

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION**  
Washington, D.C. 20549

**FORM 8-K**

**CURRENT REPORT**  
**Pursuant to Section 13 or 15(d)**  
**of the Securities Exchange Act of 1934**

**Date of Report (Date of earliest event reported): March 19, 2018**

**Eloxx Pharmaceuticals, Inc.**

(Exact name of registrant as specified in its charter)

**Delaware**  
(State or other jurisdiction  
of incorporation)

**950 Winter Street**  
**Waltham, MA**  
(Address of principal executive offices)

**001-31326**  
(Commission  
File Number)

**84-1368850**  
(IRS Employer  
Identification No.)

**02451**  
(Zip Code)

**Registrant's telephone number, including area code: (781) 577-5300**

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligations of the registrant under any of the following provisions:

- ☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- ☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- ☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- ☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

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**Item 2.02            Results of Operations and Financial Condition.**

On March 19, 2018, Eloxx Pharmaceuticals, Inc. (the “Company”) issued a press release announcing its financial results for the fiscal fourth quarter and fiscal year ended December 31, 2017. A copy of the Company’s press release is furnished as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated herein by reference.

The information in this Current Report on Form 8-K, including the information contained in the press release furnished as Exhibit 99.1, shall not be deemed “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or the Exchange Act, or otherwise subject to the liabilities of that section, and shall not be deemed incorporated by reference into any of the Company’s filings under the Securities Act of 1933, as amended or the Exchange Act, whether made before or after the date hereof, regardless of any general incorporation language in such filing, except as shall be expressly set forth by specific reference in such filing.

**Item 7.01            Regulation FD Disclosure.**

On March 19, 2018, the Company released an updated investor presentation, which will be used by the Company from time to time, including in meetings with investors.

A copy of the above-referenced presentation is furnished as Exhibit 99.2 to this Current Report on Form 8-K. The information furnished pursuant to Item 7.01 of this current report, including Exhibit 99.2, shall not be deemed to be “filed” for purposes of Section 18 of the Exchange Act. As such, this information shall not be incorporated by reference into any of the Company’s reports or other filings made with the U.S. Securities and Exchange Commission. The furnishing of the information in this current report is not intended to, and does not, constitute a determination or admission by the Company that the information in this current report is material or complete, or that investors should consider this information before making an investment decision with respect to any security of the Company.

**Item 9.01            Financial Statements and Exhibits.**

(d) Exhibits

<u>Exhibit No.</u>	<u>Description</u>
99.1	<a href="#"><u>Press Release of the Company dated March 19, 2018.</u></a>
99.2	<a href="#"><u>Presentation of the Company dated March 20, 2018.</u></a>

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**SIGNATURES**

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

**ELOXX PHARMACEUTICALS, INC.**

Date: March 19, 2018

By: /s/ Gregory Weaver  
Gregory Weaver  
Chief Financial Officer



## Eloxx Pharmaceuticals Reports Full Year 2017 Financial and Operating Results and Provides Business Update

*Company to host webcast and conference call on Tuesday, March 20, 2018 at 8 am ET Management to discuss recent data and progress on development plans*

**Waltham, MA.** – March 19, 2018 – Eloxx Pharmaceuticals, Inc. (“Eloxx”), (ELOX) a clinical-stage biopharmaceutical company dedicated to the discovery and development of novel therapeutics to treat cystic fibrosis, cystinosis and other diseases caused by nonsense mutations limiting production of functional proteins, today reported its financial results for the twelve months ended December 31, 2017, and provided an update on its clinical development plans.

“Given the clinical accomplishments in 2017 for our lead product candidate, ELX-02, we are poised to seek regulatory clearance to initiate Phase 2 clinical trials in cystic fibrosis and cystinosis this year,” said Robert E. Ward, Chairman and CEO of Eloxx Pharmaceuticals. “Patients with nonsense mutations have a high burden of disease, and have few, if any, treatment options available. In cystic fibrosis, therapies to address the needs of patients with nonsense mutations have the potential to lead to the next breakthrough in treating this disease state. We are committed to bringing forward therapeutic options which have the potential to transform lives.”

