exicure had "developed extensive preclinical data supporting the development of our SNAs for neurological disorders."

61. Analysts responded positively: a December 16, 2019 report by an H.C. Wainwright analyst noted that Exicure was "[m]oving aggressively into rare neurological disorders," having "announced Friedreich's ataxia (FA) as the therapeutic indication for the company's first neurology development program," and expected "rapid progress towards an IND filing with the FA-targeted SNA program and believe that this could potentially enter the clinic within 12 - 18months." A December 16, 2019 report by a Guggenheim investment analyst similarly stated: "We view this move into the key neuro disease space as positive, as we believe SNAs carry key advantages (like durability) over a more traditional linear nucleic acid approach."

62. On May 18, 2020, Exicure hired Dr. Douglas Feltner as Chief Medical Officer, with Giljohann stating that Feltner would focus on "building a growing pipeline of neurological drug candidates."

63. On August 12, 2020, Exicure issued a press release stating that "We remain on track to initiate IND-enabling studies for Friedreich's ataxia in the fourth quarter of this year." An IND, or investigational new drug application, allows drug companies to begin human clinical trials.

64. Internally, however, the XCUR-FXN program had not generated any viable drug candidate by summer 2020.

#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 21 of 122 PageID #:620

65. FE-3 was a part of Exicure's early R&D effort that focused on finding sequences of oligonucleotides that could be used to impact genetic expression in a way that could potentially treat FA. This screening process requires reliable assays that reveal if an oligonucleotide sequence is having an impact on genetic expression.

66. FE-3 worked on finding and qualifying assays that could be used in the screening process to identify oligonucleotide sequences to treat FA. FE-3 explained that before an assay was used in the screening process, the lab had to run the assay through quality control tests to make sure it could accurately identify an oligonucleotide's impact on genetic expression of FXN genes.

67. FE-3 tested numerous assays using a QuantStudio 12k machine and cells from a patient with FA. However, when FE-3 tested multiple assays to screen for oligonucleotides that upregulate the targeted gene expression, none could be qualified as reliable.

68. Despite these results, Corbett falsely claimed that an unreliable assay qualified for use. At a weekly meeting of the R&D team in summer 2020, Corbett presented information claiming that one of the upregulation assays had been qualified for use in screening. The assay Corbett presented as qualified was an assay that failed FE-3's quality control tests. When FE-3 ran this particular assay through the quality control tests, it could not reliably determine that upregulation was occurring. "[Corbett] showed that the assay was qualified to use to screen," FE-3 said. "I didn't qualify that assay." But Corbett presented "it in a way that made it seem like it was working, but it wasn't."

69. FE-3 told Anderson and Corbett that the assay was unreliable and should not be used to evaluate XCUR-FXN, but they ignored the warning. Specifically, after hearing Corbett claim the assay was qualified during the weekly meeting, FE-3 went to Anderson and told him that

### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 22 of 122 PageID #:621

the assay was not qualified and could not be used in the screening process. Anderson dismissed FE-3's concern and told FE-3 to raise the issue directly with Corbett.

70. FE-3 then emailed Corbett stating that the assay he claimed was qualified had not been qualified to screen for oligonucleotides that caused upregulation. "I told Grant [Corbett] in the email he could not use the assay because it was not qualified," FE-3 said. Corbett met with FE-3 and dismissed FE-3's concerns, stating that he was analyzing the quality testing based on a published article about the assay. The meeting gave FE-3 the impression that Corbett was going to use the assay for screening for oligonucleotides that caused upregulation.

71. Under FDA regulations, using an unreliable, unqualified assay to evaluate XCUR-FXN was a "problem[]... likely to affect study integrity" that was required to be identified by the independent quality assurance unit and brought "to the attention of the study director and management immediately." 21 C.F.R. § 58.35(b)(3). However, Exicure lacked a control to ensure that this happened—and, in any event, both Anderson and Corbett dismissed FE-3's concerns.

72. Corbett failed to identify a viable candidate for the XCUR-FXN program. Shortly after Exicure moved into its new Chicago headquarters (in July 2020), Corbett told FE-3 that he did not have an oligonucleotide sequence to move forward into further study. "He said, *I don't think we have anything. I don't think we are seeing anything* that is worth [pushing forward]. There is nothing there," FE-3 said. "He said he had to repeat [an] experiment." FE-3 added that Corbett "seemed very stressed" at the time of this conversation.

73. Soon thereafter, Corbett manipulated the results from a screening experiment on XCUR-FXN to claim that XCUR-FXN dramatically increased FXN mRNA levels in cells from FA patients. In reality, it had no effect.

## Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 23 of 122 PageID #:622

74. For his fabricated results, Corbett was rewarded with a Lego set from CEO Giljohann for high-level job performance, as FE-3 learned from a Company-wide announcement during a weekly "town hall" meeting on Monday morning. FE-3 explained that Giljohann had "an encouragement type of thing where he would give Legos to people who performed well." FE-3 believed that Corbett received his Lego set because he had selected a sequence that moved forward into further testing in the XCUR-FXN program.

# C. Defendant Corbett Falsifies the Results of *In Vitro* Experiments and, Due to Exicure's Lack of Controls, No One Checks the Validity of His Purported Results

75. Based on the false data from the screening experiment, Exicure's senior management greenlighted XCUR-FXN for further experiments and animal studies.

76. From autumn 2020 through early 2021, Corbett conducted further experiments on XCUR-FXN and falsified data in at least two *in vitro* experiments (*i.e.*, experiments conducted outside a living organism):

- Testing XCUR-FXN on human neurons and fibroblasts from FA patients
- Testing XCUR-FXN on mitochondria from induced neurons from FA patients

77. Both experiments showed XCUR-FXN had no effect on frataxin or FXN mRNA levels. However, Corbett used Prism software to fabricate false data until the graphs showed the desired visual result. Corbett's fabrication and falsification were highly material: the reality was that XCUR-FXN had no effect on frataxin or FXN mRNA levels (*i.e.*, did not work), but Corbett simply made up false data and results to create the false appearance of a substantial effect.

78. Corbett's fabrication of objectively false data and graphs was not the product of any honest error, interpretation, or opinion, much less a legitimate scientific methodology. Indeed, Corbett's actions violated federal regulations that expressly bar "research misconduct," which

## Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 24 of 122 PageID #:623

includes "*fabrication*" and "*falsification*" in "reporting research results," such as "*making up data or results*," "*changing or omitting data or results such that the research is not accurately represented*," and "manipulating research materials, equipment, or processes," as distinct from "honest error or differences of opinion." 42 C.F.R. § 93.103. Exicure had received federal government grants in the past, and its executives and scientists knew that fabricating and falsely reporting data violated federal law.<sup>5</sup>

# D. Defendants Disseminate the False *In Vitro* Data to Investors, Starting at Exicure's January 7, 2021 R&D Day

79. By the end of 2020, investors and analysts were keenly focused on the new FA drug and whether it worked, and Defendants were ready to take the false XCUR-FXN data public.

80. On December 28, 2020, Exicure announced "a virtual R&D Day on Thursday, January 7th, 2021" to "discuss Exicure's neuroscience pipeline, including its lead program for Friedreich's Ataxia which has progressed into IND-enabling studies."

81. The Class Period begins on January 7, 2021, when Exicure conducted its R&D Day. Demonstrating Defendant Corbett's central role in the fraudulent scheme, he presented to investors alongside Defendant Giljohann and Anderson.

82. In opening remarks, Giljohann stated that "Exicure is building a rapid-progressing program in Friedreich's Ataxia" and hailed XCUR-FXN as the Company's "flagship program" in the "neurology space." Giljohann concluded: "I'm going to now turn it over to Grant Corbett, who's going to talk about our differentiation in neuroscience for our spherical nucleic acid platform. Grant?"

<sup>&</sup>lt;sup>5</sup> Indeed, CEO Giljohann was listed as the principal investigator/project leader on Exicure's prior government-funded projects. *See* https://reporter.nih.gov/search/Xqnt50llIUWAGj-XueWs9A/projects

# Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 25 of 122 PageID #:624

83. Corbett then presented. The presentation identified Corbett as the speaker on "SNA Differentiation for Neuroscience":

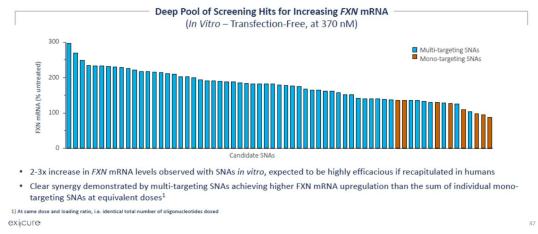
# SNA Differentiation for Neuroscience Grant Corbett, PhD – Head, Neuroscience Research

84. Corbett touted the purportedly superior ability of Exicure's SNAs to reach the brain and central nervous system, particularly in regions important to treating FA. For example, Corbett stated: "we've observed prominent uptake of SNAs in the cortex, thalamus, brain stem cerebellum, dorsal root ganglia of the spinal cord, *regions differentially critical for Friedreich's Ataxia*," among other conditions. Corbett also presented data that he claimed were "suggestive of SNA's ability to reach deep brain neurons, a pharmacokinetic aspect *critical to address deficits observed in Friedreich's Ataxia*." In addition, Corbett claimed that "we observed broad and persistent distribution of SNAs in the rat brain," with "evidence of nuclear uptake [that] is *consistent with the observed pharmacological effects of SNAs, such as frataxin upregulation*."

85. Next, Anderson presented the false data from Corbett's XCUR-FXN experiments using false slides created by Defendant Corbett and approved by Defendant Giljohann. Anderson knew at the time that Corbett was using an unreliable, unqualified assay—because FE-3 had told him that—but presented the data anyway.

86. Specifically, Anderson showed the following slide, claiming that "Exicure's SNAs induced high FXN mRNA upregulation" and that "in human fibroblast cells from a Friedreich's Ataxia patient," "[w]e saw the treatment with candidate SNAs resulted in a *two to threefold increase in FXN mRNA*, which would be expected to be highly efficacious in humans." The increasingly large blue bars on the left side purported to show that candidates in the XCUR-FXN program yielded a "2-3x increase in FXN mRNA levels" in vitro:

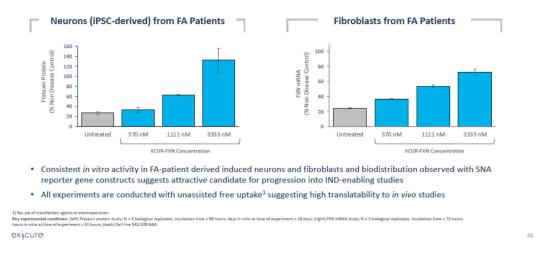




87. This was false: XCUR-FXN did not raise FXN mRNA levels, and Corbett had used an unreliable, unqualified assay and fabricated the results of the experiment.

88. Next, Anderson displayed the following slide and stated that in human neurons and fibroblasts from FA patients, XCUR-FXN "upregulates frataxin protein as well as the frataxin mRNA." Anderson claimed that the slide "illustrates the *dose-dependent increase in FXN mRNA* that is induced" as well as "a *dose-dependent increase in frataxin protein*, which reaches the same frataxin level that we measured in neurons from the nondisease control donor." The stair steps in the slide (the blue bars) falsely indicated that XCUR-FXN had a dramatic effect compared to untreated neurons and fibroblasts (the grey bars), and that higher doses purportedly led to increasingly higher levels of frataxin protein and FXN mRNA:





89. Similarly, Anderson declared that XCUR-FXN increased frataxin levels in

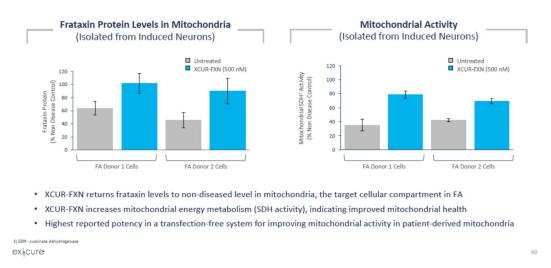
mitochondria, stating:

The left graph shows the level of frataxin protein, specifically in the mitochondria of induced neurons from 2 different FA patient donors normalized to the level in a non-disease controlled donor. The gray bars are untreated neurons and the blue bars are the frataxin level after treatment with the SNA.

As expected, without treatment, there is low frataxin protein in the mitochondria of both donors. But *after treatment with the SNA, the mitochondrial frataxin level is increased and is similar to the level found in the non-disease control [neurons]*. Taking this one step further, in the same neurons, we asked how well the mitochondria are working by assessing SDH activity as a measure for energy metabolism.

As shown in the right graph, *SNA treatment substantively increased mitochondrial activity in neurons from both FA donors. These data show that the SNA without transfection, potently improves mitochondrial activity in patientderived neurons.* We were thrilled to see these preclinical results that connect the anticipated mechanism of action to increase mRNA, protein, localized protein and ultimately to the cellular function that is needed to correct the molecular deficit that *drives* Friedreich's Ataxia.

90. The accompanying slide showed these purported results:



# XCUR-FXN Increases Mitochondrial Frataxtin and Activity

91. Again, these results were false. XCUR-FXN had no effect on frataxin or FXN mRNA levels, but Corbett fabricated the data and used it to prepare graphs showing false results. He was able to do so because, contrary to FDA requirements, no one checked that Exicure's public disclosures matched the actual data from experiments or "that the reported results accurately reflect the raw data." 21 C.F.R. § 58.35(b)(6).

92. At the end of the call, analysts—unaware that the data was fabricated—peppered Giljohann, Corbett, and the other presenters with technical questions. Corbett responded in detail, demonstrating his central role in the fraudulent scheme.

93. For example, a Guggenheim analyst asked: "[B]ecause your approach will still require getting protein into mitochondria, do you have confidence that you can get sufficient penetration there?" In response, Corbett falsely claimed that Exicure's experiments had shown XCUR-FXN to increase frataxin levels in mitochondria: "Yes. *So in our preclinical, our* in vitro *experiments, we've shown up-regulation of mitochondria frataxin.*"

94. Unaware of the truth, analysts praised Exicure's false results. On January 7, 2021, a report by a Guggenheim analyst stated that "SNA tech is well-suited for CNS diseases as they

### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 29 of 122 PageID #:628

can effectively penetrate neurons with an extended half-life," tracking Corbett's false statements during the presentation, stated that "**XCUR preclinical studies observed a 2-3x increase in FXN mRNA levels in vitro**" (emphasis in original), and reported portions of Exicure's false slides. Similarly, a January 7, 2021 report by a BMO analyst stated: "We believe initial XCUR-FXN preclinical data support increased frataxin production, pointing to benefit in Friedreich's Ataxia (FRDA)," and "We believe the in-vitro data confirms the SNA approach for FRDA, with Spinraza clinically validating ASOs in CNS diseases." The latter referred specifically to Corbett's presentation of how SNAs purportedly achieved broad and persistent distribution in the CNS, including in regions critical for Friedreich's Ataxia.

95. At the January 11, 2021 H.C. Wainwright BioConnect Conference, CEO Giljohann falsely stated: "*What we've seen, and we just released this week as well, is that we are able to dose dependently and consistently upregulate frataxin protein and frataxin mRNA* in both monospecific and, I think more excitingly, [bispecific] spherical nucleic acids as well."

96. In the wake of these false statements, Exicure's share price climbed, reaching its Class Period peak of \$2.80 on February 10, 2021.

97. On March 9, 2021, at the H.C. Wainwright Global Life Science Conference, Giljohann personally presented the false data on XCUR-FXN's purported efficacy: "Again, on slide 24, *we've been able to show that our drugs work, both increasing the amount of frataxin protein and messenger RNA* in cell lines that are derived from Friedreich's Ataxia patients. We are rapidly moving into clinical validation, with an IND expected before the end of this year in Friedreich's Ataxia, and expect first in-patient in early 2022."

98. Exicure's 2020 Form 10-K, filed on March 11, 2021 and signed by Defendant Giljohann, reiterated the false data yet again, declaring that "*XCUR-FXN has shown potent, dose-*

# *dependent upregulation of frataxin protein*" and that "*XCUR-FXN increased frataxin protein levels*... *to near normal levels*" in mitochondria, fibroblasts and neurons.

99. Upon the filing of the 2020 Form 10-K, Exicure's stock surged 28.9% in a single day, reaching \$2.50 on March 11, 2021. Analysts praised the results as heralding not only an effective treatment for FA, but also a lucrative pipeline of drugs for other inherited diseases. On March 11, 2021, a report by a BMO analyst stated: "We expect XCUR-FXN to provide platform validation in Friedreich's ataxia, opening up opportunity in other hereditary disorders."

# E. Corbett Fabricates *In Vivo* Data and Defendants Publicly Release It Starting in April 2021

100. In early 2021, Defendants' scheme reached the next stage: an *in vivo* experiment to test XCUR-FXN's efficacy on living mice. The *in vivo* mice experiment was conducted by a CRO, Charles River (FE-1, FE-2). Corbett was Charles River's point of contact at Exicure and supervised Charles River in running the XCUR-FXN experiments (FE-2).

101. The *in vivo* experiment used so-called "Pook800J" mice, a specific type of laboratory mouse created to harbor the genetic mutation that causes FA. Specifically, Pook800J mice are genetically altered to remove the mouse FXN gene and replace it with the human disease allele with approximately 800 repeats of the GAA (guanine-adenine-adenine) bases of the DNA sequence, the genetic mutation that causes FA.

102. FE-2 described the *in vivo* experiment in detail. The Pook800J mice were divided into a control group and several treatment groups injected with increasingly high doses of XCUR-FXN. After dosing, Charles River "sacrificed" all of the mice and dissected them, taking slices of tissue from different parts of each mouse's brain and dorsal root ganglia—specifically, the brainstem, cerebellum, cortex, and midbrain regions of the brain, and the cervical, thoracic,

### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 31 of 122 PageID #:630

and lumbar regions of the dorsal root ganglia (FE-2).<sup>6</sup> Charles River then provided the tissue samples to Exicure for measurement of their protein content—specifically, whether the mice that received XCUR-FXN had higher frataxin levels in their brain and nervous system than the control group (FE-2).

103. FE-2 performed the protein measurement on samples extracted from the mouse tissue. To do so, FE-2 used assays to measure (1) the total concentration of all proteins and (2) the concentration of frataxin protein. The assay tests used chemical reactions that identified protein concentrations by color; the more intense the color, the higher the protein concentration. A machine called a spectrophotometer, or plate reader, measured the intensity of the colors in each sample to determine the protein concentration amount. FE-2 recalled that there were at least 400 samples, which took FE-2 two to three weeks to analyze. FE-2 performed this work in early 2021.

104. The data from the mouse tissue was transmitted in three steps. <u>First</u>, the plate reader created and maintained a file with the results on its own system. <u>Second</u>, FE-2 used a flash drive to download a copy of the file from the plate reader and put the file on FE-2's computer. <u>Third</u>, FE-2 sent the results of the assay tests FE-2 ran on the mouse tissue to Corbett as an Excel file using Google Drive. FE-2 indicated that the Google Drive was shared by Exicure employees, including Corbett, and the data files on the Google Drive were accessible to most Exicure employees, including senior executives such as Giljohann, Matthias Schroff (Exicure's Chief Operating Officer), Feltner (Chief Medical Officer), Weston Daniel, Ph.D. (Exicure's VP Translational Research), and Anderson. FE-2 noted that the Google Drive indicated who last

<sup>&</sup>lt;sup>6</sup> Dorsal root ganglia are clusters of neurons that connect to the spinal cord at various locations along its length.

## Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 32 of 122 PageID #:631

modified a document (and when), and that Excel files include metadata that shows the history of alterations to the spreadsheet.

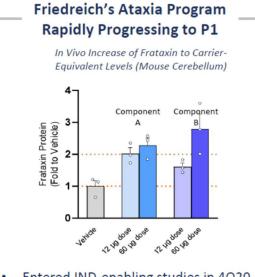
105. Corbett used the protein concentration data to calculate whether XCUR-FXN was increasing frataxin protein levels. Specifically, Corbett calculated the percentage of frataxin compared to total proteins in each sample, then divided that figure by the frataxin percentage of the control group (called the "vehicle"). The result expressed the sample's frataxin concentration as "fold to vehicle"—for example, 2x "fold to vehicle" meant there was twice the frataxin concentration in the sample compared to the control. FE-2 recognized Exicure's publicly reported graph showing that XCUR-FXN induced a 2-3x "fold to vehicle" frataxin increase in mice as using the assay tests FE-2 conducted.

