

## Washington, D.C. 20549

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. ☐

**Item 7.01 Regulation FD Disclosure.**

On October 2, 2018, Eloxx Pharmaceuticals, Inc. (the “Company”) presented certain information at the 2018 Cantor Global Healthcare Conference at the InterContinental New York Barclay Hotel in New York City. A copy of the presentation materials is attached hereto as Exhibit 99.1 and is incorporated herein by reference. A live webcast of the meeting was made available to the general public. A copy of the presentation materials may be found at the Company’s website (www.eloxxpharma.com) and clicking the Investor Relations webpage.

In accordance with General Instruction B.2 of Form 8-K, the information in this Current Report on Form 8-K, including Exhibit 99.1, is furnished pursuant to this Item 7.01 and shall not be deemed to be “filed” for the purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the “Exchange Act”), or otherwise subject to the liabilities of that section, and shall not be incorporated by reference into any registration statement or other document filed under the Securities Act of 1933, as amended, or the Exchange Act, except as shall be expressly set forth by specific reference in such filing.

**Item 9.01 Financial Statements and Exhibits.**

(d) [Exhibit 99.1 The Company’s 2018 Cantor Global Healthcare Conference presentation materials dated October 2, 2018.](#)

This exhibit is furnished pursuant to Item 7.01 and shall not be deemed to be “filed.”

<u>Exhibit No.</u>	<u>Description</u>
<a href="#">99.1</a>	<a href="#">The Company’s 2018 Cantor Global Healthcare Conference presentation materials dated October 2, 2018.</a>

## 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: October 2, 2018

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

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

**Cantor Global Healthcare, October 2, 2018**

# Forward-Looking Statements

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*This press release contains forward-looking statements, which are generally statements that are not historical facts. Forward-looking statements can be identified by the words "expects," "anticipates," "believes," "intends," "estimates," "plans," "will," "outlook" and similar expressions. Forward-looking statements are based on management's current plans, estimates, assumptions and projections, and speak only as of the date they are made. We undertake no obligation to update any forward-looking statement in light of new information or future events, except as otherwise required by law. Forward-looking statements involve inherent risks and uncertainties, most of which are difficult to predict and are generally beyond our control. Actual results or outcomes may differ materially from those implied by the forward-looking statements as a result of the impact of a number of factors, including: the development of the Company's read-through technology; the approval of the Company's patent applications; the Company's ability to successfully defend its intellectual property or obtain 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 Company's ability to obtain applicable regulatory approvals for its current and future product candidates; the acceptance by the market of the Company's products should they receive regulatory approval; the timing and success of the Company's preliminary studies, preclinical research, clinical trials, and related regulatory filings; the ability of the Company to consummate additional financings as needed; as well as those discussed in more detail in our Annual Report on Form 10-K and our other reports filed with the Securities and Exchange Commission.*

# Eloxx Pharmaceutical Highlights

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- **Experienced Leadership Team**
- **ELX-02 Clinical Progress**
  - ✓ Phase 1a SAD Submitted for Publication
  - Phase 1b MAD Ongoing
  - ✓ Final Approval CTA for Cystic Fibrosis Phase 2 in Belgium
  - ✓ Open IND for Cystinosis Phase 2 in US
- **Expect to Complete MAD and Reach Top Line Phase 2 Data in 2019**
- **Upcoming ELX-02 Presentation at the North American Cystic Fibrosis Society Meeting on October 18**
  - “Measuring mRNA Levels in Cystic Fibrosis Organoids with Nonsense Mutations Following Treatment with ELX-02”
- **Progressing Novel Library Molecules Toward IND**
  - Existing Data Support Activity on Nonsense Mediated Ocular Orphan Targets
  - Supportive Preclinical Studies Have been Initiated
  - On Track for Advancing Pipeline in 2018
- **Well Funded to 2020**
  - Cash and Cash Equivalents of \$63.4 million at June 30, 2018

# Built a Highly Experienced Leadership Team

Robert Ward  
CHAIRMAN AND CEO

Radius AstraZeneca

Greg Williams, PhD  
COO

Radius The  
Medicines  
Company

David Snow  
CBO

AstraZeneca Radius

Greg Weaver  
CFO

Prometic ORYZON

Neil Belloff, Esq.  
GENERAL COUNSEL

Celgene

Deutsche  
Telekom

John van Duzer, PhD  
VP CMC

Celgene

Acetylon  
Pharmaceuticals, Inc.

