Eloxx Pharmaceuticals

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

FY2017 Webcast & Conference Call March 20, 2018

Forward-Looking Statements

Certain statements included in this presentation are forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. 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 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

- **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**

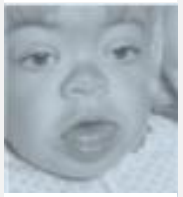
The Promise of Read-Through

>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

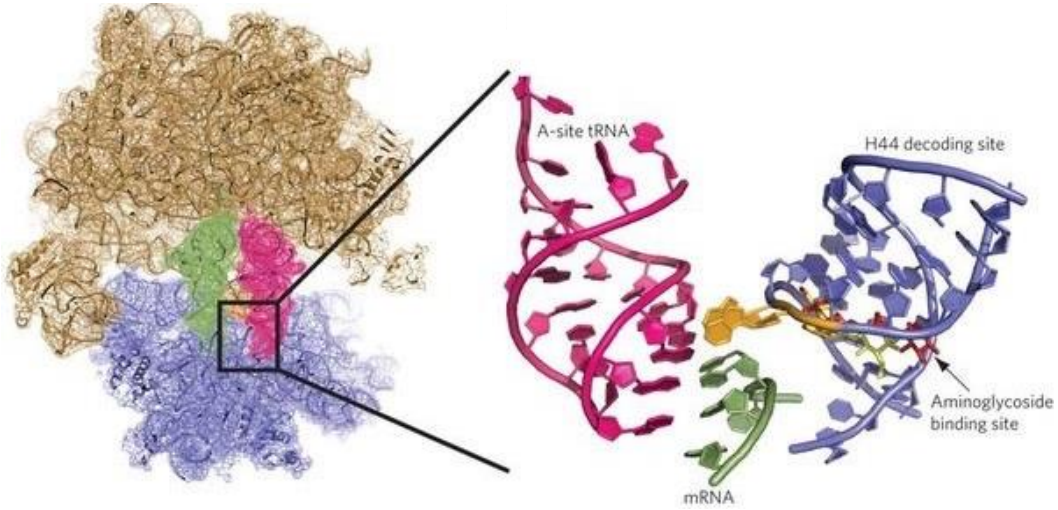
Advances in our understanding of translational read-through has enabled design of novel small molecules