### ELX-02 in Cystic Fibrosis and Cystinosis

ELX-02 is our lead asset and our current development programs are focused on cystic fibrosis and cystinosis patients with diagnosed nonsense mutations. To advance the program, we have held pre-clinical trial application (CTA) discussions with the Federal Agency for Medicines and Health Products (FAMHP) in Brussels, Belgium and pre-IND discussions with the U.S. Food & Drug Administration (FDA) for cystic fibrosis and cystinosis, respectively.

Currently, the European Medicines Agency (EMA) has designated ELX-02 as an orphan medicine for the treatment of mucopolysaccharidosis type I (MPS I), and the FDA has granted orphan drug designation to ELX-02 for the treatment of MPS I and for the treatment of Rett Syndrome.

- We are on-track for an expected mid-2018 submission of our CTA in Belgium for cystic fibrosis.

- We are on track for an expected mid-2018 submission of our IND for cystinosis.
- We expect to initiate Phase 2 studies in cystic fibrosis and cystinosis, by the end of 2018, subject to regulatory review and clearance of our CTA and IND, respectively.

As part of our clinical program, we have completed a Phase 1 single ascending dose (SAD) study in a total of 60 healthy volunteers at sites in Israel (ClinicalTrials.gov Identifier: NCT02807961) and Belgium (ClinicalTrials.gov Identifier: NCT03292302). Currently ongoing is the Phase 1 multiple ascending dose (MAD) study in 45 healthy volunteers in Belgium (ClinicalTrials.gov Identifier: NCT03309605).

- We anticipate that the Phase 1 MAD study will be completed in 2018. The results from the completed Phase 1 SAD study will be included in the planned IND and CTA submissions.

#### **Full Year 2017 Financial Results**

As of December 31, 2017, we had cash and cash equivalents of \$24.0 million, which we expect to fund operations at least through the end of the first quarter of 2019 based on our current operating plans. We received net proceeds of \$16.8 million in Q4 2017 related to completing our Series C financing.

We incurred a loss for the year ended December 31, 2017 of \$21.2 million. Our research and development expenses were \$16.4 million for the year ended December 31, 2017, compared to \$9.0 million for the year ended December 31, 2016, an increase of \$7.4 million due to a licensing fee and growth in clinical development.

Our general and administrative expenses were approximately \$4.0 million for the year ended December 31, 2017 compared to approximately \$0.8 million for the year ended December 31, 2016, an increase of approximately \$3.2 million. The increase in our general and administrative expenses was primarily related to an increase in our headcount and related salaries, stock-based compensation, other personnel related costs, and costs related to opening our HQ office in Waltham, MA.

#### **Fourth Quarter 2017 Financial Results**

As of September 30, 2017, we reported cash of \$13.5 million. For the three months ended December 31, 2017, our use of cash in operations was \$6.3 million. For the three months ended December 31, 2017, our research and development expense totaled \$8.4 million, which included \$3.4 million in non-cash expense. For the three months ended December 31, 2017, our general & administrative expense totaled \$2.2 million. The Company recorded a net loss of \$10.6 million for the three months ended December 31, 2017.

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**Conference Call Information:**

**Date:** Tuesday, March 20, 2018

**Time:** 8:00 a.m. ET

**Domestic Dial-in Number:** (866) 913-8546

**International Dial-in Number:** (210) 874-7715

**Conference ID:** 3875349

**Live Webcast:** accessible from the Company's website at [www.eloxxpharma.com](http://www.eloxxpharma.com) or with this link <https://edge.media-server.com/m6/p/w36bkfpa>

A replay will be available on the Company's website approximately two hours after the call.

**About Eloxx Pharmaceuticals**

Eloxx Pharmaceuticals, Inc. is a clinical-stage biopharmaceutical company developing novel RNA-modulating drug candidates that are designed to treat rare and ultra-rare premature stop codon diseases. Premature stop codons are point mutations that disrupt protein synthesis from messenger RNA. As a consequence, patients with premature stop codon diseases have reduced or eliminated protein production from the mutation bearing allele accounting for some of the most severe phenotypes in these genetic diseases. These premature stop codons have been identified in over 1,800 rare and ultra-rare diseases. Read-through therapeutic development is focused on extending mRNA half-life and increasing protein synthesis by enabling the cytoplasmic ribosome to read through premature stop codons to produce full-length proteins. Eloxx's lead product candidate, ELX-02, is a small molecule drug candidate designed to restore production of full-length functional proteins. ELX-02 is in the early stages of clinical development focusing on cystic fibrosis and cystinosis. ELX-02 is an investigational drug that has not been approved by any global regulatory body. Eloxx is headquartered in Waltham, MA, with R&D operations in Rehovot, Israel.