Neal Sharpe, PhD  
VP TRANSLATIONAL SCIENCE

Biogen

MERCK

Barbara Ryan  
INVESTOR RELATIONS

Radius Deutsche Bank



# The Potential for Read-Through of Rare Genetic Diseases

**>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

Aminoglycosides' tolerability profile historically limited suitability for read-through treatment of serious genetic diseases

Aminoglycosides first showed read-through activity in nonsense mediated diseases

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



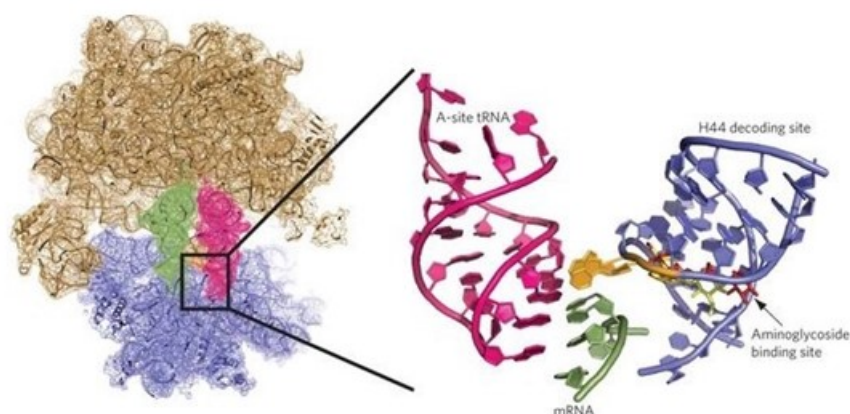
## Targeting Read-Through Development Activities

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Eloxx read-through program is pursuing product candidates with the following characteristics:

Activity independent of gene size or complexity of genetic disorder	Molecular scaffold with defined ribosomal effect	Active at all three premature stop codons	
Reduces rate of nonsense mediated decay	Restores protein production to a clinically significant level	Acceptable tolerability profile	Suitable for chronic administration

## Defined Ribosomal Binding Site

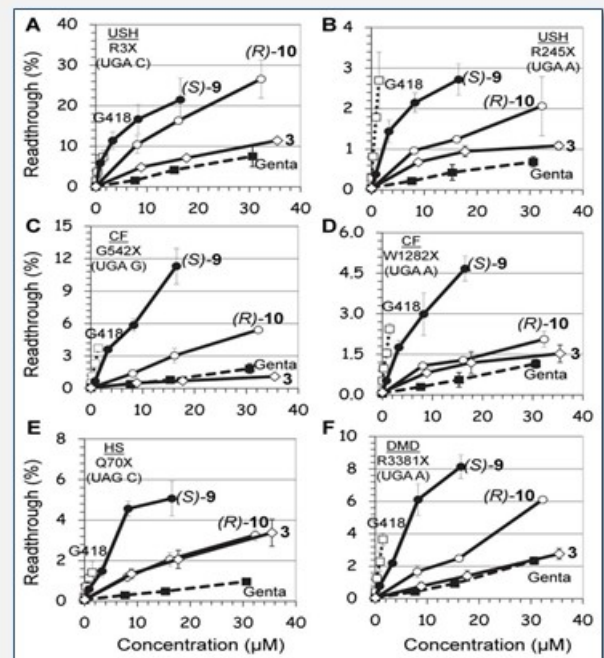


- Well defined molecular interaction with helix 44
- Role in stabilization of tRNA binding at Site A
- Optimizing scaffold by altering interaction with prokaryotic and mitochondrial ribosomes
- Defined tissue penetration and tolerability profile for read-through applications

Aminoglycoside activity observed on single pre-translocation ribosome complexes. Feldman, MB; Terry DS; Altman RB; Blanchard SC. Nature Chemical Biology volume 6, pages 54–62 (2010)