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

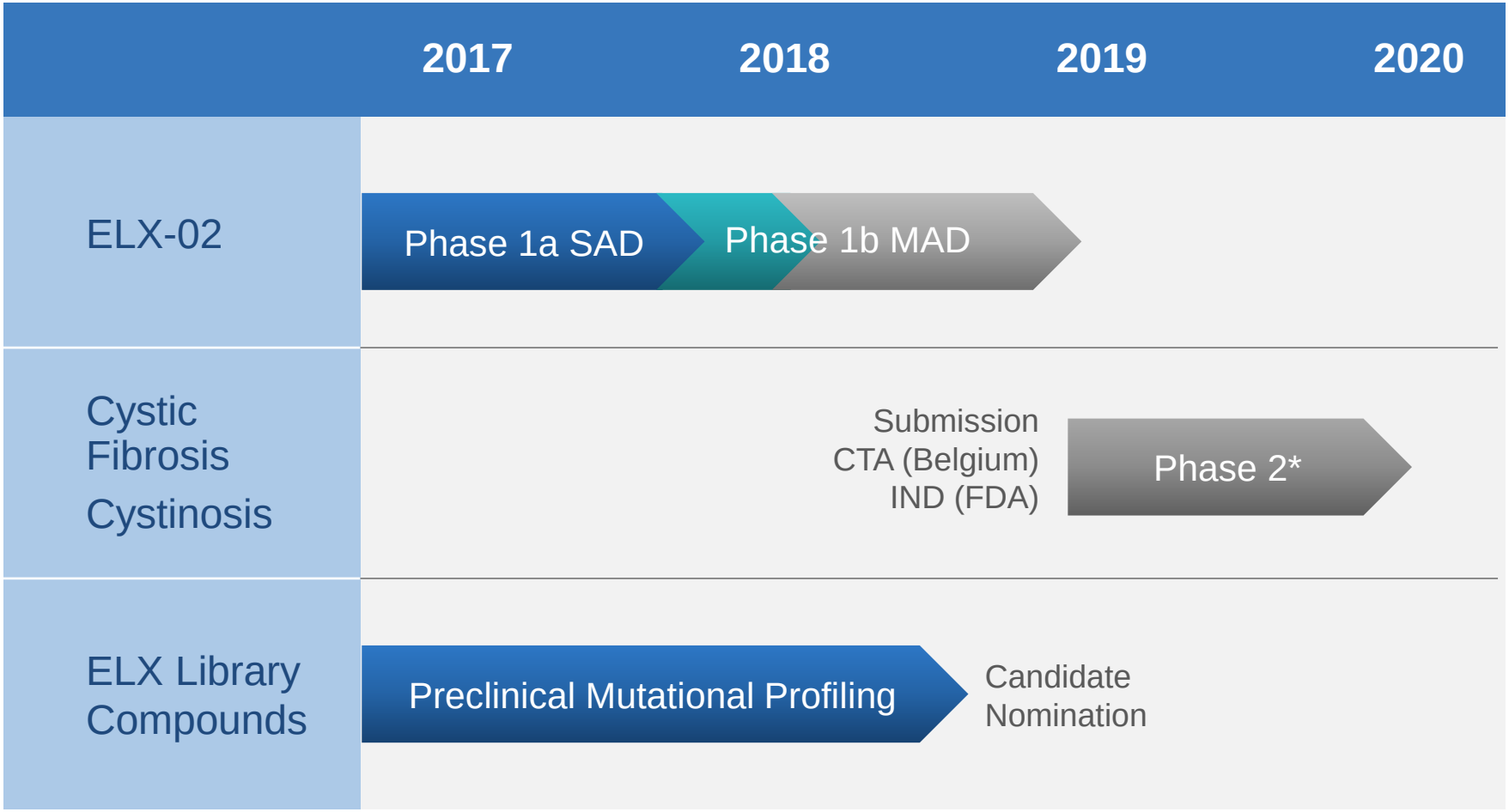
A Phase 1, Randomized, Double-Blinded, Placebo-Controlled, Third Party Open, Multiple Dose Escalation, Single Center Study to Evaluate the Safety, Tolerability and Pharmacokinetics of Subcutaneously Administered ELX-02 in Independent Consecutive Cohorts of Healthy Subjects

Planned Enrollment: 45

ONGOING

TO DATE:
- No SAE Observed
- No renal or otoacoustic SAE
- Generally well tolerated

Our Current Development Pipeline

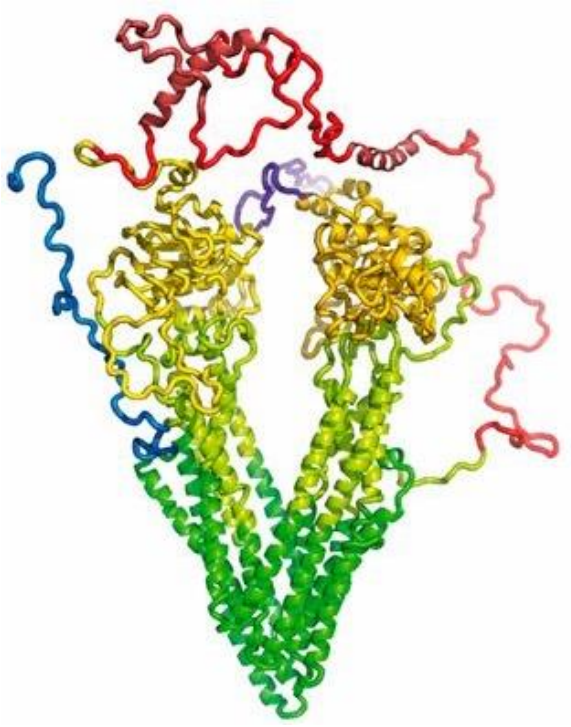


ELX-02 and the ELX Library Compounds are investigational agents and have not been approved for use by any regulatory agency