**Forward-Looking Statements**

Certain statements included in this press release are forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995 and involve a number of risks and uncertainties. These include statements of management's intentions, belief, plans and future expectations and, therefore, you are cautioned not to place undue reliance on them. Such forward-looking statements involve risks and uncertainties and actual results could differ materially from any forward-looking statements expressed or implied herein. The risks and uncertainties that could result in actual results to differ materially from those forward-looking statements express or implied herein include, but are not limited to: the Company's ability to continue as a going concern; the ability of the Company to consummate additional financings; the development of the Company's technology; the

approval of the Company’s patent applications; the Company’s ability to successfully defend its intellectual property or obtain the necessary licenses at a cost acceptable to the Company, if at all; the successful implementation of the Company’s research and development programs and collaborations; the success of the Company’s license agreements; the acceptance by the market of the Company’s products if approved; the timing and success of the Company’s preliminary studies, preclinical research, clinical trials and related regulatory filings; and the continued quotation of the Company’s common stock on the over-the-counter securities market, as well as other factors expressed from time to time in the Company’s 10-K, 10-Qs and other filings with the U.S. Securities and Exchange Commission (the “SEC”). As a result, this press release should be read in conjunction with the Company’s periodic filings with the SEC. The forward-looking statements contained herein are made only as of the date of this press release, and the Company undertakes no obligation to publicly update or revise such forward-looking statements to reflect subsequent events or circumstances.

Contact:

Barbara Ryan  
203-274-2825  
*barbarar@eloxpharma.com*

ELOXX PHARMACEUTICALS, INC.

CONSOLIDATED BALANCE SHEETS  
Amounts in thousands, except share and per share data

	December 31,	
	2017	2016
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 24,049	\$ 2,212
Restricted bank deposit	102	38
Prepays and other current assets	355	837
Total current assets	24,506	3,087
Property and equipment, net	278	41
Total assets	24,784	3,128
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities		
Accounts payables	1,530	1,899
Accrued expenses	1,893	619
Total current liabilities	3,423	2,518
Commitments and contingencies (Note 9)		
Stockholders' equity:		
Series A, B-1, B-2 and C Preferred Stock, \$0.01 par value 5,000,000 and 19,965,708 shares authorized as of December 31, 2017 and 2016, respectively; 0 and 7,638,263 shares issued and outstanding as of December 31, 2017 and 2016, respectively	—	76
Common stock, \$0.01 par value 500,000,000 and 29,948,562 shares authorized as of December 31, 2017 and 2016, respectively; 27,527,738 and 4,205,278 shares issued and outstanding as of December 31, 2017 and 2016, respectively	274	42
Additional paid-in capital	60,047	18,238
Accumulated deficit	(38,960)	(17,746)
Total stockholders' equity	21,361	610
Total liabilities and stockholders' equity	\$ 24,784	\$ 3,128



ELOXX PHARMACEUTICALS, INC.

CONSOLIDATED STATEMENTS OF OPERATIONS  
Amounts in thousands, except per share and per share data

		Year ended December 31,	
	2017	2016	2015
Operating expenses:			
Research and development, net	\$ 16,398	\$ 8,986	\$ 5,842
General and administrative expenses	3,992	854	442
Total operating expenses	20,390	9,840	6,284
Loss from operations	(20,390)	(9,840)	(6,284)
Financial and other expenses, net	824	7	122
Net loss	\$ 21,214	\$ 9,847	\$ 6,406
Basic and diluted net loss per share	\$ 4.75	\$ 2.60	\$ 1.67
Weighted average number of Common Stock used in computing basic and diluted loss per share	4,976,377	4,205,277	4,148,389



Unlocking protein production with **translational read-through** for **rare genetic** diseases