# Discovery of the Eloxx Novel Compound Library

- Novel compounds derived from aminoglycoside scaffold
- Screened for read-through activity on known disease related nonsense mutations
- Reduced mitochondrial inhibition (range 12-140X)
- Reduced prokaryotic ribosomal inhibition



Increased Selectivity towards Cytoplasmic versus Mitochondrial Ribosome Confers Improved Efficiency of Synthetic Aminoglycosides in Fixing Damaged Genes: A Strategy for Treatment of Genetic Diseases Caused by Nonsense Mutation. Kandasamy, K; Atia-Gilkin D; et al. J Med Chem (2012) 55(23):10630-10643

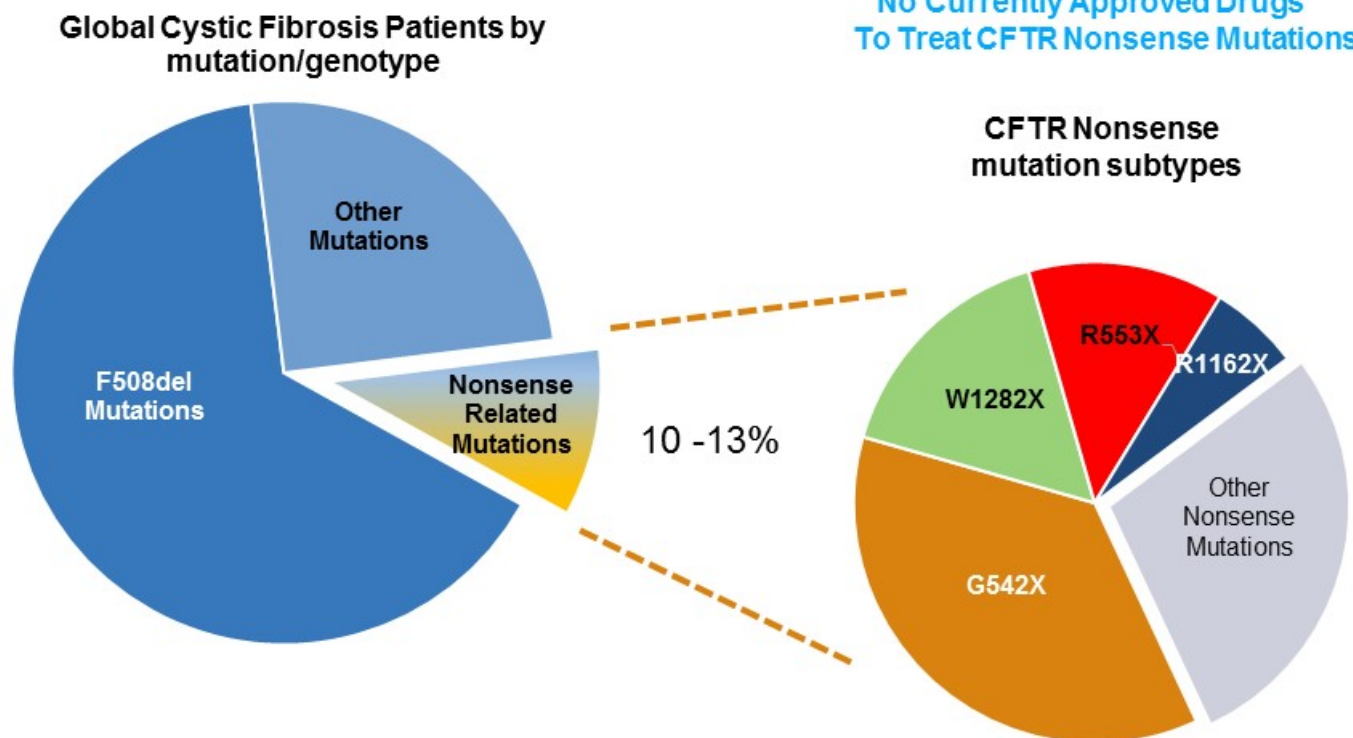
# Substantial Advantages for Orphan Drug Development