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

Cystic Fibrosis Development Program

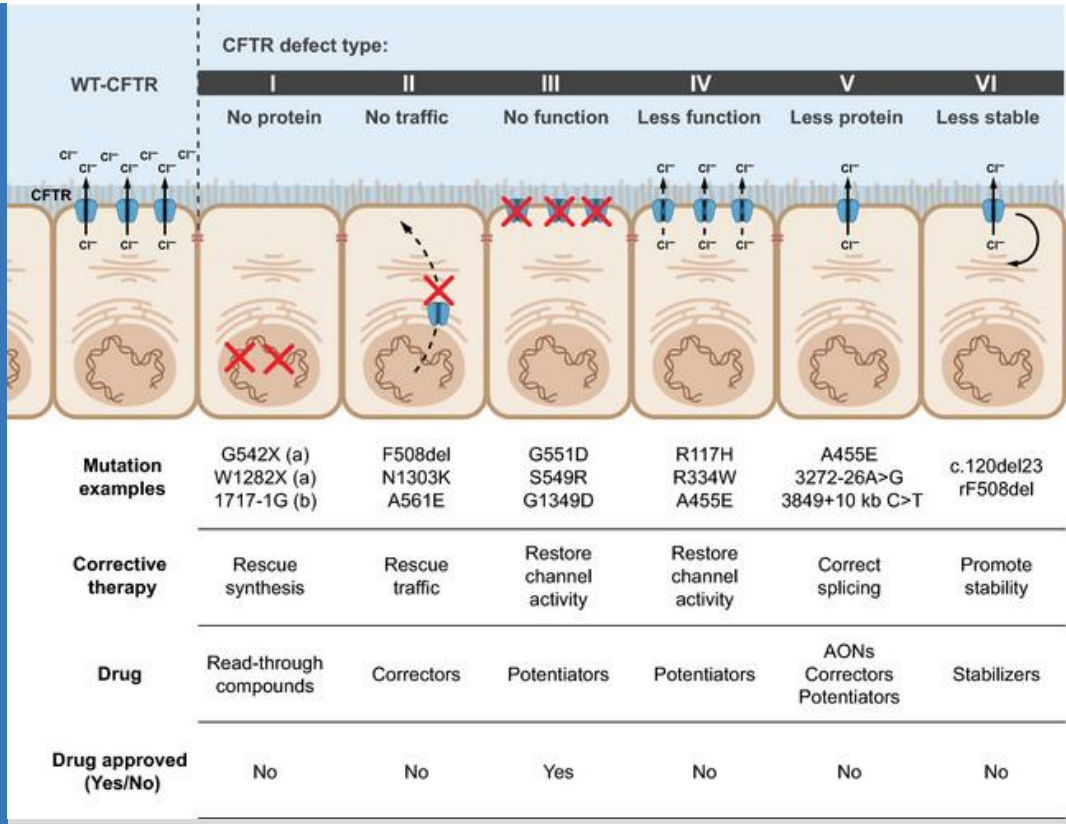
- Most prevalent genetic disease in the western world
  - CF is the most common fatal inherited disease in Caucasians
- Caused by mutations in transmembrane conductance regulator (CFTR)
  - Chloride channel
- Mutations lead to dysregulation in multiple organ systems
- Current standard of care based on 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



	I	II	III	IV	V	VI
Mutation examples	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
Corrective therapy	Rescue synthesis	Rescue traffic	Restore channel activity	Restore channel activity	Correct splicing	Promote stability
Drug	Read-through compounds	Correctors	Potentiators	Potentiators	AONs Correctors Potentiators	Stabilizers
Drug approved (Yes/No)	No	No	Yes	No	No	No