**FY2017 Webcast & Conference Call March 20, 2018**

# Forward-Looking Statements

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The risks and uncertainties that could result in actual results to differ materially from those forward-looking statements expressed or implied herein include, but are not limited to: the Company's ability to continue as a going concern; the ability of the Company to consummate additional financings; the development of the Company's technology; the approval of the Company's patent applications; the Company's ability to successfully defend its intellectual property or obtain the necessary licenses at a cost acceptable to the Company, if at all; the successful implementation of the Company's research and development programs and collaborations; the success of the Company's license agreements; the timing and success of the Company's preliminary studies, preclinical research, clinical trials and related regulatory filings; if approved, the acceptance by the market of the Company's products; and the continued quotation of the Company's common stock on the over-the-counter securities market, as well as other factors expressed from time to time in the Company's 10-K, 10-Qs and other filings with the SEC. The forward-looking statements contained herein are made only as of the date of this presentation, and the Company undertakes no obligation to publicly update such forward-looking statements to reflect subsequent events or circumstances.

# Eloxx Pharmaceuticals Highlights

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- **Key positive organoid data in Cystic Fibrosis**
  - Heterozygous and homozygous CFTR mutations
- **Key positive model data in Cystinosis**
  - Reduction of kidney cystine levels
- **On track for completion of Phase 1 studies**
  - SAD completed
  - MAD enrolling
- **Initiation of Phase 2 studies in Cystic Fibrosis and Cystinosis (4 Q)**
- **Participation at Key Scientific Conferences**
- **Eloxx to nominate second novel molecule for development in rare/ultra-rare orphan disease**



# The Promise of Read-Through

**>1,800**

**Genetic diseases involve nonsense mutations**



Cystic Fibrosis



Cystinosis



MPS I Syndrome



Rett Syndrome



Duchenne Muscular Dystrophy

- In every genetic disease a subset of patients have nonsense mutations that impair the production of essential proteins
- Translational read through is directed at restoring the production of full length proteins by overcoming the premature stop codon and nonsense mediated decay

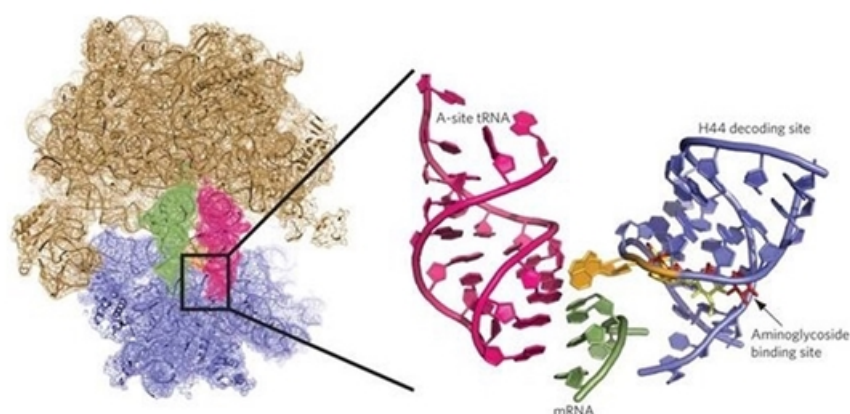
**Advances in our understanding of translational read-through has enabled design of novel small molecules**

Built upon a molecular scaffold with a defined ribosomal effect

Active at all three premature stop codons

Potential to achieve clinically meaningful restoration of functional essential protein

# Aminoglycoside Ribosomal Interaction



- Well defined molecular interaction with helix 44
- Role in stabilization of tRNA binding at Site A
- Optimizing scaffold by altering interaction with prokaryotic and mitochondrial ribosomes
- Defined tissue penetration and tolerability profile for read-through applications

Aminoglycoside activity observed on single pre-translocation ribosome complexes. Feldman, MB; Terry DS; Altman RB; Blanchard SC. Nature Chemical Biology volume6, pages54–62 (2010)



## ELX-02 Clinical Development – Phase 1 Studies

CLINICALTRIALS.GOV

**Identifier: NCT03292302**

A Phase 1a, Randomized, Double-blinded, Placebo-Controlled, Single Dose Escalation Study to Evaluate the Safety, Tolerability, and Pharmacokinetics of ELX-02 in Healthy Adult Volunteers