106. The *in vivo* mice experiment showed no change in frataxin levels—but revealing that to investors, when Defendants had previously reported positive *in vitro* data, would immediately deflate Exicure's share price. Thus, Corbett fabricated data indicating that XCUR-FXN raised frataxin levels in mice. FE-2 suspected that Corbett manipulated the data in the Prism software program to alter the graphs generated. FE-2 explained that Corbett used Prism to generate presentation-ready graphs showing the results of experiments; when data is entered or modified in Prism, the graph updates in real time, making it easier to manipulate data to achieve a desired visual result.

107. By April 2021, the *in vivo* mice experiment was complete (FE-1). The purported *in vivo* results were then released to investors in Exicure's April 12, 2021 investor presentation, which was publicly disseminated through Exicure's website. This first, crucial presentation of *in vivo* results falsely claimed that XCUR-FXN had tripled frataxin levels in mice: "~**3x frataxin upregulation achieved in CNS of FA mouse model**" (emphasis in original).

# Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 33 of 122 PageID #:632

108. Slide 4 presented a graph based on Corbett's false data, where the blue and purple stair steps purported to show that XCUR-FXN raised frataxin levels in mice:



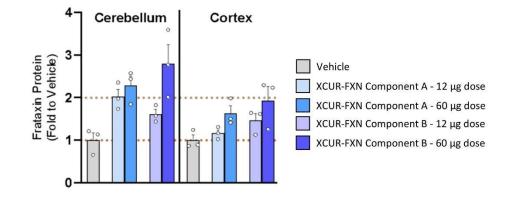
- Entered IND-enabling studies in 4Q20
- IND filing anticipated by end of 2021
- Potential for early target engagement read-out in FIH via CSF frataxin levels

109. Whether XCUR-FXN raised frataxin levels—and if so, by how much—was highly material. Prior research had shown that FA patients had frataxin levels between 25% and 60% of frataxin levels in healthy individuals, and Exicure explained on January 7, 2021 that "Frataxin Levels Are Closely Tied to Disease Outcomes in FA." The same presentation also stated that "*even small increases* in frataxin levels could drive meaningfully milder" symptoms of FA. Thus, the statements that XCUR-FXN purportedly *doubled or tripled* frataxin levels in mice represented an outstanding result and signaled a highly effective treatment for FA.

110. Exicure's May 12, 2021 Form 10-Q for the first quarter of 2021 repeated these false claims, stating that "[a]s illustrated in the figure below, *the components of XCUR-FXN increased frataxin protein levels by 2-3 fold* in the cerebellum and the cortex, the two regions we believe are

## Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 34 of 122 PageID #:633

critical for addressing neurological manifestations of FA. We also found similar increases in FXN levels in the brainstem, the midbrain and throughout the spinal cord."

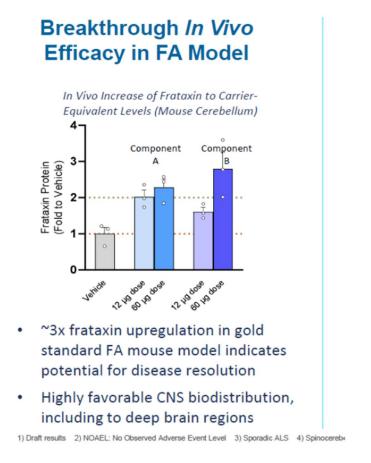


111. Analysts praised these promising *in vivo* results. A May 13, 2021 report by a Chardan analyst stated: "Preclinical data in a Pook800J mouse model demonstrated that 14 days post intra-cisterna magna injection of SNAs consisting of the two individual oligonucleotide components of XCUR-FXN, *frataxin protein levels were increased by 2-fold and greater* in the cerebellum and the cortex regions of the brain and spinal cord."

# F. Defendants Present the False *In Vivo* Data at Exicure's July 15, 2021 R&D Day

112. On July 8, 2021, Exicure announced its July 15, 2021 Virtual R&D Day, stating: "The event will showcase Exicure's neuroscience pipeline, including its lead program for Friedreich's Ataxia (FA), XCUR-FXN, which is designed to address the underlying molecular cause of FA. Exicure will present new and previously unreleased preclinical data and discuss progress with XCUR-FXN, which is on track for IND filing in late Q4 2021."

113. At the July 15, 2021 R&D Day presentation, Defendant Giljohann presented a false slide created by Defendant Corbett, excerpted below, purporting to show XCUR-FXN's "Breakthrough *In Vivo* Efficacy":



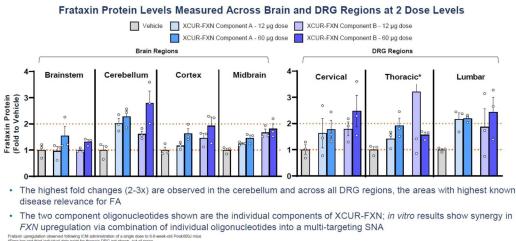
114. Based on this false slide, Giljohann declared that Exicure had achieved "what we believe is *breakthrough efficacy in an* in vivo *Friedreich's Ataxia model, where we've been able to show, throughout the mouse, [sic] cerebellum important markers like frataxin upregulated.*" The "breakthrough efficacy" was purely the product of Corbett's fabrication and falsification; in reality, XCUR-FXN had no effect on frataxin levels.

115. Weston Daniel, Ph.D., Exicure's VP Translational Research, further presented the false data created by Defendant Corbett. Daniel stated: "Exicure has, to our knowledge, generated the most promising *in vivo* results in the gold-standard Pook800J mouse, showing *3x frataxin upregulation in the cerebellum.*" He explained that Exicure had "very exciting results with XCUR-FXN components in the gold-standard mouse model, called the Pook800J mouse, where

we're seeing a tripling of frataxin protein levels in key tissues." Daniel displayed the following

false slide created by Defendant Corbett:

# XCUR-FXN Components Upregulate Frataxin Protein by 2-3x *In Vivo,* Largest Reported Effect Size in This "Gold-Standard" Pook800J Mouse

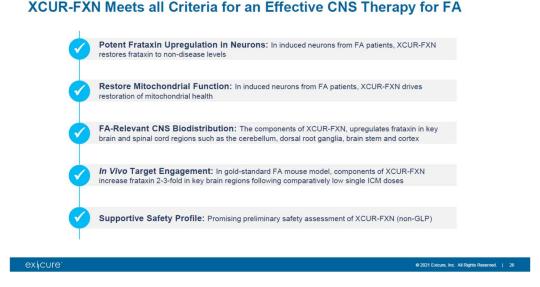


116. The blue and purple bars purported to show that XCUR-FXN caused a doubling and tripling of frataxin levels relative to the control group (in grey). Again, this was simply false.

117. Daniel explained: "[W]e injected this ICV into these Pook800J mice, and then we dissected the animals and looked for frataxin changes as a function of treatment. *And I'm extremely excited to report that in key—in key FA-driving disease regions, like the cerebellum, second from left on this plot, as well as in dorsal root ganglia regions of the spinal cord, which is the right-hand side of the plot, we're seeing a two to three-fold upregulation of frataxin protein.* Now this is significant because, recall, in the blood level discussion at the outset of my section, I described how we believe that doubling frataxin levels could slow disease progression, and tripling of frataxin levels could perhaps slow or even stop disease progression. And *what we're seeing here with the components of XCUR-FXN, in this gold-standard mouse model, is that two to three-fold upregulation of frataxin protein. So, very significant effects in our view.*"

#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 37 of 122 PageID #:636

118. Daniel concluded with a slide declaring that "XCUR-FXN Meets all Criteria for an Effective CNS Therapy for FA."



119. Daniel summarized: "What were the five things we set out to do? Well, I believe we've done all of them. Those goals we set out were, again, to have *potent upregulation of frataxin neurons, which would restore mitochondrial function; we observed both of those things*. We wanted to see *FA-relevant biodistribution and activity in animals; we saw both of those those things*. And we wanted to see some supportive preliminary safety data, which I just showed you. So the combination of these five things makes us ... very excited about XCUR-FXN. These observations make us excited to take this drug toward patients in the early part of next year."

120. Analysts praised the presentation. A July 15, 2021 report by a BMO analyst stated: **"Neuro Update Shows XCUR-FXN Promise, Helps Validate Further Pipeline Potential**" (emphasis in original); "In vivo Pook800J gold-standard mouse model showed strong target engagement with 2-3x frataxin upregulation in the cerebellum/DRG regions, which XCUR believes is considered highly therapeutic if recapitulated in humans and would bring FA patients close/back to phenotypically normal frataxin carrier levels." A July 16, 2021 report by a Chardan

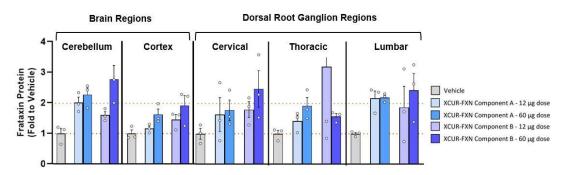
#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 38 of 122 PageID #:637

analyst stated: "New preclinical data from the Pook800J mouse model was presented. This data demonstrated the ability of the two components to be used in XCUR-FXN, upregulated frataxin protein by 2-3x . . . . Notably these increases were observed in the cerebellum and across all dorsal root ganglia regions, the areas with highest known disease relevance for FA."

121. Exicure's Form 10-Q for the second quarter of 2021, filed on August 12, 2021,

repeated the false data and false chart, stating:

As illustrated in the figure below, *the components of XCUR-FXN increased frataxin protein levels by 2-3 fold in the cerebellum, cortex, and dorsal root ganglia neurons*; these regions we believe are critical for addressing neurological manifestations of FA. We also found similar increases in frataxin protein levels in the brainstem, the midbrain and throughout the spinal cord.



# G. By September 2021, CEO Giljohann Is Aware That Corbett's Data Was False, Yet Continues to Disseminate It

122. FE-1 explained that in summer 2021, Exicure advanced XCUR-FXN into a larger, longer-term *in vivo* mice study of efficacy as well as safety. Crucially, unlike the first *in vivo* mice experiment, the results of this second mice experiment were reported directly to FE-1's team—not to Corbett.

123. This second mice study was conducted by Charles River and used three cohorts of Pook800J mice, or approximately 30 to 45 animals; two cohorts were given XCUR-FXN, while the third cohort was given a placebo (FE-1).

#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 39 of 122 PageID #:638

124. The results of the experiment were immediately alarming—and directly contrary to Corbett's false data and Defendants' prior public statements. In September 2021, after one week of the experiment, Charles River reported results to FE-1's team; the first report showed that several mice had already died and others were sick (FE-1). These data did not match Corbett's results, despite using mice of the same sex and age as Corbett (FE-1).

125. By mid-September 2021, FE-1 learned of two specific discrepancies between Corbett's results and the data from Charles River.

126. <u>First</u>, while Corbett's data purportedly showed that XCUR-FXN worked, the September 2021 Charles River experiments made clear that it had *no effect*. As FE-1 explained, *"it was very clear that [XCUR-FXN] didn't work*." Specifically, Corbett's results had shown high "stair steps," with the mice responding to increasing doses of XCUR-FXN, while their response to the placebo was almost flat. By contrast, Charles River's results for XCUR-FXN showed almost a flat line with the placebo. Despite the exact same testing parameters, Charles River's experiments were not showing the positive results that Corbett had reported. FE-1 explained: "They did them under the same methodology, the same type of animal. It just didn't add up. It just didn't make sense to me." Charles River also tried raising the dose of XCUR-FXN to see if they could get the same results as Corbett. Again, however, the drug wasn't working.

127. <u>Second</u>, during Charles River's experiments, both cohorts of mice that received XCUR-FXN had deaths—and *over half of one cohort died*. FE-1 noted that this "alarming" result was a potential safety issue and "raised flags."

128. In short, without Corbett's manipulation and fabrication, the results Charles River reported in September 2021 showed that XCUR-FXN did not work and had alarming toxicity.

#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 40 of 122 PageID #:639

129. These alarming results were quickly reported to Exicure's senior management. Specifically, in mid-September 2021, Defendant Giljohann, COO Schroff, Chief Medical Officer Feltner, and Anderson (as well as Scott Mix and FE-1) participated in a conference call with Charles River to discuss the poor results from the experiment's first week. During the call, Giljohann and Anderson said the Company needed to investigate XCUR-FXN's sterility before continuing the study.

130. Despite Giljohann knowing about Charles River's troubling results by September 2021, Defendants did nothing to correct their prior misstatements. Further, they continued to repeat the false data and results, while giving investors no hint of the material, undisclosed fact that the second mice study showed the opposite of Corbett's doctored results.

131. At the September 13, 2021 H.C. Wainwright 23rd Annual Global Investment Conference, Giljohann claimed that XCUR-FXN worked both *in vitro* and *in vivo*. Giljohann stated that "we've shown that different components of our spherical nucleic acid are able to reach up into the brain and increase in this case frataxin protein by targeting the frataxin gene." He added: "we're able to get into Friedreich's Ataxia patient cells *in vitro* and show a dose dependent upregulation of the relevant frataxin proteins. When we take this into an animal model [*in vivo*], as well, *you can see that we're getting a really nice increase. The highest fold changes are two to three-fold above the baseline* and it's across both the cerebellum and the DRG. Again, those critical regions for FA."

132. After the mid-September 2021 call, Charles River briefly stopped dosing the remaining mice with XCUR-FXN while Exicure's in-house team ran diagnostics and checked the drug's sterility. This took one week and showed that XCUR-FXN was sterile. After XCUR-FXN's sterility was confirmed, Charles River continued the mice experiment for another

#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 41 of 122 PageID #:640

week, through the end of September 2021. The results were no better, and even larger numbers of mice died than before. At the end of September 2021, Giljohann, Schroff, Feltner, and Anderson (with Mix, FE-1 and others) participated in another call with Charles River where these results were discussed and Charles River was directed to stop the study.

133. Thus, by the end of September 2021, there was no doubt that XCUR-FXN did not work and showed alarming toxicity in mice: Charles River had twice reported these disastrous results and Exicure had stopped the *in vivo* study. At the same time, Exicure started to face "substantial doubt" about its "ability to continue as a going concern." As Exicure later admitted (in its Form 10-Q filed on November 19, 2021), based on its "current operating plans and existing working capital at September 30, 2021, it is uncertain whether our current liquidity is sufficient to fund operations over the next twelve months . . . . As a result, there is substantial doubt about our ability to continue as a going concern."

134. Exicure's increasingly tenuous financial position heightened Defendants' incentive to bury the truth about XCUR-FXN. As the November 19, 2021 Form 10-Q explained, "We have no committed sources of additional capital at this time and substantial additional financing will be needed by us to fund our operations." While "[m]anagement" purportedly "believe[d]" that Exicure would "be able to obtain additional funding through equity or debt financings, collaboration agreements, strategic partnerships and licensing arrangements," to reveal that Exicure's flagship drug was worthless and premised on false data would doom those efforts.

135. Thus, Defendants continued to tout the same false XCUR-FXN data to attract new funding, even as CEO Giljohann was aware that XCUR-FXN had no effect on frataxin levels and had killed a number of mice. At the September 30, 2021 Benzinga Healthcare Small Cap Conference, Giljohann confidently proclaimed that XCUR-FXN "works in the animals":

You can see here in the blue bars that we're getting a nice increase in the frataxin level after dosing. It also works in the animals. So we've shown that as well—if you look at different levels across the regions of the brain, we're seeing high delivery of the appropriate regions of the brain, we're seeing upregulation of frataxin protein. So from our perspective, all the components are here. We're getting into cells, we're getting into tissues, we're getting into the right areas of the brain, and then we're able to have an effect.

136. Five days later, at the October 5, 2021 Chardan Genetic Medicines Conference, Giljohann stated that the "data . . . showed that after intrathecal injection, we are able to get deep into the brain and into the brain regions that we need to target that FXN gene," that "we wrapped up our tox study in rats with really no significant findings there," and that in terms of "sets of compelling preclinical data, the most on track in terms of showing that we are ready to go to the clinic would be those lines that we are drawing from the in vitro results to the in-vivo distribution to that penetration into the right tissues." Giljohann declared: "[W]e are past that hype stage where there is a great new technology. *We have already shown that the hype works* in our spherical nucleic acid [technology]."

137. Despite the clear evidence that XCUR-FXN did *not* work—from the program's inception using an unreliable assay (as reported to Anderson and Corbett at the time), through Corbett's fabrication of false data in the *in vitro* and *in vivo* experiments, and culminating with the September 2021 reports to Giljohann that XCUR-FXN had no effect on frataxin levels and killed a number of mice—Defendants again reported the false data to investors. On October 13, 2021, Defendants publicly disseminated an investor presentation through Exicure's website that reiterated the false data from the *in vivo* mice experiment and falsely claimed that XCUR-FXN had shown "*In vivo* frataxin upregulation of  $\sim$ 3x in the CNS of mice following intrathecal single dose administration of the individual components of XCUR-FXN at comparatively low doses (60 µg)."

#### H. The Truth Emerges: Defendants Admit That Over Seven Months of Exicure's SEC Filings and Public Presentations Were False

138. On November 8, 2021, Corbett purportedly resigned. As Exicure later admitted, "[a]s part of his resignation, [Corbett] claimed that when he was employed by the Company, he intentionally misreported certain raw data related to the research and development of XCUR FXN."

139. But the falsity of the XCUR-FXN data was not new information that Exicure suddenly discovered upon Corbett's departure. Instead, as detailed above, Exicure's longstanding control failures created conditions that were ripe for the fabrication and falsification of data and results; the XCUR-FXN program was based on an unreliable assay, as reported to Anderson and Corbett before the Class Period; and Giljohann had known since at least September 2021 of results that were starkly contrary to Corbett's data and Defendants' prior public statements. After months of internal knowledge that Corbett had fabricated XCUR-FXN data, both Corbett and Giljohann were expelled from the Company, and their scheme collapsed.

140. On November 15, 2021, Exicure filed a Form 12b-25 stating that Exicure would miss the filing deadline for its Form 10-Q for the third quarter of 2021 and revealing that: "On November 9, 2021, the Audit Committee of the Board of Directors of the Company was notified of a claim made by a former Company senior researcher regarding alleged improprieties that researcher claims to have committed with respect to the Company's XCUR-FXN preclinical program for the treatment of Friedreich's ataxia. The Audit Committee has retained external counsel to conduct an internal investigation of the claim." Following this disclosure, the price of Exicure stock plummeted 38.5% between November 16 and 18, 2021.

141. On November 19, 2021, Exicure filed its Form 10-Q for the third quarter of 2021, which included a new disclosure that Exicure was "unable to determine the potential impact" of

#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 44 of 122 PageID #:643

the "alleged improprieties" on its "research and development activities or the timing of completion of our current research and development of our XCUR-FXN preclinical program for the treatment of FA." Following this disclosure, Exicure's stock dropped 30.09% on November 19, 2021.

142. The Class Period ends on Friday, December 10, 2021, when Exicure filed a Form 8-K reporting the results of the internal investigation.

143. The December 10, 2021 Form 8-K revealed that the former "senior researcher" referenced in prior disclosures was "Dr. Grant Corbett, the Company's former Group Leader of Neuroscience," and that Defendant Corbett had "claimed that when he was employed by the Company, he intentionally misreported certain raw data related to the research and development of XCUR FXN."

144. In addition, Exicure admitted that false data created by Corbett had been included in the Company's "SEC filings," "public presentations," and other "public statements" by "Company management" for over seven months.

145. Specifically, the December 10, 2021 Form 8-K stated that "beginning in the autumn of 2020, Dr. Corbett misreported raw data from certain research and development experiments related to XCUR-FXN," "Dr. Corbett misreported the results of at least three different experiments that were conducted through at least February 2021," and the "misreported data" related to XCUR-FXN's "efficacy."