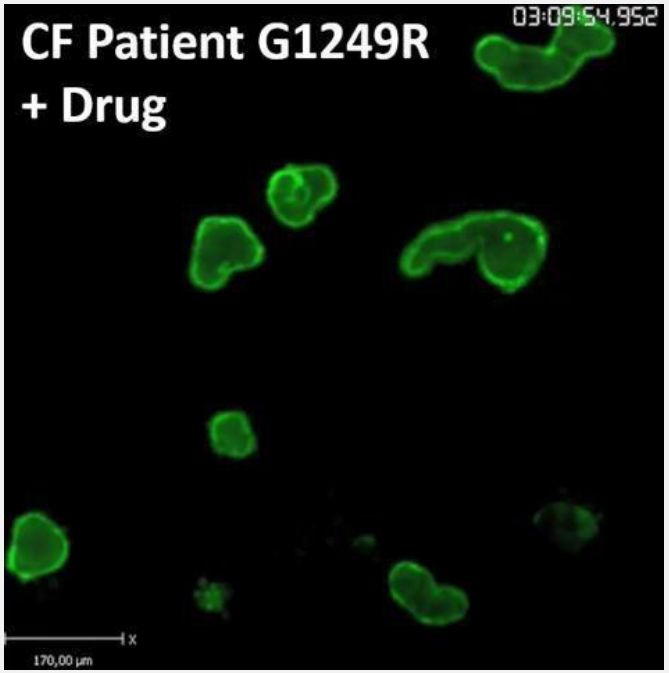
Novel personalized therapies for cystic fibrosis: Treating the basic defect in all patients. Journal of Internal Medicine 277(2) · September 2014

Organoids Pre-clinical Patient Stratification

Potential Use To Define Clinical Trial Populations

A CF swelling assay on cystic fibrosis patient organoids

Patient Organoid without drug treatment:
No Swelling of Organoids



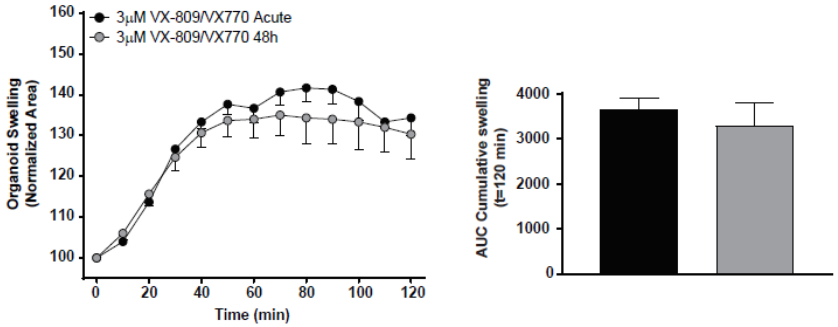
Patient Organoid with drug treatment:
Swelling of Organoids

Heterozygous nonsense mutations

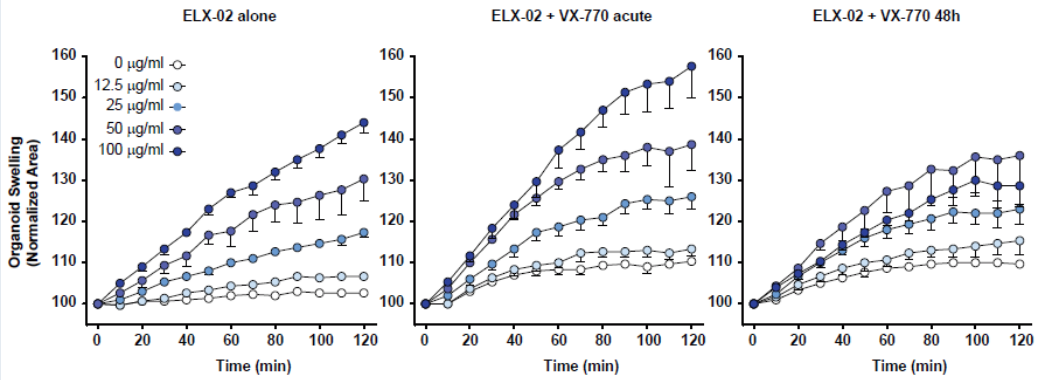
First investigational read-through agent to demonstrate in vitro activity in organoid cultures

4-points dose titration of **ELX-02** compound at 5µM Forskolin after 48h incubation in absence or presence of VX-770 (added acutley or incubated for 48h) in **F508del/G542X** organoid cultures. Combination VX-809/ VX770 (added acutley or incubated for 48h) was performed as control.