COMPLETED



CLINICALTRIALS.GOV

**Identifier: NCT03309605**

A Phase 1, Randomized, Double-Blinded, Placebo-Controlled, Third Party Open, Multiple Dose Escalation, Single Center Study to Evaluate the Safety, Tolerability and Pharmacokinetics of Subcutaneously Administered ELX-02 in Independent Consecutive Cohorts of Healthy Subjects

ONGOING



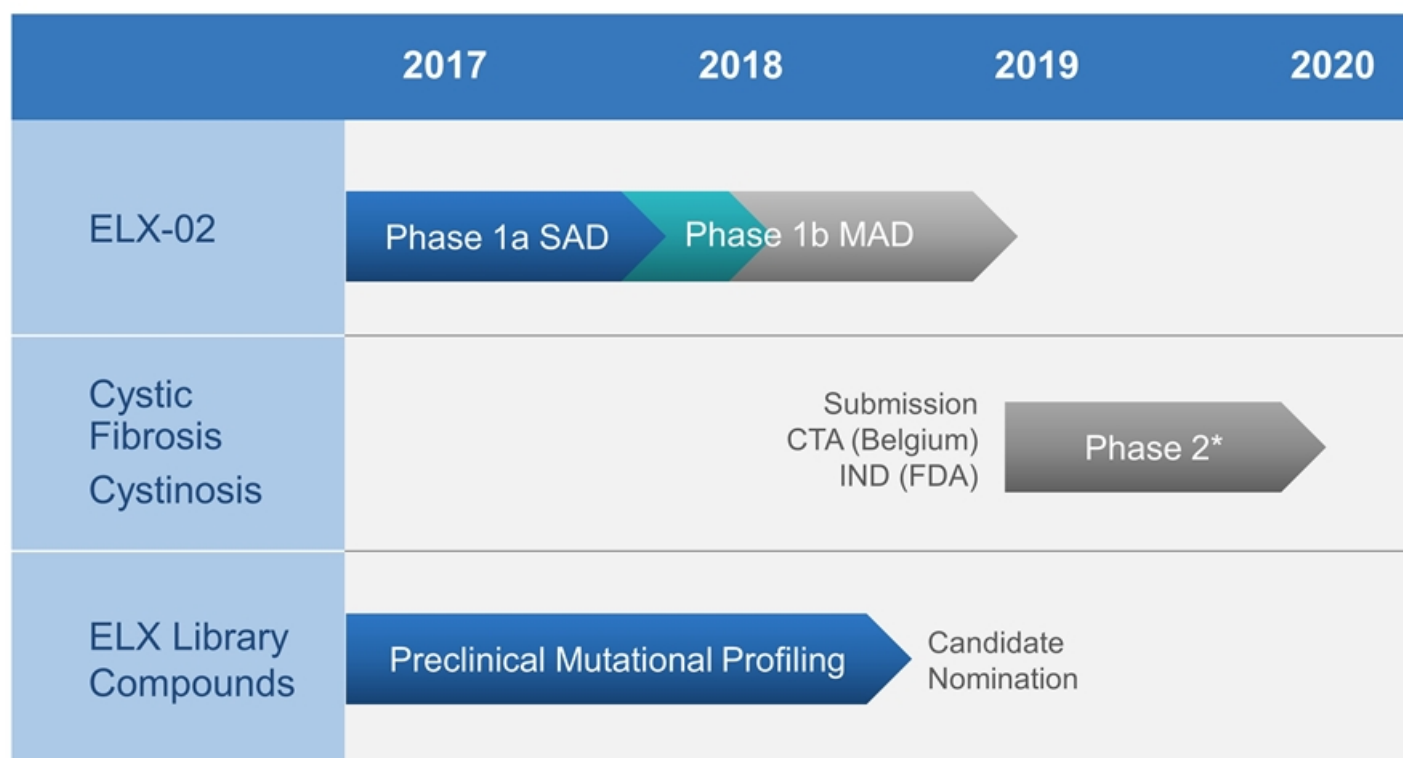
**Planned Enrollment: 45**

### TO DATE:

- No SAE Observed
- No renal or otoacoustic SAE
- Generally well tolerated



## Our Current Development Pipeline



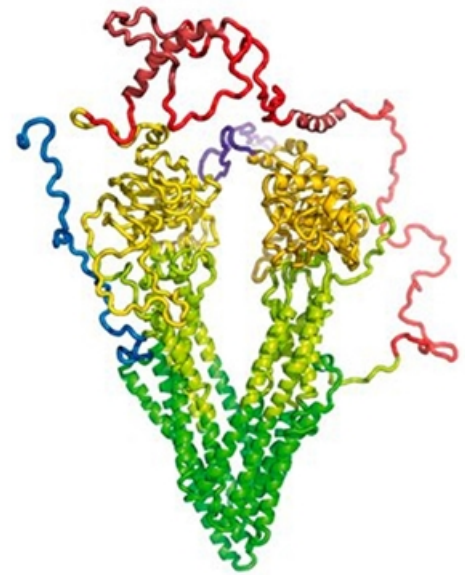
ELX-02 and the ELX Library Compounds are investigational agents and have not been approved for use by any regulatory agency

*\*Subject to Regulatory Review of CTA and IND respectively*



# Cystic Fibrosis Development Program

- Most prevalent genetic disease in the western world
  - CF is the most common fatal inherited disease in Caucasians
- Caused by mutations in transmembrane conductance regulator (CFTR)
  - Chloride channel
- Mutations lead to dysregulation in multiple organ systems
- Current standard of care based on molecular chaperones for trafficking and conformation
  - Target Class II – Class V CFTR Defects
  - No currently approved drugs for Class I CFTR Defects
- Currently available data for our investigational drug, ELX-02, suggests the potential for:
  - Active for both homozygous and heterozygous Class I nonsense mutations
  - Increase translational read-through
  - Improve chloride currents in HBEs and organoids
  - Demonstrate synergy with correctors and potentiators in heterozygous population



Zoltan Bozoky et al. PNAS  
2013;110:47:E4427-E4436

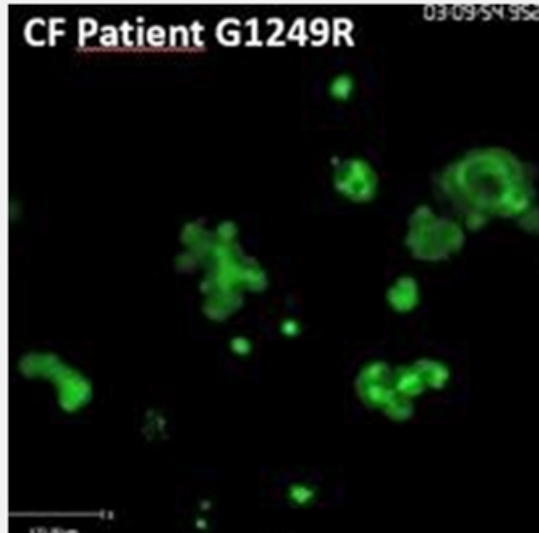
# Cystic Fibrosis: CFTR Molecular Defect

- Premature stop codons or nonsense mutations are Class I
- Estimated that 22% of patients have Class I mutations on one or both CFTR alleles
  - Approximately 13% of the CF patients carry a nonsense mutation on one or both CFTR alleles
- Eloxx's development path for read-through therapeutics will be focused on the patient subset with diagnosed nonsense mutations

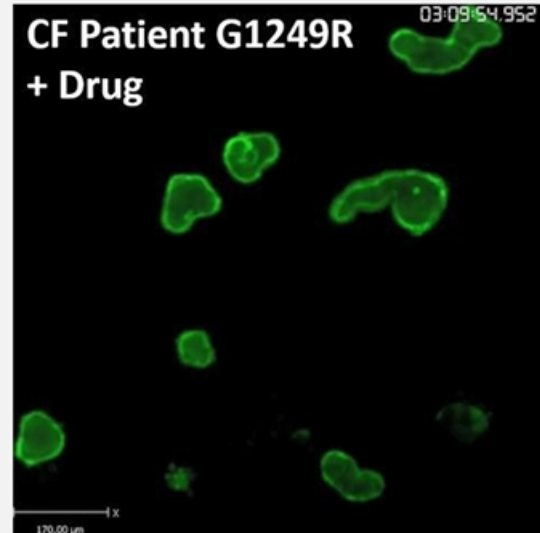
	CFTR defect type:					
	I	II	III	IV	V	VI
	No protein	No traffic	No function	Less function	Less protein	Less stable
<b>Mutation examples</b>	G542X (a) W1282X (a) 1717-1G (b)	F508del N1303K A561E	G551D S549R G1349D	R117H R334W A455E	A455E 3272-26A>G 3849+10 kb C>T	c.120del23 rF508del
<b>Corrective therapy</b>	Rescue synthesis	Rescue traffic	Restore channel activity	Restore channel activity	Correct splicing	Promote stability
<b>Drug</b>	Read-through compounds	Correctors	Potentiators	Potentiators	AONs Correctors Potentiators	Stabilizers
<b>Drug approved (Yes/No)</b>	No	No	Yes	No	No	No