146. Crucially, the Form 8-K also admitted that the "misreported data was included in various public presentations and SEC filings from as early as January 7, 2021 through as late as August 12, 2021" (a range that is understated by two months, as detailed above), and that "Company management" had made "public statements that included Dr. Corbett's misreported data." These admissions demonstrate that Defendants' prior statements were false when made.

#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 45 of 122 PageID #:644

147. Relatedly, the December 10, 2021 Form 8-K announced the purging of Exicure's senior management. Effective immediately, Defendant Giljohann was out as CEO and off the Board, with a brief transition period as "Chief Technology Officer" through January 31, 2022. Defendant Bock replaced Giljohann as CEO. The Form 8-K also announced that Chief Medical Officer Feltner was departing on January 30, 2022.

148. Further, the Form 8-K announced that Exicure was abandoning XCUR-FXN, stating: "On December 10, 2021, the Company announced its commitment to a plan to wind down ... the Company's XCUR-FXN preclinical program for the treatment of Friedreich's ataxia." The Form 8-K also stated that "the Company will eliminate approximately 50% of the Company's existing workforce on a staggered basis through January 2022."

149. On this news, Exicure's stock plunged 40.68% on December 13, 2021, and another 6.28% on December 14, 2021. In total, between November 15, 2021 and December 14, 2021, Exicure's stock dropped from \$1.07 to \$0.25, losing over 76% of its value in less than a month.

#### I. Post-Class Period Events

150. Without XCUR-FXN, Exicure is now an empty shell with no products and no ongoing R&D.

151. In February 2022, Exicure's new CEO, Defendant Bock, departed after less than two months—and four days after qualifying for a \$180,000 "retention award." On February 4, 2022, Exicure filed an 8-K stating that Defendant Bock was out as CEO and off the Board, replaced by Schroff as CEO.<sup>7</sup>

<sup>&</sup>lt;sup>7</sup> Exicure's December 10, 2021 Form 8-K stated: "Subject to Mr. Bock's continued employment through January 31, 2022, the Company will pay Mr. Bock a one-time retention award of \$180,000, subject to applicable tax withholdings, as soon as practicable and no later than five business days after January 31, 2022." Bock qualified for the \$180,000 payment on Monday, January 31, 2022, and resigned that Friday, February 4, 2022.

#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 46 of 122 PageID #:645

152. On September 26, 2022, Exicure announced that it would "suspend all pre-clinical activities," was "halting all R&D activities," and would reduce its remaining workforce by 66%.

153. Exicure now has only a skeleton crew of 13 remaining employees (as of December 31, 2022). Its Form 10-K for 2022 (filed on March 27, 2023) warned that "there is substantial doubt about our ability to continue as a going concern," and that "available funds could be sufficient into early in the fourth quarter of 2023, [but] we could spend our available financial resources much faster than we currently expect." Exicure also stated on March 27, 2023 that it "has received numerous deficiency notes with respect to various Nasdaq listing requirements" and faces delisting from NASDAQ unless it regains compliance with these requirements.

154. Most recently, on May 15, 2023, Exicure failed to timely file its Form 10-Q for the first quarter of 2023 "because of recent turnover within the Company's accounting and financial reporting department" and the May 8, 2023 resignation of its auditor, KPMG LLP.

#### V. DEFENDANTS' FALSE AND MISLEADING STATEMENTS

155. Defendants' statements that are alleged to be false and misleading are identified in the sections below. For the avoidance of doubt, the only statements that Plaintiff alleges to be actionable are included in this section.

156. The alleged false and misleading statements described below are attributable to the Individual Defendants under Rule 10b-5(b) because Defendants Giljohann and Bock each signed certain of Exicure's SEC filings described below, Defendants Giljohann and Corbett each made false and misleading statements on conference calls, and as Exicure's CEO, Defendant Giljohann approved Exicure's investor presentations and is responsible for their content.

157. Separately, under Rule 10b-5(a) and (c), Defendants Corbett and Giljohann are primarily liable for all of the challenged statements because they participated in a scheme to defraud by engaging in conduct that had the principal purpose and effect of creating a false

#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 47 of 122 PageID #:646

appearance of fact. These Defendants created false data and graphs concerning XCUR-FXN and prepared, approved, and/or disseminated them with intent to defraud.

158. Corbett personally falsified the XCUR-FXN data and used the Prism software to created false graphs based on his fabricated data—inherently deceptive acts, with no legitimate business purpose, in furtherance of the scheme.

159. Corbett knew that his false data and false graphs were being disseminated to investors. Indeed, Corbett had direct contact with investment analysts during Exicure's January 7, 2021 R&D Day, where he spoke publicly and delivered multiple slides; Anderson presented false graphs on XCUR-FXN's purported efficacy that Corbett prepared; and Corbett witnessed Anderson's presentation and personally answered analysts' questions about XCUR-FXN.

160. As CEO, Defendant Giljohann approved the false data and false graphs that Defendant Corbett created, which were included in Exicure's SEC filings and investor presentations, as detailed below. Thus, these Defendants' fraudulent scheme resulted in multiple misrepresentations to the market.

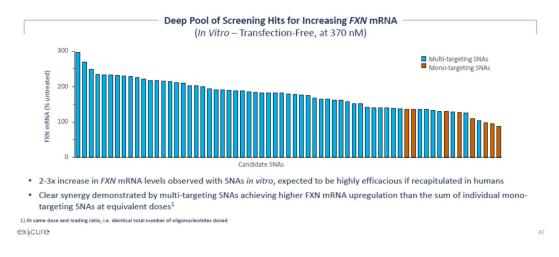
#### A. False and Misleading Statements Made Orally and in Investor Presentations

161. This category of misstatements includes misstatements made orally and in investor presentations publicly posted to Exicure's website.

#### 1. January 7, 2021 R&D Day Presentation

162. Exicure's January 7, 2021 R&D Day Presentation included the following slide stating that candidate SNAs in the XCUR-FXN program yielded a "2-3x increase in FXN mRNA levels" *in vitro*.

#### Exicure SNAs Demonstrate Highest Reported FXN mRNA Upregulation In System Without Transfection Agents



163. Based on Corbett's false data and graph, Anderson stated: "Here, I'm showing the

level of FXN mRNA in human fibroblast cells from a Friedreich's Ataxia patient. We saw the

#### treatment with candidate SNAs resulted in a two to threefold increase in FXN mRNA ....."

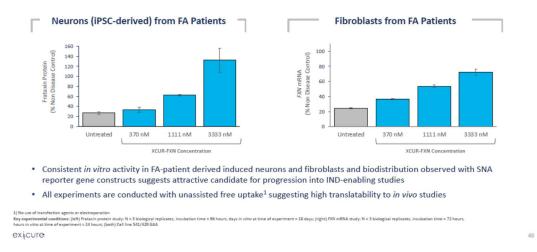
164. Next, based on Corbett's false data and graphs, Anderson stated:

Taking a closer look at our XCUR-FXN, I'm showing here that *Exicure's SNA upregulates frataxin protein as well as the frataxin mRNA*. The right graph shows FXN mRNA in FA patient fibroblast, similar to the experiment on the previous slide, but here, normalized the level of FXN in fibroblast from a nondisease control donor. And *this illustrates the dose-dependent increase in FXN mRNA that is induced by SNA treatment*. As shown in the left graph, we also assess the level of frataxin protein, now looking in induced neurons from the same FA patient donor. Similar to the FXN mRNA data, *we see a dose-dependent increase in frataxin protein, which reaches the same frataxin level that we measured in neurons from the nondisease control donor*.

Note that these experiments are conducted without transfection reagent, just as they would be in vivo. The *high activity observed here in vitro*, combined with biodistribution of reported SNAs that we showed earlier, made XCUR-FXN an attractive candidate to progress into IND-enabling studies.

165. The accompanying slide showed XCUR-FXN's purportedly dramatic effect:





166. Anderson stated that after treatment with XCUR-FXN, "the mitochondrial

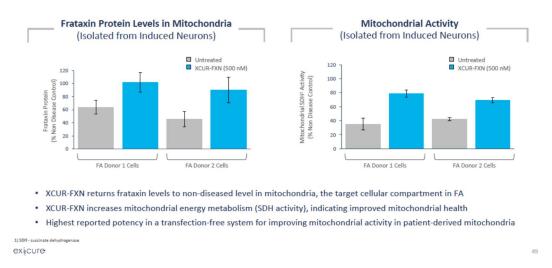
frataxin level is increased and is similar to the level found in the non-disease control [neurons],"

and that XCUR-FXN "potently improves mitochondrial activity in patient-derived neurons":

As expected, without treatment, there is low frataxin protein in the mitochondria of both donors. But *after treatment with the SNA, the mitochondrial frataxin level is increased and is similar to the level found in the non-disease control [neurons]*. Taking this one step further, in the same neurons, we asked how well the mitochondria are working by assessing SDH activity as a measure for energy metabolism.

As shown in the right graph, SNA treatment substantively increased mitochondrial activity in neurons from both FA donors. These data show that the SNA without transfection, potently improves mitochondrial activity in patientderived neurons.

167. The accompanying slide showed these purported results:



#### XCUR-FXN Increases Mitochondrial Frataxtin and Activity

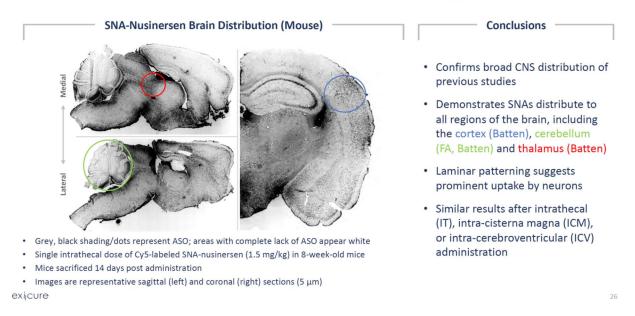
168. The statements in Paragraphs 162-167 were false and misleading when made because the reported data were false, and XCUR-FXN had not yielded a "2-3x increase in FXN mRNA levels" *in vitro* or "induced high FXN mRNA upregulation"; had not caused a "dose-dependent increase in FXN mRNA" or "a dose-dependent increase in frataxin protein, which reaches the same frataxin level that we measured in neurons from the nondisease control donor"; and had not increased the "mitochondrial frataxin level [to be] similar to the level found in the non-disease control [neurons]" or "potently improve[d] mitochondrial activity in patient-derived neurons"; nor had Exicure "observed" that XCUR-FXN had "high activity . . . in vitro." Instead, Corbett's experiments had shown that XCUR-FXN did not work: the XCUR-FXN program was based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and Corbett had falsified the results of his experiments on fibroblasts, neurons, and mitochondria. As Exicure itself has admitted, Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021."

#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 51 of 122 PageID #:650

169. During the same presentation on January 7, 2021, Corbett stated: "we've observed *prominent uptake of SNAs* in the cortex, thalamus, brain stem cerebellum, dorsal root ganglia of the spinal cord, *regions differentially critical for Friedreich's Ataxia*," among other conditions.

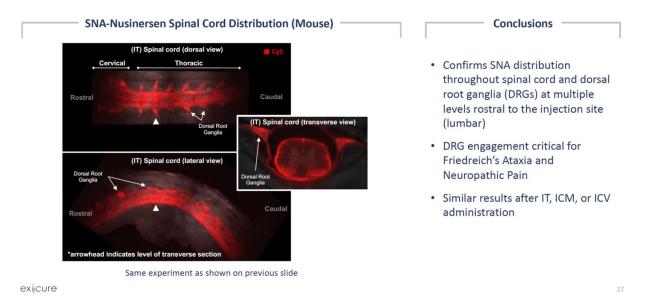
170. Corbett further claimed that SNAs reached brain regions that "are strongly implicated in the indications we're targeting," with the accompanying slide illustrating the point and purportedly showing that SNAs reached the cerebellum, a brain region linked to FA:

## SNAs Reach Areas of the Brain Critical in Neurodegeneration



171. Similarly, Corbett claimed that there was "widespread SNA distribution throughout all levels of the cord and, critical for Friedreich's Ataxia and pain, near complete engagement of the dorsal root ganglia." The accompanying slide explained that "DRG engagement [is] critical for Friedreich's Ataxia."

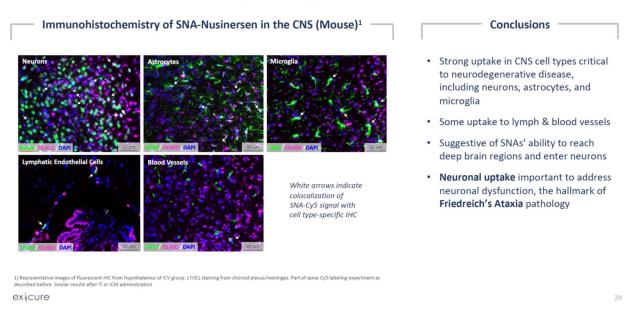
## SNAs Reach Areas of the Spinal Cord Critical in Neurodegeneration



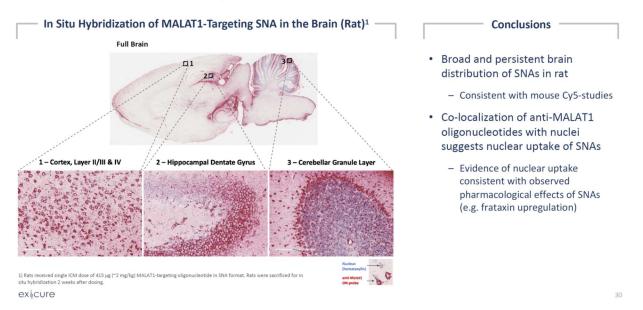
172. Corbett further claimed that in monkeys, SNAs reached "regions critically but differentially implicated in Friedreich's Ataxia" (among other conditions), with "very strong signals" supporting "broad and persistent distribution throughout the nonhuman primate CNS and that SNAs are capable of reaching a number of regions implicated in diseases of the nervous system."

173. At the cellular level, Corbett presented data that he claimed were "suggestive of SNA's ability to reach deep brain neurons, a pharmacokinetic aspect critical to address deficits observed in Friedreich's Ataxia."

## SNAs Distribute Across Key Cell Types in the CNS



174. Going further, Corbett touted "SNA distribution at a subcellular level," stating that "we observed broad and persistent distribution of SNAs in the rat brain," including "localization of SNAs to the cytoplasm and nucleus of neurons in the cortex, hippocampal dentate gyrus and in granular layer of the cerebellum. This evidence of nuclear uptake is *consistent with the observed pharmacological effects of SNAs, such as frataxin upregulation*." The accompanying slide illustrated this purported result.



## SNAs Distribute Broadly in the Brain and Show Nuclear Uptake

175. The statements in Paragraphs 169-174 were false and misleading when made because Exicure had not seen "prominent uptake of SNAs" in brain and spinal cord "regions differentially critical for Friedreich's Ataxia," much less "observed" any "frataxin upregulation." Further, these positive statements misleadingly omitted the material fact that Corbett's experiments had shown that XCUR-FXN did not work: the XCUR-FXN program was based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and Corbett had falsified the results of his experiments on fibroblasts, neurons, and mitochondria. As Exicure itself has admitted, Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021."

176. In response to an analyst question about whether XCUR-FXN was raising frataxin levels in mitochondria, Corbett stated:

# Yes. So in our preclinical, our in vitro experiments, we've shown up-regulation of mitochondria frataxin.

#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 55 of 122 PageID #:654

177. The statements in Paragraph 176 were false and misleading when made because the reported data were false, and Exicure had not "shown up-regulation of mitochondria frataxin," as Corbett claimed. Instead, Corbett's experiments had shown that XCUR-FXN did not work: the XCUR-FXN program was based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and Corbett had falsified the results of his experiments on mitochondria. As Exicure itself has admitted, Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021."

#### 2. January 7, 2021, February 2, 2021, and March 15, 2021 Investor Presentations

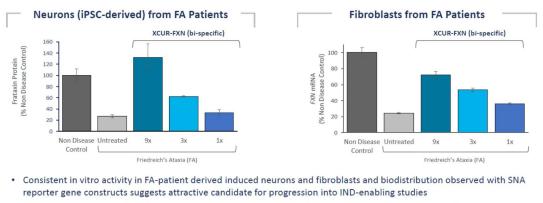
178. Exicure's investor presentations dated January 7, 2021, February 2, 2021, and March 15, 2021 were publicly disseminated through Exicure's website, and each presentation contained the following false and misleading statements:

179. Slide 12 claimed that XCUR-FXN was "Increasing Frataxin Levels in CSF":



180. Slide 24 displayed data purporting to show that XCUR-FXN significantly raised frataxin protein and FXN mRNA levels in neurons and fibroblasts from FA patients:

# XCUR-FXN Dose-Dependently Upregulates Frataxin Protein and FXN mRNA



All experiments are conducted with unassisted free uptake, without the use of transfection agents or electroporation, suggesting high translatability to in vivo studies

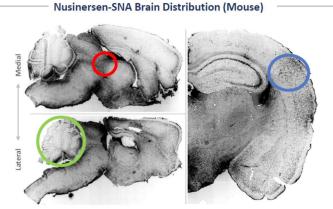
exicure | 24

181. The statements in Paragraphs 179-180 were false and misleading when made because the reported data were false, and in truth, XCUR-FXN did not affect frataxin protein or FXN mRNA levels. Corbett's experiments had shown that XCUR-FXN did not work: the XCUR-FXN program was based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and Corbett had falsified the results of his experiments on fibroblasts, neurons, and mitochondria. As Exicure itself has admitted, Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021."

182. Slides 18 and 19 claimed that Exicure's SNAs were reaching areas of the brain and neurons critical to neurodegeneration and "Friedreich's Ataxia pathology":

Key experimental conditions: (left) Frataxin protein study: N = 3 biological replicates, incubation time = 96 hours, days in vitro at time of experiment = 18 days; (right) FXN mRNA study: N = 3 biological replicates, incubation time = 72 hours, hours in vitro at time of experiment = 24 hours; (both) Cell line 541/420 GAA

# SNAs Reach Areas of the Brain Critical in Neurodegeneration



- · Grey, black shading/dots represent ASO; areas with complete lack of ASO would appear white
- Single intrathecal dose of Cy5-labeled nusinersen-SNA (1.5 mg/kg) in 8-week old mice
- · Mice sacrificed 14 days post administration

administration

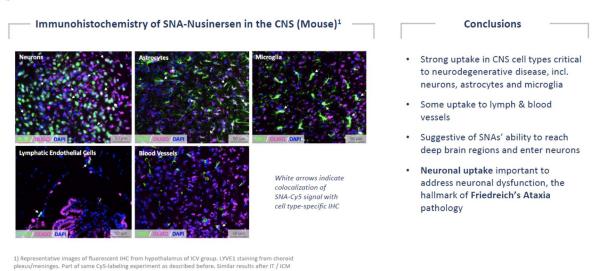
• Images are representative sagittal (left) and coronal (right) sections (5 μm)

Conclusions

- Confirms broad CNS distribution of previous studies
- Demonstrates SNAs distribute to all regions of the brain, including the cortex (Batten), cerebellum (FA, Batten) and thalamus (Batten)
- Laminar patterning suggests prominent uptake by neurons
- Similar results after intrathecal (IT) / intra-cisterna magna (ICM) / intracerebroventricular (ICV) administration

exicure 18

## SNAs Distribute Across Key Cell Types in the CNS, incl. Neurons



exicure 19

183. The statements in Paragraph 182 were false and misleading when made because SNAs were not reaching areas of the brain and neurons critical to neurodegeneration and "Friedreich's Ataxia pathology." Further, these positive statements misleadingly omitted the

#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 58 of 122 PageID #:657

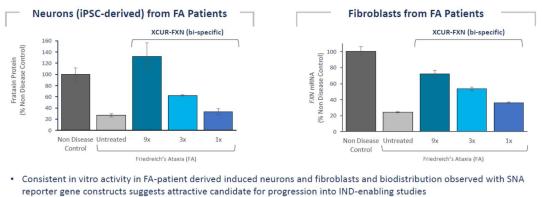
material fact that Corbett's experiments had shown that XCUR-FXN did not work: the XCUR-FXN program was based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and Corbett had falsified the results of his experiments on fibroblasts, neurons, and mitochondria. As Exicure itself has admitted, Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021."