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- **ELOXX Focus on High Unmet Medical Need**
  - Nonsense mutations represent important patient segments in over 1,800 diseases
  - Many of these diseases have no approved therapeutics
  - In some diseases the nonsense patient population is appropriate size for traditional clinical development
- **Developing Novel Therapeutics through Established Pathways**
  - Many Orphan Diseases have existing preclinical assays or animal models with correlations to clinical endpoints
  - Validated Phase 2 endpoints can guide phase transition and design of Pivotal trials
- **Orphan Designation confers important Regulatory Considerations**
  - Potential for closer collaboration, accelerated development
  - Several economic or exclusivity incentives
  - In the US, Rare Pediatric Disease Priority Review Voucher Program
- **Ongoing Global Regulatory Interest in Accelerating Development for High Unmet Medical Need**

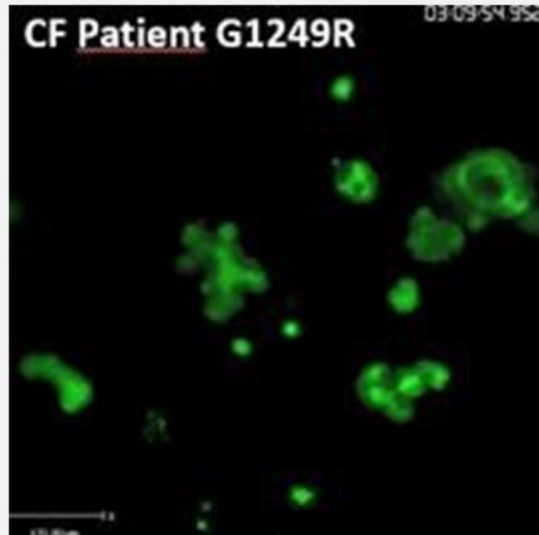
# Cystic Fibrosis Nonsense Mutation Patients Represent a Key Unmet Need Population