Novel personalized therapies for cystic fibrosis: Treating the basic defect in all patients. *Journal of Internal Medicine* 277(2) · September 2014

## A CF swelling assay on cystic fibrosis patient organoids



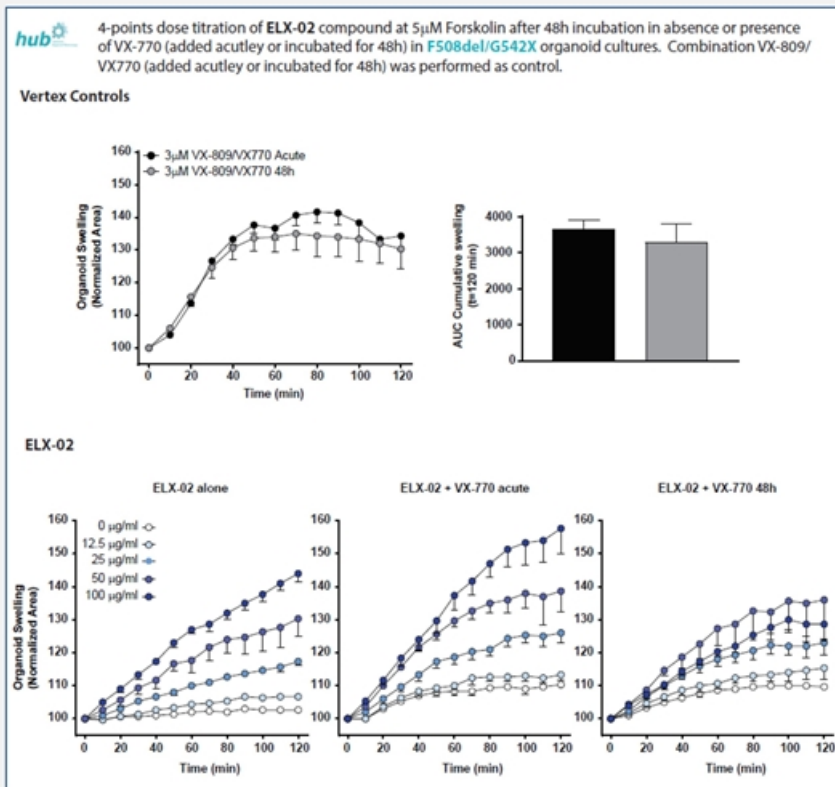
Patient Organoid without drug treatment:  
No Swelling of Organoids



Patient Organoid with drug treatment:  
Swelling of Organoids

# Heterozygous nonsense mutations

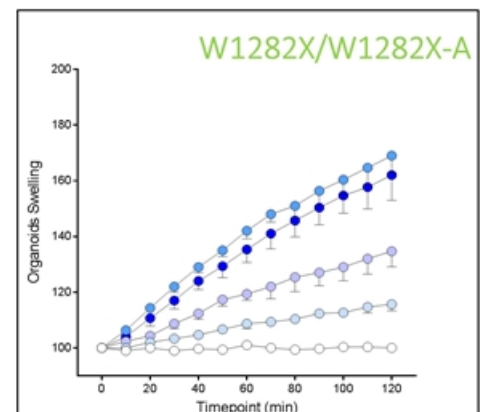
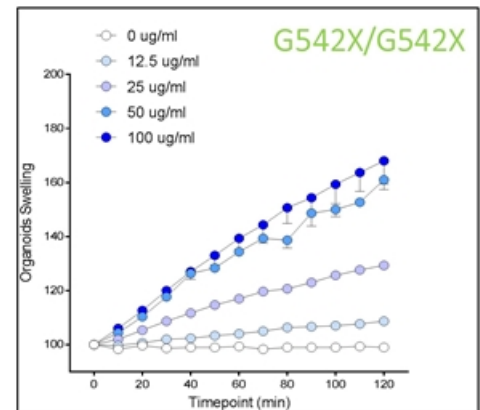
First investigational read-through agent to demonstrate *in vitro* activity in organoid cultures