#### 3. January 11, 2021 H.C. Wainwright BioConnect Conference; March 9, 2021 H.C. Wainwright Global Life Science Conference

184. At the January 11, 2021 H.C. Wainwright BioConnect Conference, Giljohann stated: "What we've seen, and we just released this week as well, is that we are able to dose dependently and consistently upregulate frataxin protein and frataxin mRNA in both monospecific and, I think more excitingly, [bispecific] spherical nucleic acids as well."

185. On March 9, 2021, at the H.C. Wainwright Global Life Science Conference, Giljohann stated: "Again, on slide 24, we've been able to show that our drugs work, both increasing the amount of frataxin protein and messenger RNA in cell lines that are derived from Friedreich's Ataxia patients." Slide 24 is shown below:

# XCUR-FXN Dose-Dependently Upregulates Frataxin Protein and FXN mRNA



All experiments are conducted with unassisted free uptake, without the use of transfection agents or electroporation,

exicure 24

186. The statements in Paragraphs 184-185 were false and misleading when made because the reported data were false, Exicure had not "been able to show that [its] drugs work," and XCUR-FXN did not "dose dependently" or "consistently upregulate frataxin protein and frataxin mRNA" or "increas[e] the amount of frataxin protein and messenger RNA in cell lines that are derived from Friedreich's Ataxia patients." Instead, Corbett's experiments had shown that XCUR-FXN did not work: the XCUR-FXN program was based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and Corbett had falsified the results of his experiments on fibroblasts, neurons, and mitochondria. As Exicure itself has admitted, Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021."

#### 4. April 12, 2021 Investor Presentation

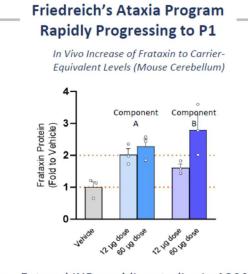
187. Exicure's April 12, 2021 investor presentation, which was publicly disseminated through Exicure's website, stated with respect to XCUR-FXN: "~3x frataxin upregulation

suggesting high translatability to in vivo studies

Key experimental conditions: (left) Frataxin protein study: N = 3 biological replicates, incubation time = 96 hours, days in vitro at time of experiment = 18 days; (right) FXN mRNA study: N = 3 biological replicates, incubation time = 72 hours, hours in vitro at time of experiment = 24 hours; (both) Cell line 541/420 GAA

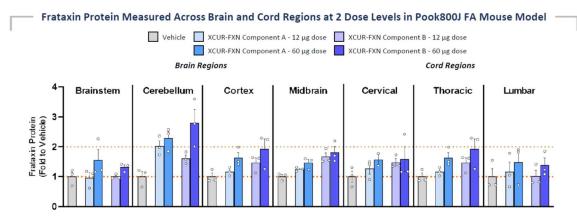
#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 60 of 122 PageID #:659

**achieved in CNS of FA mouse model** indicates potential for disease resolution" (emphasis in original). Slide 4 showed the purported results of *in vivo* testing of XCUR-FXN on mice:



- Entered IND-enabling studies in 4Q20
- IND filing anticipated by end of 2021
- Potential for early target engagement read-out in FIH via CSF frataxin levels
- 188. Similarly, slide 21 stated:

# Magnitude of *In Vivo* Frataxin Upregulation by the Components of XCUR-FXN Indicates Possibility for Disease Resolution in Patients



• The highest fold change (2-3x) is observed in the cerebellum, the brain area with highest known disease relevance for FA

• The two component oligonucleotides shown are the individual components of XCUR-FXN; *in vitro* results show synergy in *FXN* upregulation via combination of individual oligonucleotides into a multi-targeting SNA

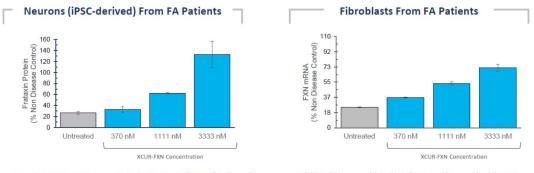
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#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 61 of 122 PageID #:660

189. The statements in Paragraphs 187-188 were false and misleading when made because the reported data were false, and in truth, XCUR-FXN had not "achieved" "~3x frataxin upregulation" or a "2-3x" frataxin increase in mice. Instead, Corbett's experiments on mice had shown that XCUR-FXN did not work: it did not affect the frataxin levels of the mice, and Corbett simply manipulated the raw data using the Prism software to create charts that falsely showed a frataxin increase. Further, the XCUR-FXN program was based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and as Exicure itself has admitted, Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021."

190. Slides 19 and 20 showed XCUR-FXN's purported effect on frataxin protein and mRNA in neurons, fibroblasts, and mitochondria:

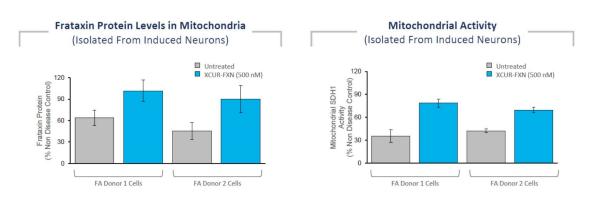
## **XCUR-FXN Dose-Dependently Upregulates Frataxin** Protein and mRNA



Consistent in vitro activity in FA-patient derived induced neurons and fibroblasts and biodistribution observed with SNA reporter gene constructs suggests attractive candidate for progression into IND-enabling studies

· All experiments are conducted with unassisted free uptake<sup>1</sup> suggesting high translatability to in vivo studies

1) No use of transfection agents or electroporation Key experimental conditions: (left) Frataxin protein study: N = 3 biological replicates, incubation time = 96 hours, days in vitro at time of experiment = 18 days; (right) FXV mRNA study: N = 3 biological replicates incubation time = 72 hours, hours in vitro at time of experiment = 24 hours; (both) Cell line 541/420 GAA exicure @2021 Exicure, Inc.@ All Rights Reserved.



### **XCUR-FXN Increases Mitochondrial Frataxtin and Activity**

191. The statements in Paragraph 190 were false and misleading when made because the reported data were false, and in truth, XCUR-FXN did not affect frataxin protein or FXN mRNA levels. Corbett's experiments had shown that XCUR-FXN did not work: the XCUR-FXN program was based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and Corbett had falsified the results of his experiments on fibroblasts, neurons, and mitochondria. As Exicure itself has admitted, Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021."

#### 5. May 27, 2021 Investor Presentation

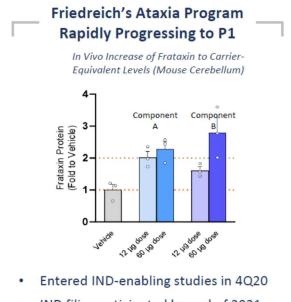
192. Exicure's May 27, 2021 investor presentation, which was publicly disseminated through Exicure's website, stated with respect to XCUR-FXN: "~3x frataxin upregulation achieved in CNS of FA mouse model indicates potential for disease resolution." Slide 4 showed the purported results of *in vivo* testing of XCUR-FXN on mice:

<sup>•</sup> XCUR-FXN returns frataxin levels to non-diseased level in mitochondria, the target cellular compartment in FA

XCUR-FXN increases mitochondrial energy metabolism (SDH<sup>1</sup> activity); indicating improved mitochondrial health

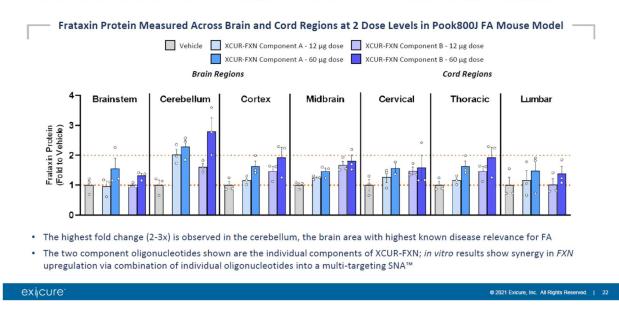
<sup>·</sup> Highest reported potency in a transfection-free system for improving mitochondrial activity in patient-derived mitochondria

<sup>1)</sup> SDH - succinate dehydrogenase **EXICUTE** ©2021 Exicure, Inc.® All Rights Reserve



- . IND filing anticipated by end of 2021
- Potential for early target engagement • read-out in FIH via CSF frataxin levels
- 193. Similarly, slide 22 stated:

#### Magnitude of In Vivo Frataxin Upregulation by the Components of **XCUR-FXN Showed Possibility for Disease Resolution in Patients**

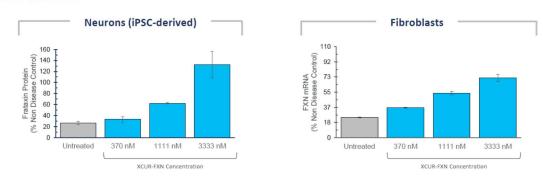


194. The statements in Paragraphs 192-193 were false and misleading when made because the reported data were false, and in truth, XCUR-FXN had not "achieved" "~3x frataxin

#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 64 of 122 PageID #:663

upregulation" or a "2-3x" frataxin increase in mice. Instead, Corbett's experiments on mice had shown that XCUR-FXN did not work: it did not affect the frataxin levels of the mice, and Corbett simply manipulated the raw data using the Prism software to create charts that falsely showed a frataxin increase. Further, the XCUR-FXN program was based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and as Exicure itself has admitted, Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021."

195. Slides 20 and 21 showed XCUR-FXN's purported effect on frataxin protein and mRNA in neurons, fibroblasts, and mitochondria:



XCUR-FXN Dose-Dependently Upregulates Frataxin Protein and mRNA

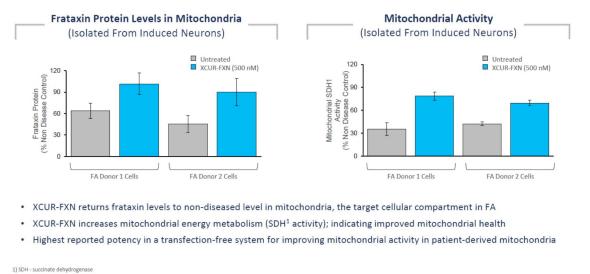
 Consistent *in vitro* activity in FA patient-derived induced neurons and fibroblasts and biodistribution observed with SNA™ reporter gene constructs suggest attractive candidate for progression into IND-enabling studies

1) No use of transfection agents or electroporation Key experimental conditions: (left) Frataxin protein study: N = 3 biological replicates, incubation time = 96 hours, days in vitro at time of experiment = 18 days; (right) FXN mRNA study: N = 3 biological replicates, incubation time = 72 hours, hours in vitro at time of experiment = 24 hours; (both) Cell line 541/420 GAA

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<sup>·</sup> All experiments are conducted with unassisted free uptake1 suggesting high translatability to in vivo studies



#### **XCUR-FXN Increases Mitochondrial Frataxtin and Activity**

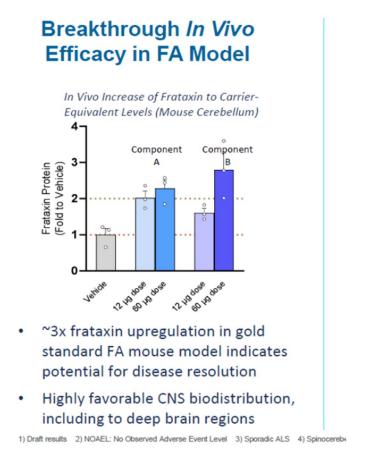
exicure.

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196. The statements in Paragraph 195 were false and misleading when made because the reported data were false, and in truth, XCUR-FXN did not affect frataxin protein or FXN mRNA levels. Corbett's experiments had shown that XCUR-FXN did not work: the XCUR-FXN program was based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and Corbett had falsified the results of his experiments on fibroblasts, neurons, and mitochondria. As Exicure itself has admitted, Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021."

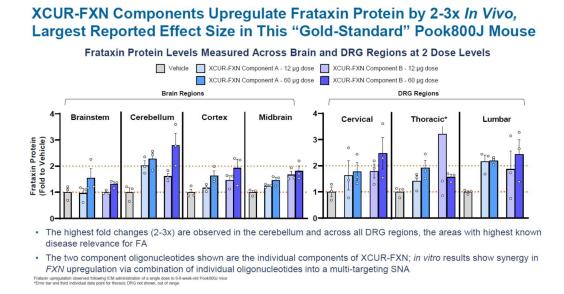
#### 6. July 15, 2021 R&D Day Presentation

197. At the July 15, 2021 R&D Day, Giljohann stated that "we've been able to show, throughout the mouse cerebellum, Friedreich's Ataxia important markers, like frataxin, upregulated," and presented a graph, created by Corbett and excerpted below, stating that XCUR-FXN had "*Breakthrough* In Vivo *Efficacy*":



198. Based on Corbett's false data and graphs, Daniel stated: "Exicure has, to our knowledge, generated the most promising *in vivo* results in the gold-standard Pook800J mouse, showing *3x frataxin upregulation in the cerebellum*." He explained that Exicure had "very exciting results with XCUR-FXN components in the gold-standard mouse model, called the Pook800J mouse, where *we're seeing a tripling of frataxin protein levels in key tissues*."

199. Based on the following graph created by Corbett, Daniel claimed that XCUR-FXN doubled or tripled frataxin levels in living mice:



200. Based on this false graph, Daniel stated "that in key—in key FA-driving disease regions, like the cerebellum, second from left on this plot, as well as in dorsal root ganglia regions of the spinal cord, which is the right-hand side of the plot, *we're seeing a two to three-fold upregulation of frataxin protein*." Daniel added that "*what we're seeing here with the components of XCUR-FXN, in this gold-standard mouse model, is that two to three-fold upregulation of frataxin protein*."

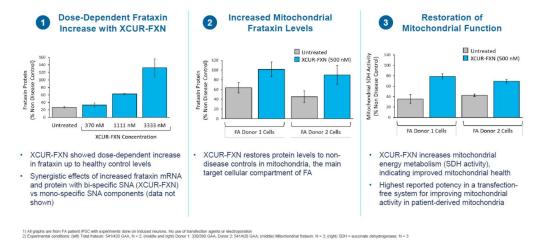
201. The statements in Paragraphs 197-200 were false and misleading when made because the reported data were false, and in truth, XCUR-FXN had not shown "~3x frataxin upregulation" in mice, "Breakthrough *In Vivo* Efficacy," "3x frataxin upregulation in the cerebellum," a "tripling of frataxin protein levels," or "a two to three-fold upregulation of frataxin protein" in the brain and DRG. Instead, Corbett's experiments on mice had shown that XCUR-FXN did not work: it did not affect the frataxin levels of the mice, and Corbett simply manipulated the raw data using the Prism software to create charts that falsely showed a frataxin increase. Further, the XCUR-FXN program was based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and as Exicure itself has admitted,

#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 68 of 122 PageID #:667

Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021."

202. During the presentation, Daniel displayed the following slide created by Corbett:

#### XCUR-FXN Dose-Dependently Upregulates Frataxin Protein and Restores Mitochondrial Function in Induced Neurons<sup>1</sup> from FA Patients



#### 203. Based on Corbett's false data and graphs, Daniel claimed:

So, focusing your attention on the left-hand side of the slide here, I've got a plot of frataxin protein as a function of XCUR-FXN concentration. And we see a dosedependent increase in that frataxin protein in these FA patient induced neurons. And the level of frataxin protein induced by XCUR-FXN at the highest concentration exceeds that of the non-disease control induced neurons. So we're really ramping up here the level of frataxin with XCUR-FXN. In the center panel, that increased level of frataxin found in the cell also translates into increased mitochondrial frataxin levels. So we've got two FA donor cells here, which were transformed into neurons. And the treated cells-those results are illustrated in blue in the center panel here—we have a total restoration of frataxin protein levels in mitochondria to the level of a non-disease control. So we're making the protein; we're getting it to the right place in the cell. And then finally, in the righthand panel, that increase in mitochondrial frataxin level ultimately leads to a restoration of mitochondrial function, which as I outlined previously, is one of those drivers of neuronal dysfunction and death. Here we've got two FA patient donor cells, which were transformed again into neurons; and we measured mitochondria function through a metabolic measure, specifically, succinate dehydrogenase activity; and we see that XCUR-FXN-treated cells, plotted in blue bars, have much greater SDH activity than untreated cells.

204. The statements in Paragraphs 202-203 were false and misleading when made

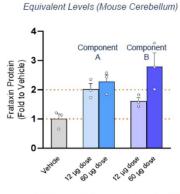
because the reported data were false, and in truth, XCUR-FXN did not affect frataxin protein or

#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 69 of 122 PageID #:668

FXN mRNA levels or mitochondrial activity. Corbett's experiments had shown that XCUR-FXN did not work: the XCUR-FXN program was based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and Corbett had falsified the results of his experiments on fibroblasts, neurons, and mitochondria. As Exicure itself has admitted, Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021."

#### 7. Exicure's July 16, 2021 Investor Presentation

205. Exicure's July 16, 2021 investor presentation, which was publicly disseminated through Exicure's website, claimed that "XCUR-FXN upregulates frataxin, targeting the molecular cause of Friedreich's Ataxia (FA)," and that "~3x frataxin upregulation achieved in CNS of FA mouse model indicates potential for disease resolution." Slide 4 showed the purported results of *in vivo* testing of XCUR-FXN on mice:



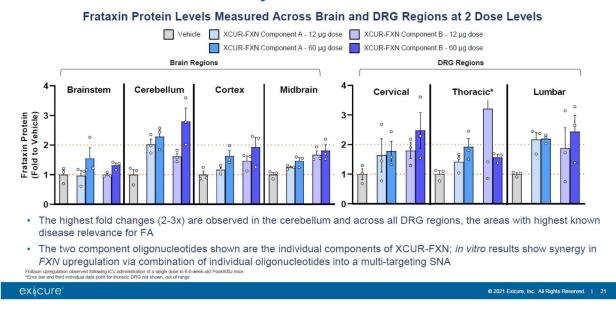
#### Friedreich's Ataxia Program Rapidly Progressing to P1

In Vivo Increase of Frataxin to Carrier-

- Entered IND-enabling studies in 4Q20
- IND filing anticipated by end of 2021
- Potential for early target engagement read-out in FIH via CSF frataxin levels

#### 206. Similarly, slide 21 stated:

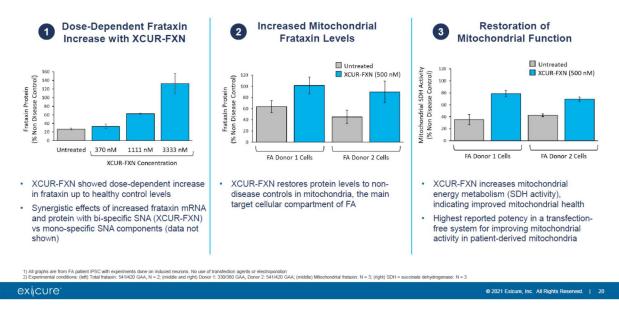
# Magnitude of In Vivo Frataxin Upregulation by the Components of XCUR-FXN Showed Possibility for Disease Resolution in Patients



207. The statements in Paragraphs 205-206 were false and misleading when made because the reported data were false, and in truth, XCUR-FXN had not shown "~3x frataxin upregulation" or a "2-3x" frataxin increase in mice. Instead, Corbett's experiments on mice had shown that XCUR-FXN did not work: it did not affect the frataxin levels of the mice, and Corbett simply manipulated the raw data using the Prism software to create charts that falsely showed a frataxin increase. Further, the XCUR-FXN program was based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and as Exicure itself has admitted, Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021."

208. Slide 20 showed XCUR-FXN's purported effect on frataxin protein and mRNA in neurons and mitochondria:

#### **XCUR-FXN** Dose-Dependently Upregulates Frataxin Protein and Restores Mitochondrial Function in Induced Neurons<sup>1</sup> from FA Patients



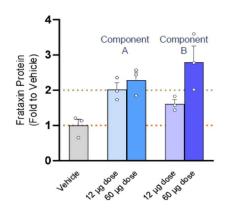
209. The statements in Paragraph 208 were false and misleading when made because the reported data were false, and in truth, XCUR-FXN did not affect frataxin protein or FXN mRNA levels. Corbett's experiments had shown that XCUR-FXN did not work: the XCUR-FXN program was based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and Corbett had falsified the results of his experiments on fibroblasts, neurons, and mitochondria. As Exicure itself has admitted, Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021."