No Currently Approved Drugs  
To Treat CFTR Nonsense Mutations

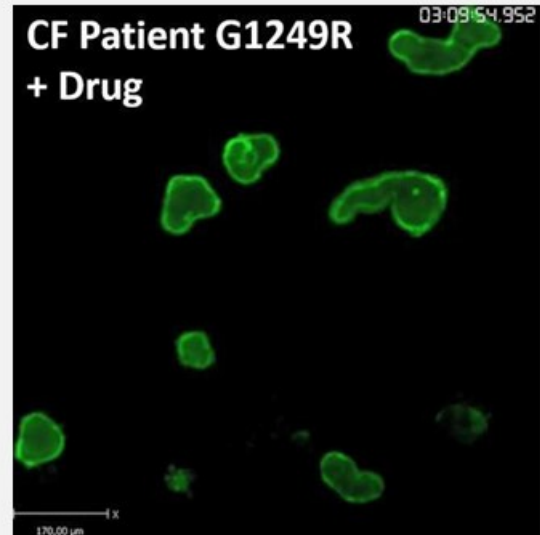




## A CF swelling assay on cystic fibrosis patient organoids



Patient Organoid without drug treatment:  
No Swelling of Organoids

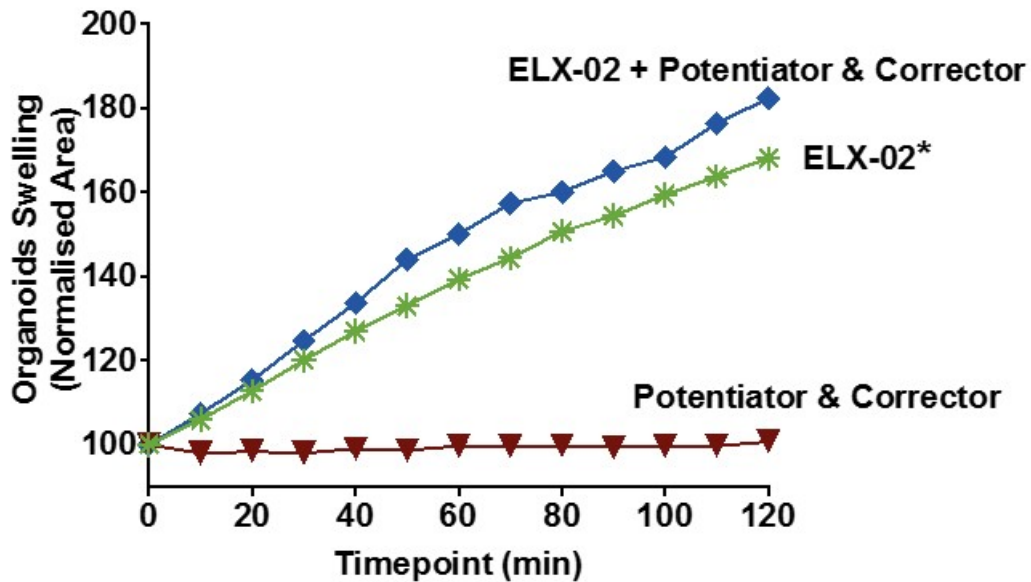


Patient Organoid with drug treatment:  
Swelling of Organoids



# Homozygous Nonsense Mutation (G542X)

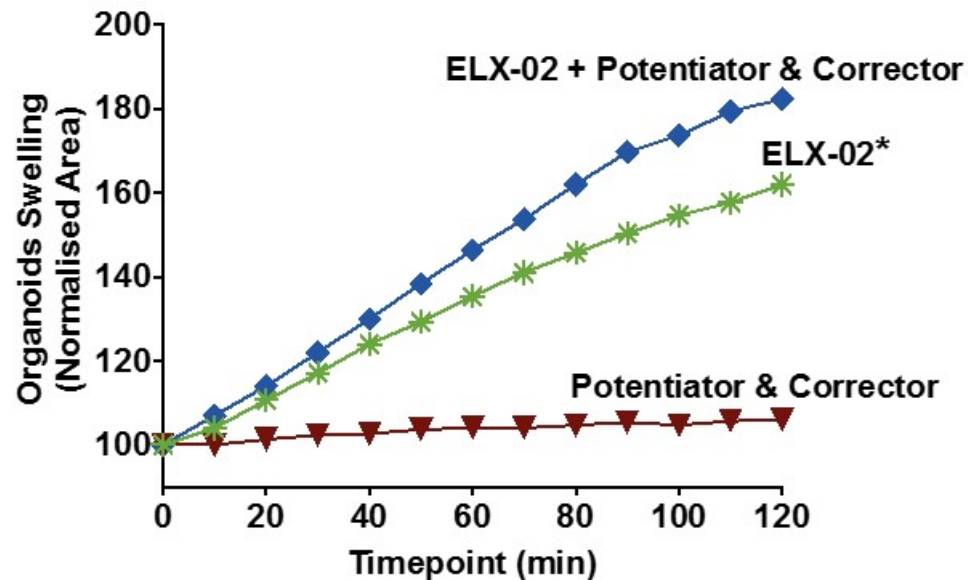
## Cystic Fibrosis Organoid Responsive to ELX-02, Enhanced by Combination



- As presented at the European Cystic Fibrosis Society 41<sup>st</sup> Conference, Belgrade, Serbia, June 2018
- 100 µg/mL ELX-02 As previously presented