# Homozygote nonsense mutations

*First investigational read-through agent to demonstrate in vitro activity in organoid cultures*

- Early-stage data involve key homozygous nonsense mutations
  - G542X prevalence estimated at 5% of CF population
  - W1282X prevalence est. at 4% of CF population
- This testing in a limited number of in vitro organoid cultures suggests organoid response to increasing exposure to our drug candidate ELX-02
  - Dose-proportional response
  - Pronounced swelling
- Organoid responses are considered important contributor to clinical trial design
  - High unmet medical need population
  - Demonstrate potential for clinical response
- Data to be submitted for scientific presentation
  - Additional homozygous and heterozygous response data
  - Evaluation of in vitro response in organoid cultures in combinations with correctors and/or potentiators



## ELX-02 Cystic Fibrosis Next Steps

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Jan 2018 Pre-CTA (Belgium) Regulatory Meeting

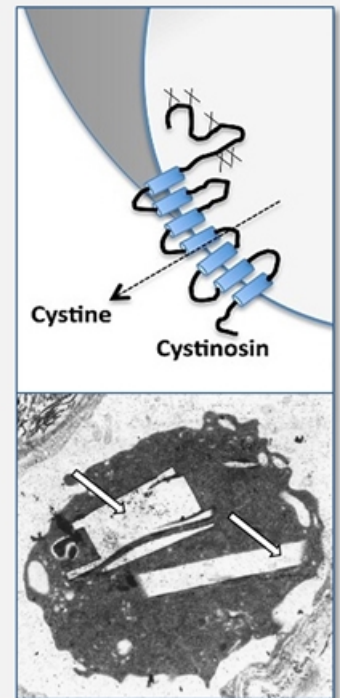
On track for mid-2018 CTA (Belgium) Submission

Targeting 4Q 2018 for FPFV Phase 2 Study



# Cystinosis Development Program

- Ultra-rare lysosomal storage disease
- Caused by mutations in cystinosin (CTNS)
  - Cysteine efflux channel
- Cystine lysosomal accumulation causes manifestations of disease
- The current standard of care, Cysteamine acts within the lysosome to convert cystine into forms which can exit the lysosome via cysteine transport pathways.
- W138X most common nonsense mutation is estimated to represent 1/3 of patient population
- Currently available data on our investigational drug candidate, ELX-02, suggest the potential to:
  - Increase translational read-through
  - Reduce NMD
  - Restore CTNS mRNA to near normal levels
  - Lower cystine accumulation in vitro and in vivo



## ELX-02 Cystinosis Next Steps

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▶ Dec 2017 Pre-IND FDA (Written Response)

▶ On track for mid-2018 IND Submission in US

▶ Targeting 4Q2018 for FPFV Phase 2 Study



## 2017 Summary Financial Results

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\$ in millions		
<b><u>Statement of Operations</u></b>		
	FY 2017	FY 2016
R&D Expense	\$ 16.4	\$ 9.0
G&A Expense	4.0	0.8
Total Operating Expenses	20.4	9.8
Other Expenses	0.8	-
Net Loss	\$ 21.2	\$ 9.8

## Financial Summary

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- \$24 million cash as of December 31, 2017
- No debt
- Funded through at least the end of the first quarter 2019
- Shares outstanding totals 27.5 million
- Traded OTC: ELOX

# Eloxx Pharmaceuticals Highlights

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- **Participation at Key Scientific Conferences**
- **Eloxx to nominate second novel molecule for development in rare/ultra-rare orphan disease**





Thank you.

- FY2017 Webcast & Conference Call March 20, 2018