# 8. August 2, 2021 and August 5, 2021 Investor Presentations

210. Exicure's investor presentations dated August 2, 2021 and August 5, 2021, which were publicly disseminated through Exicure's website, each stated with respect to XCUR-FXN that there was "~3x frataxin upregulation in mouse model . . . ." Slide 5 showed the purported results of *in vivo* testing of XCUR-FXN on mice:

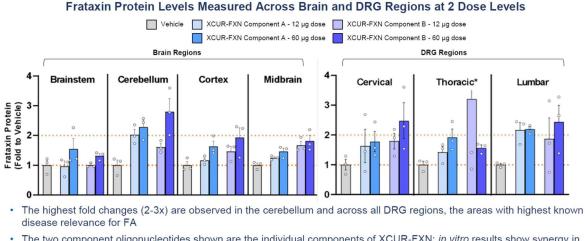
#### Friedreich's Ataxia Program Rapidly Progressing to P1

In Vivo Increase of Frataxin to Carrier-Equivalent Levels (Mouse Cerebellum)



- Entered IND-enabling studies in 4Q20 •
- IND filing anticipated by end of 2021 •
- Potential for early target engagement . read-out in FIH via CSF frataxin levels
- 211. Similarly, slide 23 stated:

### Magnitude of In Vivo Frataxin Upregulation by the Components of **XCUR-FXN Showed Possibility for Disease Resolution in Patients**



The two component oligonucleotides shown are the individual components of XCUR-FXN; in vitro results show synergy in

FXN upregulation via combination of individual oligonucleotides into a multi-targeting SNA ic DRG

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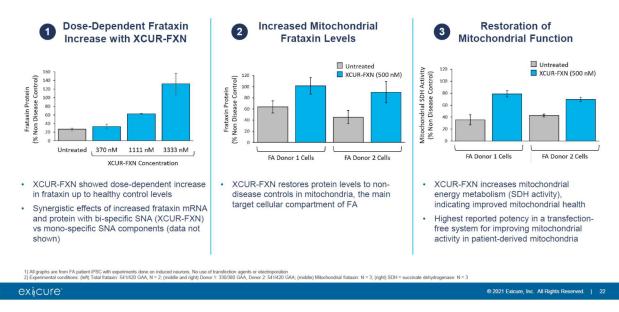
### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 73 of 122 PageID #:672

212. Slide 26 stated that XCUR-FXN had shown "*In vivo* frataxin upregulation of  $\sim$ 3x in the CNS of mice following intrathecal single dose administration of the individual components of XCUR-FXN at comparatively low doses (60 µg)."

213. The statements in Paragraphs 210-212 were false and misleading when made because the reported data were false, and in truth, XCUR-FXN had not shown "~3x frataxin upregulation" or a "2-3x" frataxin increase in mice. Instead, Corbett's experiments on mice had shown that XCUR-FXN did not work: it did not affect the frataxin levels of the mice, and Corbett simply manipulated the raw data using the Prism software to create charts that falsely showed a frataxin increase. Further, the XCUR-FXN program was based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and as Exicure itself has admitted, Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021."

214. Slide 22 showed XCUR-FXN's purported effect on frataxin protein and mRNA in neurons and mitochondria:

## **XCUR-FXN** Dose-Dependently Upregulates Frataxin Protein and Restores Mitochondrial Function in Induced Neurons from FA Patients<sup>1,2</sup>



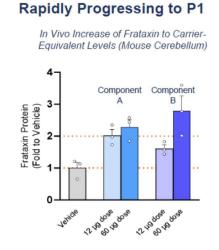
215. The statements in Paragraph 214 were false and misleading when made because the reported data were false, and in truth, XCUR-FXN did not affect frataxin protein or FXN mRNA levels. Corbett's experiments had shown that XCUR-FXN did not work: the XCUR-FXN program was based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and Corbett had falsified the results of his experiments on fibroblasts, neurons, and mitochondria. As Exicure itself has admitted, Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021."

## 9. September 8, 2021 Investor Presentation

216. Exicure's September 8, 2021 investor presentation, which was publicly disseminated through Exicure's website, stated: "XCUR-FXN: lead CNS program upregulates frataxin, targeting molecular cause of Friedreich's Ataxia (FA)"; "~3x frataxin upregulation in mouse model indicates potential for disease resolution." Slide 5 showed the purported results of *in vivo* testing of XCUR-FXN on mice:

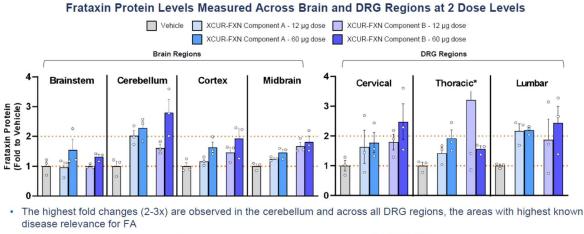
Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 75 of 122 PageID #:674

Friedreich's Ataxia Program



- Entered IND-enabling studies in 4Q20
- IND filing anticipated by end of 2021
- Potential for early target engagement read-out in FIH via CSF frataxin levels
- 217. Similarly, slide 23 stated:

## Magnitude of *In Vivo* Frataxin Upregulation by the Components of XCUR-FXN Showed Possibility for Disease Resolution in Patients



 The two component oligonucleotides shown are the individual components of XCUR-FXN; *in vitro* results show synergy in *FXN* upregulation via combination of individual oligonucleotides into a multi-targeting SNA

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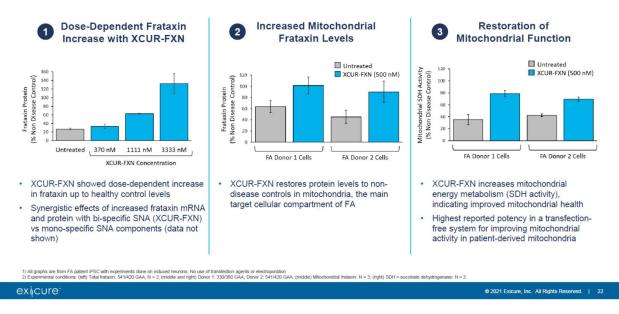
#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 76 of 122 PageID #:675

218. Slide 26 stated that XCUR-FXN had shown "*In vivo* frataxin upregulation of  $\sim$ 3x in the CNS of mice following intrathecal single dose administration of the individual components of XCUR-FXN at comparatively low doses (60 µg)."

219. The statements in Paragraphs 216-218 were false and misleading when made because the reported data were false, and in truth, XCUR-FXN had not shown "~3x frataxin upregulation" or a "2-3x" frataxin increase in mice. Instead, Corbett's experiments on mice had shown that XCUR-FXN did not work: it did not affect the frataxin levels of the mice, and Corbett simply manipulated the raw data using the Prism software to create charts that falsely showed a frataxin increase. Further, the XCUR-FXN program was based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and as Exicure itself has admitted, Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021."

220. Slide 22 showed XCUR-FXN's purported effect on frataxin protein and mRNA in neurons and mitochondria:

## **XCUR-FXN Dose-Dependently Upregulates Frataxin Protein and Restores Mitochondrial Function in Induced Neurons from FA Patients**<sup>1,2</sup>



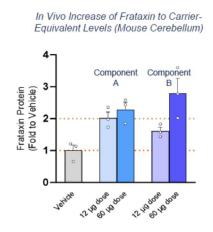
221. The statements in Paragraph 220 were false and misleading when made because the reported data were false, and in truth, XCUR-FXN did not affect frataxin protein or FXN mRNA levels. Corbett's experiments had shown that XCUR-FXN did not work: the XCUR-FXN program was based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and Corbett had falsified the results of his experiments on fibroblasts, neurons, and mitochondria. As Exicure itself has admitted, Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021."

## September 13, 2021 H.C. Wainwright 23rd Annual Global Investment Conference; September 30, 2021 Benzinga Healthcare Small Cap Conference; October 5, 2021 Chardan Genetic Medicines Conference

222. At the September 13, 2021 H.C. Wainwright 23rd Annual Global Investment Conference, Giljohann stated (while showing slide 5 of Exicure's September 8, 2021 investor presentation, shown above and reproduced in relevant part below) that "in the in vivo models in Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 78 of 122 PageID #:677

the middle here you'll see that example from our Friedrich's Ataxia program, where *we've shown that different components of our spherical nucleic acid are able to reach up into the brain and increase in this case frataxin protein by targeting the frataxin gene.*"

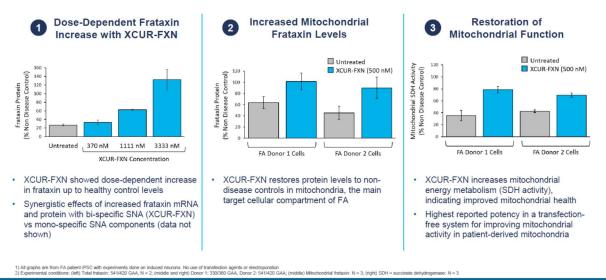
> Friedreich's Ataxia Program Rapidly Progressing to P1



- Entered IND-enabling studies in 4Q20
- IND filing anticipated by end of 2021
- Potential for early target engagement read-out in FIH via CSF frataxin levels

223. Giljohann further stated (while showing slides 22 and 23 of Exicure's September 8, 2021 investor presentation, shown above and reproduced below) that "we're able to get into Friedreich's Ataxia patient cells *in vitro* and show a *dose dependent upregulation of the relevant frataxin proteins*. When we take this into an animal model [*in vivo*], as well, *you can see that we're getting a really nice increase. The highest fold changes are two to three-fold above the baseline* and it's across both the cerebellum and the DRG."

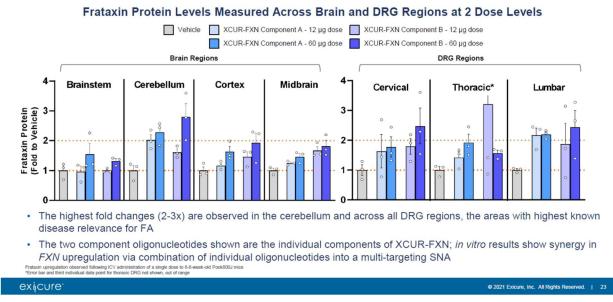
## **XCUR-FXN Dose-Dependently Upregulates Frataxin Protein and Restores Mitochondrial Function in Induced Neurons from FA Patients**<sup>1,2</sup>



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# Magnitude of *In Vivo* Frataxin Upregulation by the Components of XCUR-FXN Showed Possibility for Disease Resolution in Patients



224. The statements in Paragraphs 222-223 were false and misleading when made because the reported data were false, and XCUR-FXN was not "able to reach up into the brain" and "increase . . . frataxin protein," did not "show a dose dependent upregulation of the relevant

#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 80 of 122 PageID #:679

frataxin proteins," and did not produce "a really nice increase" in mice with "changes [that] are two to three-fold above the baseline." Instead, Corbett's experiments on mice had shown that XCUR-FXN did not work: it did not affect the frataxin levels of the mice, and Corbett simply manipulated the raw data using the Prism software to create charts that falsely showed a frataxin increase. Further, the XCUR-FXN program was based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and Corbett had falsified the results of his experiments on fibroblasts, neurons, and mitochondria (as well as mice). As Exicure itself has admitted, Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021."

225. At the September 30, 2021 Benzinga Healthcare Small Cap Conference, Defendant Giljohann displayed slides 22 and 23 of Exicure's September 8, 2021 investor presentation (shown above). Giljohann—who already knew from the most recent Charles River experiment that XCUR-FXN had no effect on frataxin levels and showed alarming toxicity, killing a number of mice—stated:

You can see here in the blue bars that we're getting a nice increase in the frataxin level after dosing. It also works in the animals. So we've shown that as well—if you look at different levels across the regions of the brain, we're seeing high delivery of the appropriate regions of the brain, we're seeing upregulation of frataxin protein.

So from our perspective, all the components are here. We're getting into cells, we're getting into tissues, we're getting into the right areas of the brain, and then *we're able to have an effect*.

226. The statements in Paragraph 225 were false and misleading when made because the reported data were false, and XCUR-FXN did not produce "a nice increase in the frataxin level," did not "work[] in animals," did not achieve "high delivery of the appropriate regions of the brain" or "upregulation of frataxin protein," and was not "able to have an effect." Instead, Corbett's

#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 81 of 122 PageID #:680

experiments on mice had shown that XCUR-FXN did not work: it did not affect the frataxin levels of the mice, and Corbett simply manipulated the raw data using the Prism software to create charts that falsely showed a frataxin increase. Indeed, at the time of these statements, Giljohann had been told in calls during September 2021 that XCUR-FXN had no effect on frataxin levels and was killing large numbers of mice—material facts that these statements misleadingly omitted. Further, the XCUR-FXN program was based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and as Exicure itself has admitted, Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021."

227. At the October 5, 2021 Chardan Genetic Medicines Conference, Giljohann stated that "in our Neurology Pipeline R&D Day and we presented some of that data, which showed that after intrathecal injection, we are able to get deep into the brain and into the brain regions that we need to target that FXN gene. . . . [W]e wrapped up our tox study in rats with really no significant findings there and currently running our non-human primate study with the idea that now with all these components put together, we can plan that, submit that IND before the end of the year. So I think really sets of compelling preclinical data, the most on track in terms of showing that we are ready to go to the clinic would be those lines that we are drawing from the in vitro results to the in-vivo distribution to that penetration into the right tissues."

228. The statements in Paragraph 227 were false and misleading when made because the reported data were false, and XCUR-FXN did not "get deep into the brain and into the brain regions that we need to target that FXN gene." Instead, Corbett's experiments on mice had shown that XCUR-FXN did not work: it did not affect the frataxin levels of the mice, and Corbett simply manipulated the raw data using the Prism software to create charts that falsely showed a frataxin

#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 82 of 122 PageID #:681

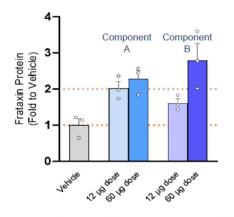
increase. Indeed, at the time of these statements, Giljohann had been told in calls during September 2021 that XCUR-FXN had no effect on frataxin levels and was killing large numbers of mice material facts that these statements misleadingly omitted. Further, the XCUR-FXN program was based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and Corbett had falsified the results of his experiments on fibroblasts, neurons, and mitochondria. As Exicure itself has admitted, Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021." The statements in Paragraph 227 were also misleading because, in referring positively to XCUR-FXN being able to "get deep into the brain and into the brain regions that we need to target that FXN gene," "those lines that we are drawing from the in vitro results to the in-vivo distribution to that penetration into the right tissues," "compelling preclinical data," and Exicure having "wrapped up our tox study in rats with really no significant findings," Defendant Giljohann concealed the material, negative facts that XCUR-FXN had no effect on frataxin levels and displayed alarming toxicity, as reported to him in September 2021.

#### 11. October 13, 2021 Investor Presentation

229. Exicure's October 13, 2021 investor presentation, which was publicly disseminated through Exicure's website, stated: "XCUR-FXN: lead CNS program upregulates frataxin, targeting molecular cause of Friedreich's Ataxia (FA)"; "~3x frataxin upregulation in mouse model indicates potential for disease resolution." Slide 5 showed the purported results of *in vivo* testing of XCUR-FXN on mice:

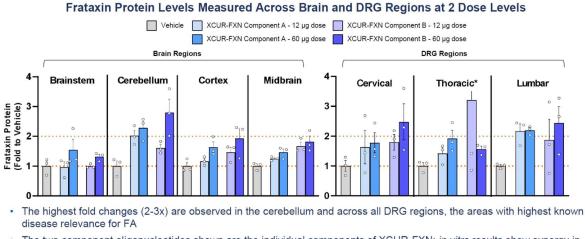
## Friedreich's Ataxia Program Rapidly Progressing to P1

In Vivo Increase of Frataxin to Carrier-Equivalent Levels (Mouse Cerebellum)



- Entered IND-enabling studies in 4Q20
- IND filing anticipated by end of 2021
- Potential for early target engagement read-out in FIH via CSF frataxin levels
- 230. Similarly, slide 23 stated:

## Magnitude of *In Vivo* Frataxin Upregulation by the Components of XCUR-FXN Showed Possibility for Disease Resolution in Patients



 The two component oligonucleotides shown are the individual components of XCUR-FXN; *in vitro* results show synergy in *FXN* upregulation via combination of individual oligonucleotides into a multi-targeting SNA
Fature upregulation classes following LM admittantiation of a single does to 63 week-dd Pool800J mice
""Torbar and the windwald alto point for thorace RBC and the work out of a more than the single does to 65 week-dd Pool800J mice

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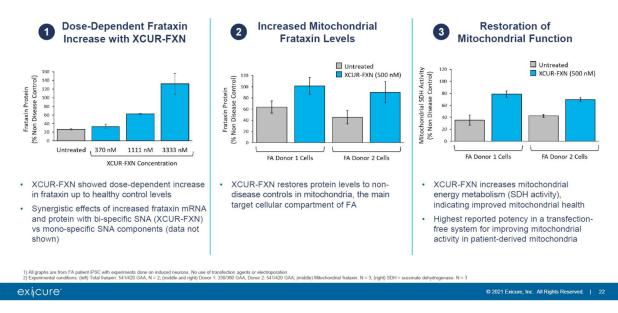
#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 84 of 122 PageID #:683

231. Slide 26 stated that XCUR-FXN had shown "*In vivo* frataxin upregulation of  $\sim$ 3x in the CNS of mice following intrathecal single dose administration of the individual components of XCUR-FXN at comparatively low doses (60 µg)."

232. The statements in Paragraphs 229-231 were false and misleading when made because the reported data were false, and in truth, XCUR-FXN had not shown "~3x frataxin upregulation" or a "2-3x" frataxin increase in mice. Instead, Corbett's experiments on mice had shown that XCUR-FXN did not work: it did not affect the frataxin levels of the mice, and Corbett simply manipulated the raw data using the Prism software to create charts that falsely showed a frataxin increase. Indeed, at the time of these statements, Giljohann had been told in calls during September 2021 that XCUR-FXN had no effect on frataxin levels and was killing large numbers of mice—material facts that these statements misleadingly omitted. Further, the XCUR-FXN program was based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and as Exicure itself has admitted, Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021."

233. Slide 22 showed XCUR-FXN's purported effect on frataxin protein and mRNA in neurons and mitochondria:

## **XCUR-FXN Dose-Dependently Upregulates Frataxin Protein and Restores Mitochondrial Function in Induced Neurons from FA Patients**<sup>1,2</sup>



234. The statements in Paragraph 233 were false and misleading when made because the reported data were false, and in truth, XCUR-FXN did not affect frataxin protein or FXN mRNA levels. Corbett's experiments had shown that XCUR-FXN did not work: the XCUR-FXN program was based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and Corbett had falsified the results of his experiments on fibroblasts, neurons, and mitochondria. As Exicure itself has admitted, Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021." Further, at the time of these statements, Giljohann had been told in calls during September 2021 that XCUR-FXN had no effect on frataxin levels and was killing large numbers of mice—material facts that these statements misleadingly omitted.

## B. False and Misleading Statements in Exicure's SEC Filings

235. Exicure's 2020 Form 10-K, filed on March 11, 2021 and signed by Defendant Giljohann (the "2020 10-K"), stated that "in *in vitro* experiments," Exicure had "observed synergistic upregulation of FXN mRNA in cells treated with XCUR-FXN compared to its mono-targeting components alone at the same total oligonucleotide dose. In FA patient-derived induced neurons, XCUR-FXN has shown potent, dose-dependent upregulation of frataxin protein. In isolated mitochondria from the same induced neurons, XCUR-FXN normalized frataxin protein levels at low concentrations, resulting in substantial improvements in mitochondrial respiration, as measured by succinate dehydrogenase (SDH) activity."

236. The 2020 10-K further stated: "As discussed during our R&D day presentation in January 2021, in preclinical experiments, we observed that XCUR-FXN increased frataxin protein levels in fibroblasts and neurons derived from FA patients to near normal levels. Importantly, we observed near normal levels of frataxin protein in mitochondrion, the target cellular compartment, and 70-80% of near normal mitochondrial activity in neurons derived from FA patients."