Vertex Controls



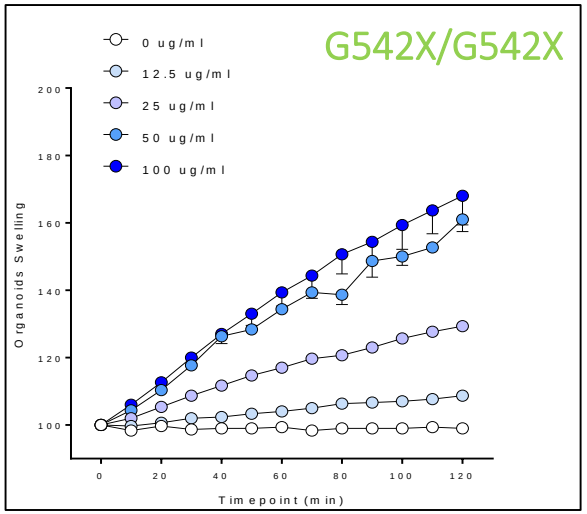
ELX-02

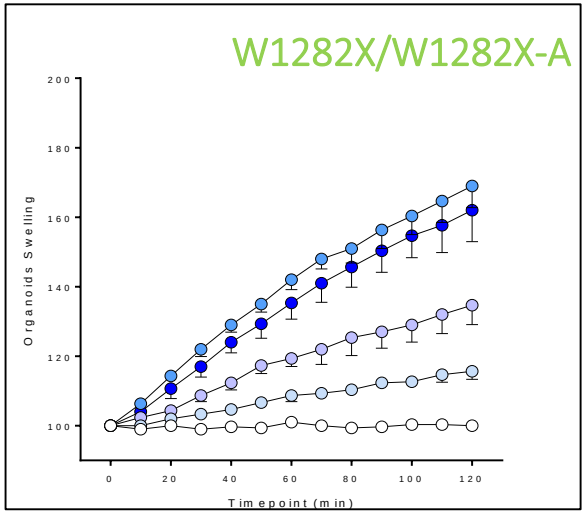


Homozygote nonsense mutations

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



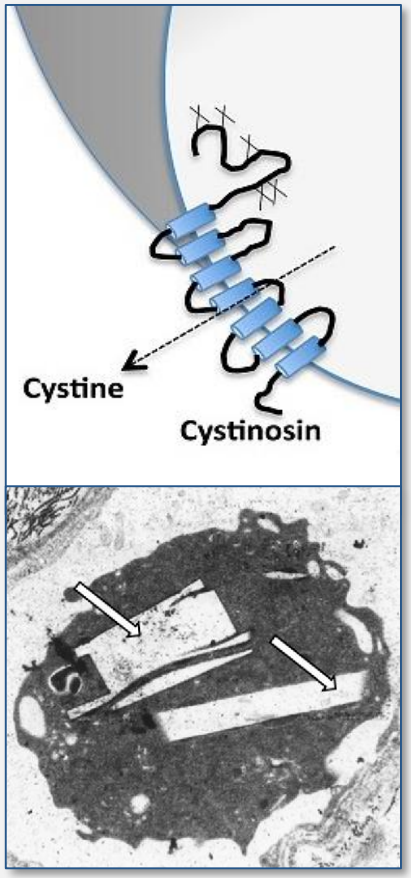


ELX-02 Cystic Fibrosis Next Steps

- 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

- 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

$ in millions		
Statement of Operations		
	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

- $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

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Thank you.

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