# Complex Heterozygous Nonsense Mutation (G542X:R1066C missense)

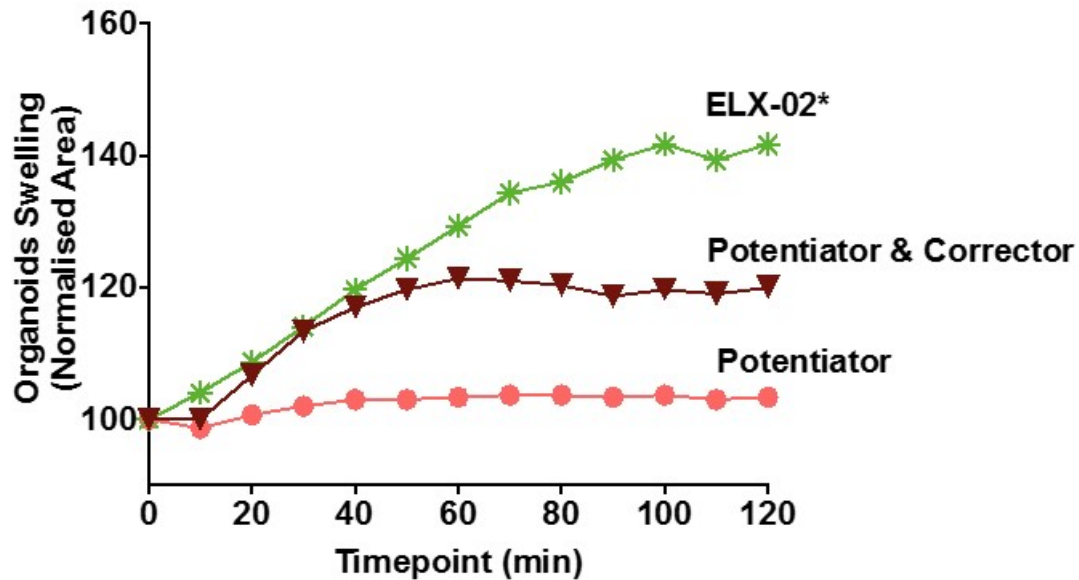
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# Complex Heterozygous Nonsense Mutation (R1162X nonsense:F508del)

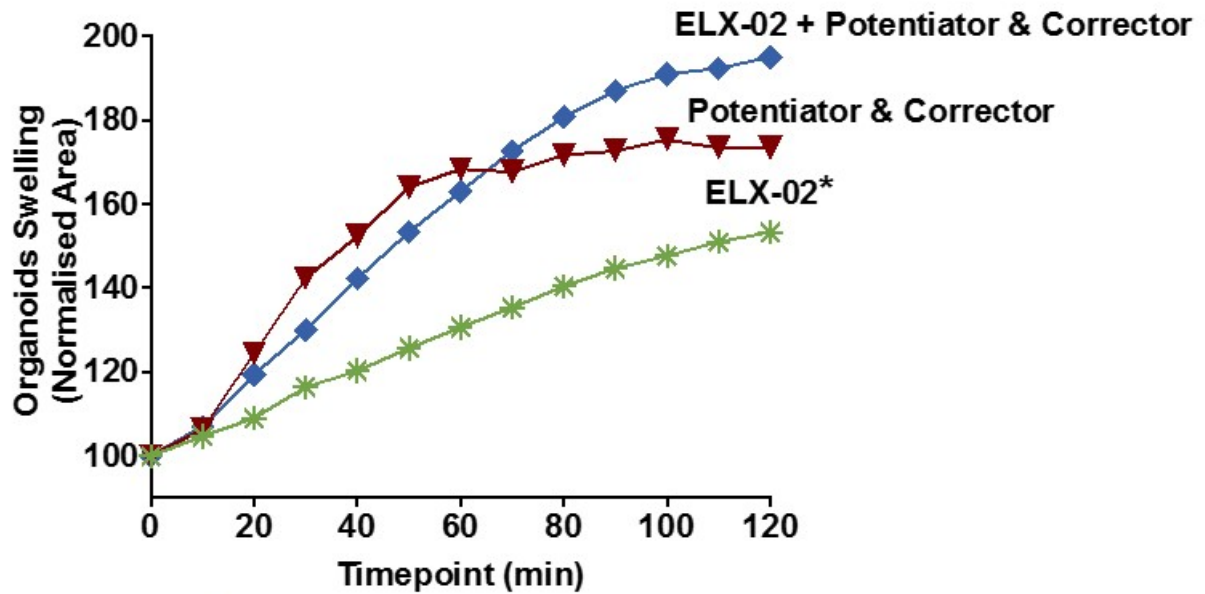
## Cystic Fibrosis Organoid Responsive to ELX-02