237. The statements in Paragraphs 235-236 were false and misleading when made because the reported data were false, and Exicure had not "observed synergistic upregulation of FXN mRNA in cells treated with XCUR-FXN," XCUR-FXN had not "shown potent, dose-dependent upregulation of frataxin protein" in induced neurons or "normalized frataxin protein levels at low concentrations [in mitochondria], resulting in substantial improvements in mitochondrial respiration," and Exicure had not "observed that XCUR-FXN increased frataxin protein levels in fibroblasts and neurons derived from FA patients to near normal levels" or "observed near normal levels of frataxin protein in mitochondrion, the target cellular compartment, and 70-80% of near normal mitochondrial activity in neurons derived from FA patients." Instead, Corbett's experiments had shown that XCUR-FXN did not work: the XCUR-FXN program was

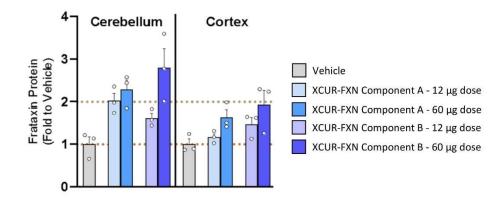
### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 87 of 122 PageID #:686

based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and Corbett had falsified the results of his experiments on fibroblasts, neurons, and mitochondria. As Exicure itself has admitted, Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021."

238. Exicure's Form 10-Q for the first quarter of 2021, filed on May 12, 2021 and signed

by Defendant Giljohann (the "1Q21 10-Q"), stated:

We have tested SNAs consisting of the two individual oligonucleotide components of XCUR-FXN in a Pook800J mouse model. This model contains a hemizygous insertion of the human disease allele with approximately 800 GAA repeats, and lacks endogenous mouse FXN. For reference, in humans, 800 GAA repeats would represent classic FA disease manifestation with an early disease onset. The mice were administered one of the two XCUR-FXN oligonucleotide components at one of two dose levels via a single intra-cisterna magna injection. After 14 days, key regions of the brain and spinal cord were harvested and analyzed for FXN protein levels. *As illustrated in the figure below, the components of XCUR-FXN increased frataxin protein levels by 2-3 fold in the cerebellum and the cortex*, the two regions we believe are critical for addressing neurological manifestations of FA. *We also found similar increases in FXN levels in the brainstem, the midbrain and throughout the spinal cord*.



239. The 1Q21 10-Q also stated: "Moreover, in *in vitro* experiments, we have observed synergistic upregulation of FXN mRNA in cells treated with XCUR-FXN compared to its individual components alone at the same total oligonucleotide dose."

#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 88 of 122 PageID #:687

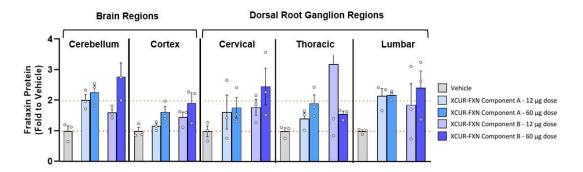
240. Exicure's press release on May 12, 2021 stated: "During the first quarter of 2021, the Company continued to advance preclinical and IND-enabling studies of XCUR-FXN in FA and disclosed encouraging preclinical mouse data, demonstrating significant upregulation of XCUR-FXN components in the brain and spinal cord."

241. The statements in Paragraphs 238-240 were false and misleading when made because the reported data were false, and XCUR-FXN had not "increased frataxin protein levels by 2-3 fold in the cerebellum and the cortex" or caused "similar increases in FXN levels in the brainstem, the midbrain and throughout the spinal cord," Exicure had not "observed synergistic upregulation of FXN mRNA in cells treated with XCUR-FXN," and Exicure did not have preclinical mouse data "demonstrating significant upregulation of XCUR-FXN components in the brain and spinal cord." Instead, Corbett's experiments on mice had shown that XCUR-FXN did not work: it did not affect the frataxin levels of the mice, and Corbett simply manipulated the raw data using the Prism software to create charts that falsely showed a frataxin increase. Further, the XCUR-FXN program was based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and Corbett had falsified the results of his experiments on fibroblasts, neurons, and mitochondria (as well as mice). As Exicure itself has admitted, Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021."

242. Exicure's Form 10-Q for the second quarter of 2021, filed on August 12, 2021 and signed by Defendant Bock (the "2Q21 10-Q"), stated:

As illustrated in the figure below, *the components of XCUR-FXN increased frataxin protein levels by 2-3 fold in the cerebellum, cortex, and dorsal root ganglia neurons*; these regions we believe are critical for addressing neurological manifestations of FA. We also found similar increases in frataxin protein levels in the brainstem, the midbrain and throughout the spinal cord.

### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 89 of 122 PageID #:688



243. Exicure's accompanying press release, dated August 12, 2021, stated: "Observed 2-3x fold change in measurable Frataxin protein in the cerebellum and dorsal root ganglia (amongst other important brain and spinal regions) in Pook800J mouse model indicating potential for disease resolution."

244. The statements in Paragraphs 242-243 were false and misleading when made because the reported data were false, and XCUR-FXN had not "increased frataxin protein levels by 2-3 fold in the cerebellum, cortex, and dorsal root ganglia neurons" or caused "similar increases in frataxin protein levels in the brainstem, the midbrain and throughout the spinal cord," and Exicure had not "Observed 2-3x fold change in measurable Frataxin protein in the cerebellum and dorsal root ganglia (amongst other important brain and spinal regions) in Pook800J mouse model." Instead, Corbett's experiments on mice had shown that XCUR-FXN did not work: it did not affect the frataxin levels of the mice, and Corbett simply manipulated the raw data using the Prism software to create charts that falsely showed a frataxin increase. Further, the XCUR-FXN program was based on an unreliable, unqualified assay, as reported to Anderson and Corbett before the Class Period, and as Exicure itself has admitted, Corbett "misreported raw data" from "at least three different experiments" on XCUR-FXN that was "included in various public presentations and SEC filings from as early as January 7, 2021."

## C. False and Misleading Certifications of Effective Controls

245. In each annual and quarterly SEC filing during the Class Period, Defendant Giljohann (and, for certain quarters, Defendants Giljohann and Bock) stated that they had (a) evaluated Exicure's disclosure controls and procedures and that they "were effective," and (b) "[d]esigned such disclosure controls and procedures ... to ensure that material information relating

to [Exicure] is made known to us by others within" Exicure. Specifically:

246. Exicure's 2020 10-K, signed by Defendant Giljohann, stated:

As of the end of the period covered by this Annual Report on Form 10-K, we carried out an evaluation, under the supervision and with the participation of our management, including our principal executive officer/interim principal financial officer, of the effectiveness of the design and operation of our disclosure controls and procedures pursuant to Exchange Act Rule 13a-15. Based upon, and as of the date of, this evaluation, our principal executive officer/interim principal financial officer concluded that *our disclosure controls and procedures were effective*.

247. The 2020 10-K attached a certification, signed by Giljohann, stating:

The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act rules 13a-15(f) and 15d-15(f)) for the registrant and have: a) *Designed such disclosure controls and procedures, or caused such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared*; . . . c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and procedures. . . c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and procedures and procedures.

248. The 1Q21 10-Q, signed by Defendant Giljohann, stated:

As of the end of the period covered by this Quarterly Report on Form 10-Q, we carried out an evaluation, under the supervision and with the participation of our management, including our principal executive officer/interim principal financial officer, of the effectiveness of the design and operation of our disclosure controls and procedures pursuant to Exchange Act Rule 13a-15. Based upon, and as of the date of, this evaluation, our principal executive officer/interim principal financial officer concluded that *our disclosure controls and procedures were effective*.

249. The 1Q21 10-Q attached a certification, signed by Giljohann, stating:

The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have: a) *Designed such disclosure controls and procedures, or caused such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared*; ... c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and procedures.

250. The 2Q21 10-Q, signed by Defendant Bock, stated:

As of the end of the period covered by this Quarterly Report on Form 10-Q, we carried out an evaluation, under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, of the effectiveness of the design and operation of our disclosure controls and procedures pursuant to Exchange Act Rule 13a-15. Based upon, and as of the date of, this evaluation, our principal executive officer and principal financial officer concluded that *our disclosure controls and procedures were effective*.

251. The 2Q21 10-Q attached certifications, signed by Giljohann and Bock, stating:

The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have: a) *Designed such disclosure controls and procedures, or caused such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared*; . . . c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and procedures.

252. The statements in Paragraphs 246-251 were false and misleading because Exicure's

disclosure controls and procedures were not effective and were not designed to ensure that material

information relating to Exicure was made known to management at the time of the statements.

## Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 92 of 122 PageID #:691

Instead, from at least 2019 and throughout the Class Period, Exicure's disclosure controls had a material weakness: No one at Exicure checked whether Exicure's SEC filings and presentations to investors matched the raw data from experiments, in violation of federal regulations. *See, e.g.*, 21 C.F.R. § 58.35(b)(6). This undisclosed material weakness facilitated the fraudulent dissemination of false data for nearly a year, as detailed above, and led to material misstatements in the 2020 10-K, the 1Q21 10-Q, and the 2Q21 10-Q.

253. Exicure's Form 10-Q for the third quarter of 2021, filed on November 19, 2021 and

signed by Defendant Bock (the "3Q21 10-Q"), stated:

As of the end of the period covered by this Quarterly Report on Form 10-Q, we carried out an evaluation, under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, of the effectiveness of the design and operation of our disclosure controls and procedures pursuant to Exchange Act Rule 13a-15. Based upon, and as of the date of, this evaluation, our principal executive officer and principal financial officer concluded that *our disclosure controls and procedures were effective*.

254. The 3Q21 10-Q attached certifications, signed by Giljohann and Bock, stating:

The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have: a) *Designed such disclosure controls and procedures, or caused such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared*; . . . c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and procedures.

255. The statements in Paragraphs 253-254 were false and misleading because Exicure's

disclosure controls and procedures were not effective and were not designed to ensure that material

information relating to Exicure was made known to management at the time of the statements.

Instead, from at least 2019 and throughout the Class Period, Exicure's disclosure controls had a

## Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 93 of 122 PageID #:692

material weakness: No one at Exicure checked whether Exicure's SEC filings and presentations to investors matched the raw data from experiments, in violation of federal regulations. *See, e.g.*, 21 C.F.R. § 58.35(b)(6). This undisclosed material weakness facilitated the fraudulent dissemination of false data for nearly a year, as detailed above, and led to material misstatements in the 3Q21 10-Q. Further, by the time of the 3Q21 10-Q, Giljohann had been told in calls during September 2021 that XCUR-FXN had no effect on frataxin levels and was killing large numbers of mice, contrary to Corbett's purported results, and on November 8, 2021, Corbett claimed to have committed "alleged improprieties" in researching XCUR-FXN. Despite the clear evidence of a material control failure and prior misstatements, the 3Q21 10-Q falsely repeated the claim that Exicure's disclosure controls were effective and that the 3Q21 10-Q contained no misstatements.

## VI. ADDITIONAL ALLEGATIONS OF SCIENTER

256. Together with the above-alleged facts, Defendants each acted with scienter in that each knew or recklessly disregarded the true facts in making the materially false and misleading statements identified herein.

## A. Defendant Corbett's Scienter Is Admitted

257. Defendant Corbett has admitted that he "intentionally misreported" data on XCUR-FXN's efficacy. Specifically, on December 10, 2021, Exicure stated that on or around November 8, 2021, Corbett "claimed that when he was employed by the Company, he *intentionally misreported* certain raw data related to the research and development of XCUR FXN."

258. This admission demonstrates Defendant Corbett's scienter: Corbett himself said he "intentionally misreported" the data. That means Corbett knew the data was false and intentionally reported the false data anyway—an inherently deceptive and fraudulent act.

## **B.** Former Employee Allegations

259. Several former Exicure employees provided information on a confidential basis that supports the strong inference that Defendants acted with scienter in making the alleged materially false and misleading statements above. Each FE was in a position to have personal knowledge of the information they conveyed. The FEs' accounts corroborate one another and the additional facts alleged herein.

260. FE-1 worked at Exicure as a Project Manager from February 2020 to October 2022. FE-1 reported to Scott Mix, Director of Translational Research; Mix reported to Weston Daniel, VP of Translational Research. FE-1's role involved planning to initiate clinical studies based on the results of preclinical experiments by Exicure's R&D Team; FE-1 reviewed results of experiments submitted to FE-1's team, secured contracts with CROs for further study, and reviewed the results of those studies. According to FE-1, based on personal knowledge:

- <u>Corbett Presents His Results on XCUR-FXN</u>: In June 2021, Corbett showed FE-1 and other members of FE-1's team a presentation, with a series of slides with data and graphs showing the results of the R&D Department's experiments on XCUR-FXN. The presentation was a full summary of Corbett's work on XCUR-FXN since he started at Exicure. The presentation included the results of *in vivo* mice experiments that were completed in April 2021. FE-1 explained that Corbett initially provided the slides in June 2021, and after making grammatical corrections, finalized the slides in late August or early September 2021.
- <u>Corbett Supervises the Initial XCUR-FXN Experiments</u>: FE-1 explained that the R&D Department's experiments were conducted by a CRO, Charles River. Corbett was Charles River's point of contact at Exicure and supervised Charles River in running the XCUR-FXN experiments.
- <u>Corbett's Results Prompt an Additional *In Vivo* Mice Study</u>: In summer 2021, FE-1 and colleagues engaged Charles River to perform a larger, longer-term *in vivo* mice study of XCUR-FXN's efficacy as well as its safety. The study used three cohorts of Pook800J mice, or approximately 30 to 45 animals. Two cohorts were given XCUR-FXN, while the third cohort was given a placebo.
- <u>Data Discrepancies Immediately Emerge</u>: In September 2021, Charles River began reporting the results of the mice study to FE-1's team. FE-1 received the results in PDF format. The first report from Charles River, provided after one week of the

experiment, showed that several mice had already died and others were sick. FE-1 explained that these data did not match Corbett's results, despite using mice of the same sex and age as Corbett. By mid-September 2021, FE-1 learned of two specific discrepancies between Corbett's results and the data from Charles River:

- First, Corbett's data showed that XCUR-FXN worked, but the September 2021 Charles River experiments showed *no effect*. As FE-1 explained, "*it was very clear that [XCUR-FXN] didn't work*." Specifically, Corbett's results had shown high "stair steps," with the mice responding to increasing doses of XCUR-FXN, while their response to the placebo was almost flat. By contrast, Charles River's results for XCUR-FXN showed almost a flat line with the placebo. Despite the exact same testing parameters, Charles River's experiments were not showing the positive results that Corbett had reported. FE-1 explained: "They did them under the same methodology, the same type of animal. It just didn't add up. It just didn't make sense to me." Charles River also tried raising the dose of XCUR-FXN to see if they could get the same results as Corbett. Again, however, the drug wasn't working.
- <u>Second</u>, during Charles River's experiments, both cohorts of mice that received XCUR-FXN had deaths—and *over half of one cohort died*. FE-1 noted that this "alarming" result was a potential safety issue and "raised flags."
- <u>CEO Giljohann Quickly Learns of the Problem</u>: Throughout this period, Mix and FE-1 had weekly calls with Charles River about the data. In mid-September 2021, Mix and FE-1 participated in a weekly call with Charles River to discuss the poor results from the experiment's first week. The other participants included Exicure CEO David Giljohann, COO Matthias Schroff, Chief Medical Officer Doug Feltner, and Senior Director, R&D Bart Anderson. During the call, Giljohann and Anderson said the Company needed to investigate XCUR-FXN's sterility before continuing the study.
- <u>Further Results Are Even Worse</u>: Charles River briefly stopped dosing the remaining mice with XCUR-FXN while Exicure's in-house team ran diagnostics and checked the drug's sterility. This took one week and showed that XCUR-FXN was sterile. Charles River then continued the experiment for another week, through the end of September 2021. The results were no better, and even larger numbers of mice died than before. At the end of September 2021, Giljohann, Schroff, Feltner, Anderson, Mix, FE-1 and others participated in another call with Charles River where these results were discussed and Charles River was directed to stop the study.
- <u>In October 2021, the Results Are Presented Again to Senior Management</u>: By early October 2021, Charles River provided a slide deck of data, and Mix instructed FE-1 to prepare a deck for internal presentation. FE-1 prepared the deck, and Mix and Daniel presented it to senior leadership in mid-October 2021. At the end of October 2021, FE-1 participated in a Company-wide all-hands meeting, with all of

Exicure's executives and staff, where it was decided that Corbett would review the XCUR-FXN project to figure out what had gone wrong. During the all-hands meeting, Mix and Daniel mentioned that they had already shown FE-1's deck to senior leadership.

- <u>Corbett Suddenly Departs in November 2021</u>: One week later, Corbett was gone. FE-1 explained that a Company-wide message advised that Corbett had resigned due to questions around XCUR-FXN that they had to investigate.
- <u>No "Second Body" Checked Corbett's Data</u>: Exicure had no "second body" in place to check Corbett's data, which FE-1 described as "unusual" in the pharmaceutical industry. Based on working at other drug companies, FE-1 explained: "Usually, they have that second person who was quality control for data." FE-1 pointed to a key deficiency in Exicure's processes and QC checks: not having someone check whether Corbett's data matched the original data from Charles River. FE-1 noted that after Corbett departed, Exicure hired a new person to be the "second body" and check the data going forward, and Mix and Daniel started comparing Corbett's data to the reports Charles River previously provided.
- Exicure Lacked Many Controls and SOPs: Even by fall 2021, Exicure's quality control personnel had not yet implemented many controls and SOPs (Standard Operating Procedures) designed to ensure that Exicure was properly handling data and would be prepared for an audit of its data. Shortly after the Company announced that Corbett had misreported data, the QC team was "issuing SOPs like gangbusters," FE-1 added. FE-1 knows this from receiving emails that required entering a separate database to read and sign SOPs.
- 261. FE-2 worked at Exicure from January 2019 to December 2021; from April 2020 to

December 2021, FE-2's title was Research Associate II on the Assay Development Team. FE-2 was based in Exicure's Chicago lab and reported to Aaron Einhorn, R&D Regulatory Affairs Program Manager. FE-2's role involved developing assays for Exicure's R&D Team to use as measuring techniques in their experiments. For several weeks in early 2021, FE-2 filled in for another lab technician and worked on Corbett's XCUR-FXN program, reporting to Corbett. According to FE-2, based on personal knowledge:

• <u>The R&D Department's *In Vivo* Mice Experiment in Late 2020 and Early 2021:</u> FE-2's work on the XCUR-FXN program involved measuring the concentration of total proteins and the concentration of frataxin levels in the brain and nervous system tissue of mice. These mice were part of the R&D Department's XCUR-FXN *in vivo* mice experiment conducted by a CRO, Charles River, in which the mice were divided into a control group and several treatment groups dosed with XCUR-FXN.