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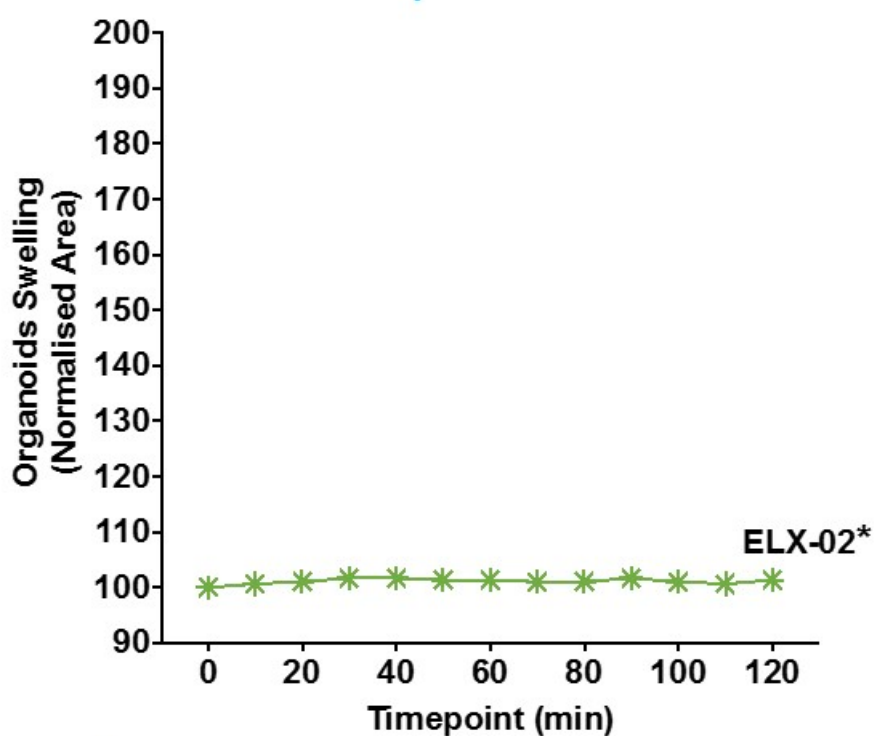
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- 100 µg/mL ELX-02 As previously presented

# Homozygous Deletion Mutation (F508del)

## Cystic Fibrosis Organoids Without Nonsense Mutations are Not Responsive to ELX-02



- As presented at the European Cystic Fibrosis Society 41<sup>st</sup> Conference, Belgrade, Serbia, June 2018
- 100 µg/mL ELX-02 As previously presented

## 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

**Submitted for  
Publication**

COMPLETED



**TO DATE:**

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

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



Completed 4<sup>th</sup> Cohort

Revising protocol for additional cohorts



## Clinical Update for ELX-02 Phase 2 in Cystic Fibrosis

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- ✓ Submitted Orphan Drug Application to EMA
- ✓ Clinical Trial Application (CTA) for Phase 2 Study received final approval by the FAMHP in Belgium
- **Expanding MAD Study**
  - ✓ 4<sup>th</sup> Cohort Completed
  - Additional Cohorts to Evaluate Drug Concentrations
- **Engaging with investigators on a protocol for Phase 2 to insure rapid execution**
  - Will evaluate changes in sweat chloride at ascending doses
  - Planned enrollment will focus on patients with G542X nonsense mutation on one (complex heterozygote) or both alleles (homozygote)
- **Expect top line data in 2019**

**NEW DATA**

Eloxx Presentation

“Measuring mRNA Levels in Cystic Fibrosis Organoids with  
Nonsense Mutations Following Treatment with ELX-02”