- <u>Charles River Provided Tissue Samples to Exicure for Analysis</u>: After the mice were dosed with XCUR-FXN, Charles River dissected the mice, and several slices of tissue were taken from different parts of each mouse's brain and dorsal root ganglia—specifically, the brainstem, cerebellum, cortex, and midbrain regions of the brain, and the cervical, thoracic, and lumbar regions of the dorsal root ganglia. Charles River then sent the tissue samples to Exicure to run additional tests on the mouse tissue.
- <u>FE-2 Measured Frataxin Concentration in Tissue Samples from the Mice</u>: FE-2 was assigned to measure proteins in samples extracted from the mouse tissue. FE-2 used assays to measure (1) the total concentration of all proteins and (2) the concentration of frataxin protein. The assay tests used chemical reactions that identified protein concentrations by color; the more intense the color, the higher the protein concentration. A machine called a spectrophotometer, or plate reader, measured the intensity of the colors in each sample to determine the protein concentration amount. FE-2 recalled that there were at least 400 samples, which took FE-2 two to three weeks to analyze.
- <u>FE-2 Transmitted the Data to Corbett</u>: The data from the mouse tissue was transmitted in three steps. First, the plate reader created and maintained a file with the results on its own system. Second, FE-2 used a flash drive to download a copy of the file from the plate reader and put the file on FE-2's computer. Third, FE-2 sent the results of the assay tests FE-2 ran on the mouse tissue to Corbett as an Excel file using Google Drive. FE-2 indicated that the Google Drive was shared by Exicure employees, including Corbett, and the data files on the Google Drive were accessible to most Exicure employees, including senior executives such as Giljohann, Schroff, Feltner, Daniel, and Anderson. FE-2 noted that the Google Drive indicated who last modified a document (and when), and that Excel files include metadata that shows the history of alterations to the spreadsheet.
- <u>Corbett Calculated the Purported Increase in Frataxin Levels</u>: FE-2 understood that Corbett used the assay results to calculate the percentage of frataxin compared to total proteins in each sample, then divided that figure by the frataxin percentage of the control group (called the "vehicle"). The result expressed the sample's frataxin concentration as "fold to vehicle"—for example, 2x "fold to vehicle" meant there was twice the frataxin concentration in the sample compared to the control. FE-2 recognized the graph (reported in Exicure's July 15, 2021 investor presentation, among other places) showing a 2-3x "fold to vehicle" frataxin increase in mice given XCUR-FXN *in vivo* as using the assay tests FE-2 conducted.
- <u>Prism Software Allowed Data Manipulation with a Real-Time Visual Result</u>: FE-2 explained that Exicure's R&D Department used a software system called Prism to generate presentation-ready graphs to illustrate the results of experiments. Based on using Prism, FE-2 explained that the software required users to manually

populate data fields that would be used to generate the type of graph requested. FE-2 and other Exicure employees typically cut and pasted data from an Excel spreadsheet and into the Prism template to make a graph. FE-2 suspected that Corbett manipulated the data in Prism to alter the graphs generated. As FE-2 explained, when data is entered or modified in Prism, the graph updates in real time, making it easier to manipulate data to achieve a desired visual result.

- Exicure's Senior Executives Attended Weekly Lab Meetings and Regular All-Hands Meetings: FE-2 stated that Exicure had weekly lab meetings each Friday where Exicure personnel presented data from the projects they were working on. Weston Daniel and Matthias Schroff consistently attended these lab meetings, while Defendant Giljohann sometimes attended. FE-2 recalled that Corbett's results on XCUR-FXN were presented at a lab meeting. In addition, Exicure held regular all-hands meetings via Zoom on Mondays. Giljohann led the all-hands meetings.
- <u>No Second Set of Eyes to Check Data</u>: FE-2 stated that there was no second set of eyes checking for accuracy on any data uploaded to the Google Drive; no one at Exicure checked to see if the data on protein concentrations that FE-2 uploaded to the Google Drive matched what the plate reader said. Nor did anyone check the data entered into Prism against the Excel spreadsheet data or the plate reader data to ensure it all matched. This state of affairs had existed since 2019, when FE-2 joined Exicure, and continued throughout 2021. "There wasn't anybody whose job it was to check data across different files and make sure they were correct," FE-2 said. "Thinking back now, it definitely seems like there should have been something in place."
- 262. FE-3 worked at Exicure from September 2016 to December 2021, starting as

Research Associate III, then becoming Research Associate IV; from June 2018 to December 2021,

FE-3 was Research and Development Manager, reporting to Bart Anderson, Senior Director of

R&D. FE-3 was a part of Exicure's early R&D effort that focused on finding sequences of

oligonucleotides that could be used to impact genetic expression in a way that could potentially

treat Friedreich's ataxia (FA). According to FE-3, based on personal knowledge:

 <u>The Screening Process Requires Reliable Assays</u>: In the case of FA, Exicure was looking for oligonucleotide sequences that would impact the genetic expression of FXN genes. This screening process requires assays that reveal if an oligonucleotide sequence is having an impact on genetic expression. FE-3 worked on finding and qualifying assays that could be used in the screening process to identify oligonucleotide sequences to treat FA. FE-3 explained that before an assay was used in the screening process, the lab had to run the assay through quality control tests to make sure it could accurately identify an oligonucleotide's impact on genetic expression of FXN genes.

- <u>FE-3 Tested Numerous Assays But Found No Reliable Assay for Upregulation</u>: To qualify assays, FE-3 put the assays through quality testing on a QuantStudio 12k machine using cells from a patient with FA. The machine generated copies of synthetic DNA (cDNA) from the cells and tested whether the assay bound to the cDNA. However, when FE-3 quality tested the assays for upregulation, none passed. FE-3 ran quality tests on multiple assays to screen for oligonucleotides that upregulate the targeted gene expression, but none could be qualified as reliable.
- <u>Corbett Claimed That an Unreliable Assay Qualified for Use</u>: At a weekly meeting of the R&D team (held on Fridays) in summer 2020, Corbett presented information claiming that one of the upregulation assays had been qualified for use in screening. The assay Corbett presented as qualified was an assay that failed FE-3's quality control tests. When FE-3 ran this particular assay through the quality control tests, it could not reliably determine that upregulation was occurring. "[Corbett] showed that the assay was qualified to use to screen," FE-3 said. "I didn't qualify that assay." But Corbett presented "it in a way that made it seem like it was working, but it wasn't."
- Anderson and Corbett Dismiss FE-3's Concerns and Use the Unreliable Assay: Shortly after hearing Corbett claim the assay was qualified during the weekly meeting, FE-3 went to FE-3's manager, Bart Anderson, and told him that the assay was not qualified and could not be used in the screening process. Anderson dismissed FE-3's concern and told FE-3 to raise the issue directly with Corbett. Next, FE-3 emailed Corbett stating that the assay he claimed was qualified had not been qualified to screen for oligonucleotides that caused upregulation. "I told Grant [Corbett] in the email he could not use the assay because it was not qualified," FE-3 said. Corbett did not respond to FE-3's email, but they met in person. During the meeting, Corbett dismissed FE-3's concerns and said he was analyzing the quality testing based on a published article about the assay. The meeting gave FE-3 the impression that Corbett was going to use the assay for screening for oligonucleotides that caused upregulation.
- <u>By Summer 2020, Corbett Had No Viable Candidate</u>: Shortly after Exicure moved into its new Chicago headquarters (in July 2020), Corbett told FE-3 that he did not have an oligonucleotide sequence to move forward into further study. "He said, I don't think we have anything. I don't think we are seeing anything that is worth (pushing forward). There is nothing there," FE-3 said. "He said he had to repeat (an) experiment." FE-3 added that Corbett "seemed very stressed" at the time of this conversation.
- <u>After Corbett Identifies XCUR-FXN, Giljohann Praises Him with a Lego Set</u>: Within a few months after the conversation above, Corbett was rewarded with a Lego set from CEO Giljohann for high-level job performance, as FE-3 learned from

a Company-wide announcement during a weekly "town hall" meeting on Monday morning. FE-3 explained that Giljohann had "an encouragement type of thing where he would give Legos to people who performed well." FE-3 believed that Corbett received his Lego set because he had selected a sequence that moved forward into further testing in the XCUR-FXN program.

• <u>Exicure Lacked Adequate Controls</u>: FE-3 recalled that there was no one at Exicure who checked Corbett's reports, presentations, and claims for accuracy against the raw data.

## C. Defendants Had Knowledge and Continuous Access to Reports Regarding the False XCUR-FXN Data

263. Throughout the Class Period, Defendants knew facts or had access to information indicating that their public statements were materially inaccurate.

264. <u>First</u>, Defendant Corbett knew the XCUR-FXN data was false because he personally fabricated it and made the false graphs in Prism, as detailed above.

265. <u>Second</u>, Defendants were regularly exposed to real-time results and data from the Company's projects. Exicure had weekly lab meetings each Friday where Exicure personnel presented data from the projects they were working on; Daniel and Schroff consistently attended, while Defendant Giljohann sometimes attended (FE-2). In addition, on Mondays, Exicure held regular all-hands Zoom meetings led by Defendant Giljohann (FE-2). Underscoring Defendants' ready access to information, Exicure was a small company, with only about 60 employees—50 of whom were in R&D—at the start of the Class Period.

266. <u>Third</u>, the raw data that contradicted Corbett's purported results was available to Defendants at all relevant times. The original data was stored on a Google Drive location that was shared by Exicure employees and accessible to Defendant Giljohann and other senior executives, such as Schroff, Feltner, Daniel, and Anderson (FE-2). Anyone who reviewed the original raw data would readily have seen that it did not match Corbett's doctored graphs. Indeed, the paper trail was so obvious that after Corbett departed, it took only a few weeks for outside counsel to

### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 101 of 122 PageID #:700

confirm that the data was falsified and had been misreported for months in the Company's SEC filings and investor presentations. The most reasonable inference is that Giljohann and Bock never checked the original data, demonstrating recklessness.

267. <u>Fourth</u>, FDA regulations confirm Defendants' access to the raw data. FDA regulations required a "final report" for each study that included, among other things, a "description of all circumstances that may have affected the quality or integrity of the data"; "[a] description of the transformations, calculations, or operations performed on the data, a summary and analysis of the data, and a statement of the conclusions drawn from the analysis"; and "[t]he locations where all specimens, raw data, and the final report are to be stored." 21 C.F.R. § 58.185(a). Exicure was also required to maintain "archives, limited to access by authorized personnel only, for the storage and retrieval of all raw data and specimens from completed studies." 21 C.F.R. § 58.51.<sup>8</sup>

268. <u>Finally</u>, as detailed above, by September 2021 Defendant Giljohann, at minimum, recklessly disregarded that Corbett's data was false: Giljohann personally attended two conference calls with Charles River in September 2021 where he learned that XCUR-FXN did not work and had no effect on frataxin levels, yet displayed alarming toxicity and killed a number of mice.

<sup>&</sup>lt;sup>8</sup> "Raw data" is defined as "any laboratory worksheets, records, memoranda, notes, or exact copies thereof, that are the result of original observations and activities of a nonclinical laboratory study and are necessary for the reconstruction and evaluation of the report of that study. In the event that exact transcripts of raw data have been prepared (e.g., tapes which have been transcribed verbatim, dated, and verified accurate by signature), the exact copy or exact transcript may be substituted for the original source as raw data. *Raw data* may include photographs, microfilm or microfiche copies, computer printouts, magnetic media, including dictated observations, and recorded data from automated instruments." 21 C.F.R. § 58.3(k).

## **D.** Core Operations Supports Scienter

269. As Exicure's flagship drug, XCUR-FXN constituted core operations of the Company. This further supports a strong inference that Defendants Giljohann and Bock knew or recklessly disregarded that Corbett's data on XCUR-FXN was false, and that the Company lacked effective controls to ensure reliable data and accurate SEC filings and disclosures.

270. CEO Giljohann hailed XCUR-FXN as the Company's "flagship program" in the "neurology space," and during the Class Period, Exicure claimed that it was advancing XCUR-FXN towards a human clinical trial in 2022.

271. XCUR-FXN was particularly important because Exicure had no drug that was approved and on the market during the Class Period. As the Company's SEC filings explained: "Our revenue sources are, and will remain, extremely limited unless and until our therapeutic candidates are clinically tested, approved for commercialization and successfully marketed."

272. The data concerning XCUR-FXN's efficacy was critical to obtain FDA approval, launch the drug, and begin generating revenue. Without *in vitro* data and *in vivo* animal data that showed an effective (and safe) drug, XCUR-FXN could not be tested on humans—much less obtain FDA approval for sale. The data supporting XCUR-FXN was so important to Exicure's financial prospects that it was the focus of two R&D Days in January and July 2021.

## E. As Exicure Burned Cash, Defendants Were Motivated to Lie to Secure New Funding

273. Exicure's tenuous financial position gave Defendants motive to lie. Since Exicure started trading on NASDAQ on July 31, 2019 (at \$2.63/share), its share price had steadily declined, and by December 2020 Exicure stock was trading below \$2.00 per share.

274. On December 31, 2020 (shortly before the start of the Class Period), Exicure had \$17.5 million in outstanding debt. At the same time, Exicure had only \$34.5 million in cash, cash

### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 103 of 122 PageID #:702

equivalents, and restricted cash, which were rapidly being depleted: in 2020 alone, Exicure spent \$42 million on research and development and general and administrative expense, but had just \$16.6 million in revenue, leading to a loss of about \$25 million. This cash burn was unsustainable. Thus, Exicure's Form 10-K for 2020 stated that Exicure "will need substantial additional funds to advance the development of our therapeutic candidates," and that Exicure "intend[ed] to do so through either collaborations, public or private equity offerings or debt financings, credit or loan facilities or a combination of one or more of these funding sources."

275. Meanwhile, Defendants had touted the prospect of a new FA drug starting in late 2019. This was crucial because Exicure's other drugs in development were yielding only tepid results: by January 2021, a study of XCUR-FXN's cancer drug candidate, cavrotolimod (AST-008), had generated responses in just 21% of patients—meaning that it did nothing to shrink tumors or lengthen survival in nearly four-fifths of the patients tested.

276. This made XCUR-FXN crucial to the Company's survival. Proving that XCUR-FXN worked was necessary for Exicure to attract new funding, partnership, and grants. Not only was XCUR-FXN itself a key source of future revenue, but it would purportedly validate Exicure's core SNA technology by providing "platform validation" for a pipeline of future drugs. For example, on March 11, 2021, a report by a BMO analyst stated: "We expect XCUR-FXN to provide platform validation in Friedreich's ataxia, opening up opportunity in other hereditary disorders."

277. By contrast, revealing that XCUR-FXN did not work would immediately call into question not only Exicure's research capabilities, but also the validity of the SNA technology underlying all of its purported drug candidates. This revelation would crater the Company's stock price and threaten its very existence—as in fact occurred at the end of the Class Period.

## F. Defendants Giljohann and Corbett Spoke Extensively about the False XCUR-FXN Data and Analysts Focused on It

278. Defendant Giljohann's and Corbett's repeated, specific representations about the

XCUR-FXN data, and the close attention analysts paid to XCUR-FXN, further support a strong

inference that Defendants had knowledge or recklessness at the time of their misstatements.

279. In numerous conference calls with investors and analysts during the Class Period,

Defendants Giljohann and Corbett specifically discussed the false data on XCUR-FXN.

- 280. For example, Giljohann falsely claimed:
  - "[W]e are able to dose dependently and consistently upregulate frataxin protein and frataxin mRNA" (January 11, 2021);
  - "[W]e've been able to show that our drugs work, both increasing the amount of frataxin protein and messenger RNA in cell lines that are derived from Friedreich's Ataxia patients" (March 9, 2021);
  - Exicure had achieved "what we believe is breakthrough efficacy in an *in vivo* Friedreich's Ataxia model" (July 15, 2021);
  - "[W]e're able to get into Friedreich's Ataxia patient cells *in vitro* and show a dose dependent upregulation of the relevant frataxin proteins," and had shown "a really nice increase" in animals, with "changes [that] are two to three-fold above the baseline" (September 13, 2021); and
  - "[W]e're getting a nice increase in the frataxin level after dosing," it "works in the animals," and "we're able to have an effect" (September 30, 2021).

281. Similarly, Corbett falsely claimed that "in vitro experiments" had "shown up-

regulation of mitochondria frataxin" (January 7, 2021).

282. Defendants Giljohann and Corbett specifically discussed the false data because XCUR-FXN's performance and prospects—as Exicure's lead drug—were a major driver of Exicure's share price. Analysts focused on the false data in their reports to investors. For example, a January 7, 2021 report by a Guggenheim analyst stated that "XCUR preclinical studies observed a 2-3x increase in FXN mRNA levels in vitro" (emphasis in original). A May 13,

2021 report by a Chardan analyst stated: "Preclinical data in a Pook800J mouse model demonstrated that . . . frataxin protein levels were increased by 2-fold and greater in the cerebellum and the cortex regions of the brain and spinal cord." A July 15, 2021 report by a BMO analyst stated that XCUR-FXN "continues to show promising preclinical data helping validate SNA target engagement with 2-3x frataxin upregulation in cerebellum/DRG regions and a solid safety profile."

## G. Exicure's Grossly Deficient Controls and Defendants' False Certifications That They Were "Effective" Support Scienter

283. The false statements that Exicure had "effective" disclosure controls, and the false certifications by Defendants Giljohann and Bock that they had designed such disclosure controls and procedures "to ensure that material information relating to" Exicure "is made known to us by others within" Exicure, further support their scienter.

284. The fact that a single Exicure scientist, purportedly acting "alone," inserted false data into nearly a year of Exicure's SEC filings and public presentations demonstrates a clear deficiency in Exicure's controls. Indeed, from at least 2019 and throughout the Class Period, Exicure had no controls to ensure reliable data, and no one checked that Exicure's public disclosures matched the actual data from experiments, contrary to the FDA requirement that an independent "quality assurance unit" at Exicure "assure . . . that the reported results accurately reflect the raw data." 21 C.F.R. § 58.35(b)(6).

285. Exicure's control failure before and throughout the Class Period was particularly notable because Anderson knew by summer 2020 that Corbett was using an unqualified, unreliable assay to screen oligonucleotides in the XCUR-FXN program, yet no one implemented the required controls in response.

286. Defendant Giljohann, in particular, knew about (or recklessly disregarded) the absence of controls that enabled Corbett's misconduct. Giljohann—a Ph.D scientist—joined

#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 106 of 122 PageID #:705

Exicure in 2011 and served as founding scientist in 2011, principal scientist from 2011 to 2012, and Chief Operating Officer from 2012 to 2013 before becoming CEO in November 2013. As a Ph.D. scientist with a decade of experience in senior scientific and executive positions at Exicure, who touted the "credibility that I bring as a scientist to the leadership role" and claimed that "we're a science-based company" and "the science starts at the top," Giljohann was aware of the FDA's requirements for data integrity and controls. Investors reasonably expected that a scientist like Giljohann at "the top" of a "science-based company" would comply with FDA requirements and establish and maintain effective controls to ensure that Exicure's public statements were truthful and based on reliable, accurate data.

287. Giljohann's further deception in response to discovering Corbett's false data confirms his scienter. As detailed above, Giljohann was told in September 2021 that XCUR-FXN was not raising frataxin levels and was killing mice, directly contrary to Corbett's purported results. In response, Giljohann not only failed to timely disclose the issue to investors, but he made further misstatements on September 30, 2021; reissued the false data in an October 13, 2021 investor presentation; and falsely certified on November 19, 2021 that Exicure had "effective" disclosure controls. These actions were, at minimum, reckless.

### H. Executive Terminations Support Scienter

288. The fact that the Defendants at the center of the fraud abruptly departed Exicure at the end of the Class Period further supports a strong inference of scienter.

289. Defendant Corbett's purported "resignation" followed months of mounting internal suspicion over the integrity of his data and results. As detailed above, by September 2021, Charles River had directly told CEO Giljohann and other senior executives that XCUR-FXN had no effect on frataxin levels and killed a number of mice, and by mid-October 2021, Charles River's final slide deck of data was presented to Exicure's senior leadership. Exicure's claim that Corbett

### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 107 of 122 PageID #:706

"voluntarily resigned" on November 8, 2021, and suddenly confessed as "part of his resignation" that he had "intentionally misreported certain raw data," omits this crucial context. The far more compelling inference is that, after months of internal knowledge that Corbett had fabricated XCUR-FXN data, Corbett was terminated.

290. Defendant Giljohann's departure also supports a strong inference of scienter. Exicure announced Giljohann's departure as CEO and Director on December 10, 2021 in the same Form 8-K that revealed that prior SEC filings, public presentations, and public statements by Exicure "management" had reported false data for over seven months. As detailed above, Giljohann personally made many of those misstatements, even after he was told twice in September 2021 that XCUR-FXN was not raising frataxin levels and was killing mice, directly contrary to Corbett's purported results. Giljohann also presided over the grossly deficient control environment that enabled Corbett's misconduct. And notably, the Form 8-K announcing Giljohann's departure did not include typical language stating that his departure was unrelated to any disagreement regarding the Company's operations, policies or practices. The most compelling inference is that Exicure's Board finally held Giljohann accountable for his misstatements and failures by terminating his employment.

## I. The Company's Self-Serving Statements Do Not Defeat the Strong Inference of Scienter

291. The strong inference of scienter is not defeated by Exicure's self-serving claims on December 10, 2021 that "Dr. Corbett acted alone in misreporting the data, without the assistance or knowledge of anyone else at the Company, including Company management and other research and development employees[,] and did not inform anyone at the Company of his actions until his resignation in November 2021," and that "Company management reasonably relied on Dr. Corbett's analysis when making public statements that included Dr. Corbett's misreported data."

#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 108 of 122 PageID #:707

292. The facts above demonstrate that these assertions are false. While the Company claimed that Corbett "acted alone" and that no one else, "including Company management and other research and development employees," had "knowledge" of the false data until November 2021, Anderson was told by summer 2020 that the assay Corbett intended to use was not qualified and could not be used in the screening process (FE-3). And in September 2021, Charles River—the laboratory handling mice experiments on XCUR-FXN—directly told CEO Giljohann (and other senior Exicure executives) on two conference calls of results that were starkly contrary to Corbett's data and Defendants' prior public statements.

293. Further, if true, Exicure's claim that no one other than Corbett had "knowledge" of the false data until November 2021 confirms that, in violation of FDA requirements, no one checked whether Exicure's SEC filings and presentations to investors matched the raw data from Corbett's experiments. This underscores that Defendants Giljohann and Bock were (at minimum) reckless. This material weakness in controls allowed false data and results to be included in Exicure's SEC filings and investor presentations for nearly a year.

294. Nonetheless, Giljohann and Bock falsely certified that Exicure had "effective" disclosure controls—including *after* Corbett admitted to falsifying data—and Giljohann personally touted false data that, according to Exicure, no one had checked for accuracy. Making these statements was an extreme departure from the standards of ordinary care because the danger of a misstatement was either known to Giljohann and Bock or so obvious that they must have been aware of it.

## J. Corporate Scienter

295. Exicure possessed scienter by virtue of the fact that Giljohann and Bock, corporate officers, had binding authority over the Company and acted with scienter, as set forth above.

#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 109 of 122 PageID #:708

296. In addition, certain allegations herein establish Exicure's corporate scienter independently of Giljohann's and Bock's scienter based on (i) the state of mind of senior executives whose intent can be imputed to the Company, and/or (ii) the knowledge of employees who approved the statements alleged herein despite knowing the statements' false and misleading nature. It can be strongly inferred that senior corporate executives at Exicure possessed scienter such that their intent can be imputed to the Company. Given the significance of XCUR-FXN to Exicure, the magnitude and duration of the misstatements, and the necessary involvement of multiple Exicure departments and personnel, additional executives unknown at this time and sufficiently senior to impute their scienter to Exicure also knew of the fraudulent scheme alleged herein.

297. As-yet-unidentified Exicure employees also approved the false statements despite knowing of their false and misleading nature. As discussed, XCUR-FXN was highly significant to Exicure and a focus of senior management's public statements, the XCUR-FXN program was initially approved based on data from an assay known at the time to be unreliable and unqualified, and the original raw data showing that Defendants' public statements were false was readily available within Exicure. At the same time, the false data and false graphs were repeatedly included in Exicure's SEC filings and investor presentations, including in an investor presentation posted to Exicure's website on October 13, 2021—several weeks *after* Charles River twice reported to CEO Giljohann and other senior executives that XCUR-FXN had no effect on frataxin levels and was killing a number of mice. From this, it can be strongly inferred that senior Exicure executives approved of the false and misleading statements in Exicure's SEC filings and investor presentations.

### VII. LOSS CAUSATION

298. Defendants' fraudulent conduct directly and proximately caused Plaintiff and the Class to suffer substantial losses as a result of purchasing or otherwise acquiring Exicure stock at artificially inflated prices during the Class Period.

299. Through their materially false and misleading statements set forth above, Defendants concealed the truth that XCUR-FXN did not work, that the data and results they publicly reported were false, and that Exicure had grossly deficient controls that enabled the fabrication and falsification of data and results.

300. When the false and misleading nature of Defendants' statements became known to the market in piecemeal fashion, as alleged herein, the price of Exicure stock substantially declined. Specifically:

## A. Exicure First Reveals "Alleged Improprieties" in XCUR-FXN Research and Its Stock Plummets 38.5%

301. On November 15, 2021, Exicure filed a Form 12b-25 stating that Exicure would miss the filing deadline for its Form 10-Q for the third quarter of 2021, and revealing that: "On November 9, 2021, the Audit Committee of the Board of Directors of the Company was notified of a claim made by a former Company senior researcher regarding alleged improprieties that researcher claims to have committed with respect to the Company's XCUR-FXN preclinical program for the treatment of Friedreich's ataxia. The Audit Committee has retained external counsel to conduct an internal investigation of the claim."

302. Following this disclosure, the price of Exicure stock plummeted 38.5%. On November 16, 2021 alone, its price declined by 27.38% (from \$1.07 to \$0.777) on unusually heavy trading volume. As investors continued to digest the information, on November 17, 2021, Exicure

stock dropped another 8.62% (from \$0.777 to \$0.71), followed by another 7.32% drop on November 18, 2021 (from \$0.71 to \$0.658), all on unusually heavy trading volume.

303. While this disclosure began to reveal Corbett's misconduct, it characterized the misconduct as "alleged" and did not detail the misconduct's scope, that Corbett acted intentionally, or that the misreported data had impacted the Company's prior SEC filings and public statements.

## B. Exicure States That It Is "Unable to Determine" the False Data's Impact on R&D for XCUR-FXN; the Stock Drops 30%

304. On November 19, 2021, Exicure filed its Form 10-Q for the third quarter of 2021, which included a new disclosure that Exicure was "unable to determine the potential impact" of the "alleged improprieties" on its "research and development activities or the timing of completion of our current research and development of our XCUR-FXN preclinical program for the treatment of FA." This new disclosure ominously signaled that the "alleged improprieties" might affect the "completion" of R&D on XCUR-FXN and Exicure's other "research and development activities."

305. In the Company's accompanying press release, dated November 19, 2021, CEO Giljohann—despite having known for months that the XCUR-FXN data was false—sought to blunt the impact of Corbett's misconduct, claiming that "our SNA platform is grounded in 15 years of intensive and rigorous scientific development" and that "we are highly confident in our platform technology which delivers DNA and RNA into cells and tissues more effectively."

306. The market disagreed: on November 19, 2021, Exicure's stock dropped 30.09%, from \$0.66 to \$0.46, on unusually heavy trading volume.

307. Analysts slashed their value of the Company. For example, a November 23, 2021 report by a Chardan analyst stated: "After speaking to Management about the recently disclosed possible preclinical data *improprieties* for the company's XCUR-FXN program for Friedreich's Ataxia, we are lowering our price target from \$7 to \$2.50." (Italics in original.) The report

107

continued: "We anticipate delays in advancing XCUR-FXN into the clinic . . . . The company was on track to file an IND for XCUR-FXN by year-end 2021, but has now withdrawn that guidance. In our model we have pushed out entry into the clinic for this program into 1Q23."

# C. Exicure Finally Admits That It Reported False Data from "at Least Three Different Experiments" for Over Seven Months and Abandons XCUR-FXN; the Stock Plunges 46%

308. On Friday, December 10, 2021, Exicure filed a Form 8-K reporting the results of

the internal investigation. The Form 8-K stated:

The Audit Committee and the Company investigated statements made by Dr. Grant Corbett, the Company's former Group Leader of Neuroscience. Dr. Corbett voluntarily resigned from the Company on November 8, 2021. As part of his resignation, he claimed that when he was employed by the Company, he *intentionally misreported* certain raw data related to the research and development of XCUR FXN. The investigation began promptly after the receipt of Dr. Corbett's resignation and allegations and was substantially completed in early December 2021. The Audit Committee provided outside counsel with significant resources, without imposing limitations on the investigation's scope, timing or access to information. The investigation involved collection and review of a significant number of documents[,] communications and data, and interviews of numerous witnesses. Dr. Corbett was also interviewed during the investigation.

The investigation revealed that: (1) beginning in the autumn of 2020, Dr. Corbett misreported raw data from certain research and development experiments related to XCUR-FXN; (2) Dr. Corbett misreported the results of at least three different experiments that were conducted through at least February 2021; (3) the misreported data related solely to efficacy rather than safety of XCUR-FXN; (4) the misreported data was included in various public presentations and SEC filings from as early as January 7, 2021 through as late as August 12, 2021; (5) Dr. Corbett acted alone in misreporting the data, without the assistance or knowledge of anyone else at the Company, including Company management and other research and development employees and did not inform anyone at the Company of his actions until his resignation in November 2021; (6) Company management reasonably relied on Dr. Corbett's analysis when making public statements that included Dr. Corbett's misreported data; and (7) no other Company program was impacted by Dr. Corbett's misreporting of the XCUR-FXN data.

The Board and the Audit Committee have begun a process with the assistance of counsel to address the results of the investigation. The Board and the Audit Committee also intend to enhance the Company's policies and procedures regarding data management and integrity.

#### Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 113 of 122 PageID #:712

309. This was the first identification of Corbett by name. It was also the first disclosure that Corbett had in fact "misreported" the data and had done so "intentionally"; that the false data concerned XCUR-FXN's "efficacy"; that the false data related to "at least three different experiments that were conducted through at least February 2021"; that the false data had impacted over seven months of Exicure's public presentations and SEC filings, as well as "public statements" by "Company management"; and that "the Company's policies and procedures regarding data management and integrity" needed to be changed.

310. The same Form 8-K simultaneously announced (1) CEO Giljohann's departure, effective immediately; (2) that Chief Medical Officer Feltner was departing on January 30, 2022; that Exicure was abandoning XCUR-FXN; and (3) that Exicure "will eliminate approximately 50% of the Company's existing workforce on a staggered basis through January 2022."

311. When the market opened on Monday morning, Exicure's stock plunged 40.68%, from \$0.4553 to \$0.2701, on December 13, 2021, and dropped another 6.28% (from \$0.2701 to \$0.2531) on December 14, 2021.

312. Analysts immediately recognized that without XCUR-FXN, and with no ongoing R&D, Exicure stock was effectively worthless. A December 13, 2021 report by a Chardan analyst reduced its price target from \$2.25 to \$0.40 and linked the layoffs and abandonment of XCUR-FXN to Corbett's misreporting, stating: "The fallout from this situation has resulted in the company being forced to take a number of steps to preserve cash" (citing 50% layoffs and stopping XCUR-FXN and cavrotolimod development). Other analysts quickly discontinued coverage, with a Guggenheim analyst stating on December 14, 2021: "We are terminating coverage of Exicure. Inc. (XCUR) due to the company's recent change in therapeutic focus (discontinuation of the key value-driving neurology FXN and oncology cavro programs, see HERE)."

#### VIII. PRESUMPTION OF RELIANCE

313. Plaintiff and the Class are entitled to a presumption of reliance on Defendants' material misrepresentations pursuant to the fraud-on-the-market doctrine. At all relevant times, the market for Exicure common stock was an efficient market for the following reasons, among others:

- A. Exicure common stock met the requirements for listing, and was listed and actively traded on the NASDAQ, a highly efficient and automated market;
- B. As a regulated issuer, Exicure filed periodic public reports with the SEC;
- C. Exicure regularly and publicly communicated with investors via established market communication mechanisms, including through regular disseminations of press releases on the national circuits of major newswire services, through investor presentations published on Exicure's website, and through other wide-ranging public disclosures, such as communications with the financial press and other similar reporting services; and
- D. Exicure was followed by multiple securities analysts employed by major brokerage firms who wrote reports which were distributed to the sales force and certain customers of their respective brokerage firms. Each of these reports was publicly available and entered the public marketplace.

314. As a result of the foregoing, the market for Exicure common stock promptly digested current information regarding Exicure from all publicly available sources and reflected such information in the price. Under these circumstances, all purchasers of Exicure common stock during the Class Period suffered similar injury through their purchase of Exicure common stock at artificially inflated prices and the presumption of reliance applies.

# IX. CLASS ACTION ALLEGATIONS

315. Plaintiff brings this action as a class action pursuant to Rule 23 of the Federal Rules of Civil Procedure on behalf of all persons who (i) purchased or otherwise acquired Exicure's common stock during the Class Period of January 7, 2021 through December 10, 2021, both inclusive (the "Class"). Excluded from the Class are Defendants and their families, directors, and officers of Exicure and their families and affiliates.

316. The members of the Class are so numerous that joinder of all members is impracticable. The disposition of their claims in a class action will provide substantial benefits to the parties and the Court. As of May 26, 2023, there were millions of shares of Exicure common stock outstanding, owned by at least thousands of investors.

317. There is a well-defined community of interest in the questions of law and fact involved in this case. Questions of law and fact common to the members of the Class which predominate over questions which may affect individual Class members include:

- A. Whether Defendants violated the Exchange Act;
- B. Whether Defendants misrepresented material facts;
- C. Whether Defendants' statements omitted material facts necessary in order to make the statements made, in light of the circumstances under which they were made, not misleading;
- D. Whether Defendants knew or recklessly disregarded that their statements were false and misleading;
- E. Whether the prices of Exicure common stock were artificially inflated;
- F. Whether Defendants' conduct caused the members of the Class to sustain damages; and

G. The extent of damage sustained by Class members and the appropriate measure of damages.

318. Plaintiff's claims are typical of those of the Class because Plaintiff and the Class sustained damages from Defendants' wrongful conduct.

319. Plaintiff will adequately protect the interests of the Class and has retained counsel experienced in class action securities litigation. Plaintiff has no interests which conflict with those of the Class.

320. A class action is superior to other available methods for the fair and efficient adjudication of this controversy.

## X. INAPPLICABILITY OF STATUTORY SAFE HARBOR

321. The statutory safe harbor and bespeaks caution doctrine applicable to forwardlooking statements under certain circumstances do not apply to any of the untrue or misleading statements alleged herein. The statements complained of herein concerned then-present or historical facts or conditions that existed or were purported to exist at the time the statements were made, such as the purported data and results of research Exicure had already conducted.

322. To the extent any of the untrue or misleading statements alleged herein can be construed as forward-looking, Exicure's accompanying "Safe Harbor" warnings issued during the Class Period were ineffective and inapplicable and cannot shield the statements at issue from liability.

323. Defendants are also liable for any false or misleading forward-looking statements pleaded herein because, at the time each such statement was made, the speaker knew the statement was false or misleading and the statement was made by or authorized and/or approved by an executive officer of Exicure who knew that the statement was false.

112

### XI. CLAIMS FOR RELIEF

#### COUNT I

### Section 10(b) of the Exchange Act and Rule 10b-5(b) (Against All Defendants)

324. Plaintiff repeats, incorporates, and re-alleges each and every allegation contained above as if fully set forth herein.

325. During the Class Period, Defendants made, disseminated or approved the false and misleading statements specified above, which they knew or recklessly disregarded were false and misleading in that the statements contained material misrepresentations and failed to disclose material facts necessary in order to make the statements made, in light of the circumstances under which they were made, not misleading. Defendants are sued as primary participants in the wrongful and illegal conduct charged herein and/or as controlling persons, as alleged above and below.

326. Defendants violated Section 10(b) of the Exchange Act and Rule 10b-5(b) thereunder in that they made untrue statements of material fact or omitted to state material facts necessary in order to make the statements made, in light of the circumstances under which they were made, not misleading.

327. Plaintiff and the Class have suffered damages in that, in reliance on the integrity of the market, they paid artificially inflated prices for Exicure common stock. Plaintiff and the Class would not have purchased Exicure common stock at market prices, or at all, if they had been aware that the market prices of Exicure common stock were artificially inflated and distorted by Defendants' false and misleading statements.

113

328. As a direct and proximate result of Defendants' wrongful conduct, Plaintiff and the Class suffered damages in connection with their purchases of Exicure common stock during the Class Period.

## COUNT II

# Section 10(b) of the Exchange Act and Rule 10b-5(a) and (c) (Against Defendants Corbett and Giljohann)

329. Plaintiff repeats, incorporates, and re-alleges each and every allegation contained above as if fully set forth herein.

330. During the Class Period, Defendants Corbett and Giljohann engaged in a scheme to fabricate and misreport data on XCUR-FXN, including through Corbett's creation of false data and creation of false graphs misstating the results of four experiments and Defendant Giljohann's approval of the false data and false graphs, and presentations that contained them, for dissemination to investors. These actions constituted devices, schemes, and artifices to defraud, and acts, practices and a course of business that operated as a fraud or deceit upon Plaintiff and the Class. In addition (though not required), Corbett's and Giljohann's scheme went beyond making and disseminating misrepresentations because Corbett's fabrication of false data and creation of false graphs and Giljohann's approval of them were inherently deceptive and fraudulent acts. Defendants Corbett and Giljohann are sued as primary participants in the wrongful and illegal conduct charged herein, as alleged above and below.

331. Defendants Corbett and Giljohann violated Section 10(b) of the Exchange Act andRule 10b-5(a) and (c) thereunder in that they:

- a) Employed devices, schemes, and artifices to defraud; and/or
- c) Engaged in acts, practices and a course of business that operated as a fraud or deceit upon Plaintiff and others similarly situated in connection with their purchases of Exicure common stock during the Class Period.

332. Plaintiff and the Class have suffered damages in that, in reliance on the integrity of the market, they paid artificially inflated prices for Exicure common stock. Plaintiff and the Class would not have purchased Exicure common stock at market prices, or at all, if they had been aware that the market prices of Exicure common stock were artificially inflated and distorted by Defendant Corbett's and Giljohann's fraudulent conduct.

333. As a direct and proximate result of Defendant Corbett's and Giljohann's wrongful conduct, Plaintiff and the Class suffered damages in connection with their purchases of Exicure common stock during the Class Period.

## **COUNT III**

# Section 20(a) of the Exchange Act (Against Defendants Giljohann and Bock)

334. Plaintiff repeats, incorporates, and re-alleges each and every allegation set forth above as if fully set forth herein.

335. Defendants Giljohann and Bock acted as controlling persons of Exicure within the meaning of Section 20(a) of the Exchange Act as alleged herein. By virtue of their high-level positions, participation in and/or awareness of the Company's operations, direct involvement in the day-to day operations of the Company, and/or intimate knowledge of the Company's actual performance, and their power to control Exicure and the public statements about Exicure, Defendants Giljohann and Bock had the power and ability to control the actions of Exicure and its employees. By reason of such conduct, Defendants Giljohann and Bock are liable pursuant to Section 20(a) of the Exchange Act.

#### XII. JURY DEMAND

336. Plaintiff, on behalf of himself and the Class, demands a jury trial.

# XIII. PRAYER FOR RELIEF

- 337. WHEREFORE, Plaintiff prays for judgment as follows:
  - A. Determining that this action is a proper class action under Rule 23 of the Federal Rules of Civil Procedure;
  - B. Awarding compensatory damages in favor of Plaintiff and other Class members against all Defendants, jointly and severally, for all damages sustained as a result of Defendants' wrongdoing, in an amount to be proven at trial, including interest thereon;
  - C. Awarding Plaintiff and the Class their reasonable costs and expenses incurred in this action, including attorneys' fees and expert fees; and
  - D. Awarding such equitable/injunctive or other further relief as the Court may deem just and proper.

Dated: May 26, 2023

Respectfully submitted,

<u>/s/ Evan A. Kubota</u> Joseph A. Fonti (*pro hac vice* forthcoming) Evan A. Kubota (*pro hac vice*) **BLEICHMAR FONTI & AULD LLP** 7 Times Square, 27th Floor New York, New York 10036 Telephone: (212) 789-1340 Facsimile: (212) 205-3960 jfonti@bfalaw.com ekubota@bfalaw.com

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Additional Counsel for Lead Plaintiff James Mathew

Case: 1:21-cv-06637 Document #: 61 Filed: 05/26/23 Page 122 of 122 PageID #:721

## **CERTIFICATE OF SERVICE**

I hereby certify that on May 26, 2023, a copy of the foregoing was filed electronically with the Clerk of Court via CM/ECF. Notice of this filing will be sent by email to all parties by operation of the Court's electronic filing system or by mail to anyone unable to accept electronic filing. Parties may access this filing through the court's CM/ECF system.

/s/ Evan A. Kubota

Evan A. Kubota