October 18

## Clinical Update for ELX-02 Phase 2 in Cystinosis

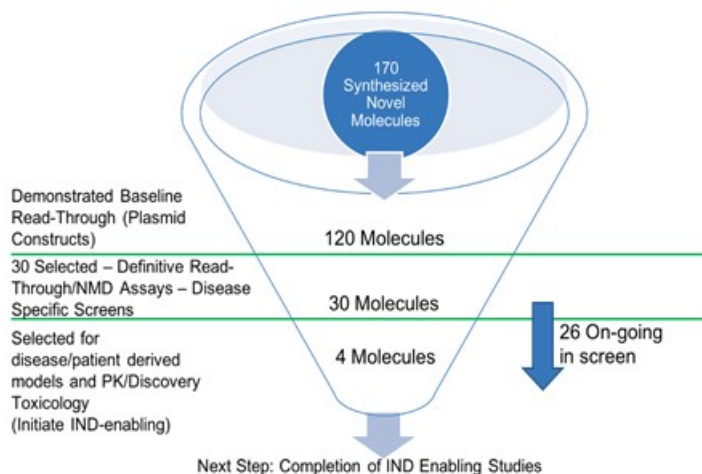
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- ✓ FDA granted ELX-02 orphan drug status in cystinosis
- ✓ IND for ELX-02 in cystinosis is open in the US
- Engaging Investigators on Phase 2 protocol
  - Will evaluate changes in cysteine levels in white blood cells
  - Study will be posted on [clintrials.gov](https://clinicaltrials.gov)
- Expect top line data on cysteine levels in 2019
- ✓ Data previously reported showed that ELX-02 decreases the cysteine content in cellular and animal models\*

\* Dr. Paul Goodyer at the 14<sup>th</sup> Annual *WORLD Symposium* on Lysosomal Diseases in a presentation titled “*Translational read through of CTNS nonsense mutations and attenuation of CTNS nonsense-mediated mRNA decay by ELX-02*”

# On Track for Advancing Pipeline in 2018

## Multiple Novel Compounds Are Advancing To IND Enabling Studies







**Extensive Intellectual  
Property Portfolio**

**Eloxx holds global rights on these library compounds  
ELX-02 Composition of Matter 2031 without extensions  
Library Composition of Matter from 2027-2038 or later  
Library Use Patents Expire 2036 or later**





## Substantial Potential Ocular Orphan Opportunity

	Incidence	US Prevalence	Nonsense Proportion
 Aniridia	1.8 : 100K	5.9K	36%
 Stickler Syndrome	1-3 : 10K	66K	20%
 Choroideremia	1-2 : 100K	6.5K	41%
 Usher Syndrome	3.2-6.2 : 100K	16.3K	25%

Population values from National Organization for Rare Disorders (NORD)

Han et al, Korean J Pediatr 2016.; Wilkin et al, Am J Med Genet 2000; Freund et al, Mol Genet Genomic Med 2016; Aparisi et al, Orphan J Rare Dis 2014



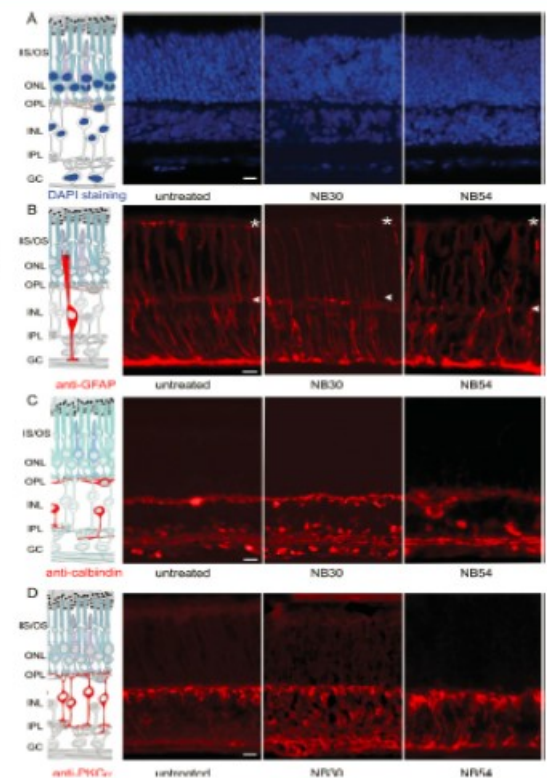
## Usher Syndrome

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- No drugs approved or in late-stage development for nonsense variants
- Significant unmet medical need for nonsense forms
  - Over 4,000 patients in North America alone
- Academic collaborations have demonstrated activity with Eloxx Novel Library Compounds
  - Read through
  - Protein expression
  - In vitro retinal compatibility

# In Vitro Retinal Biocompatibility

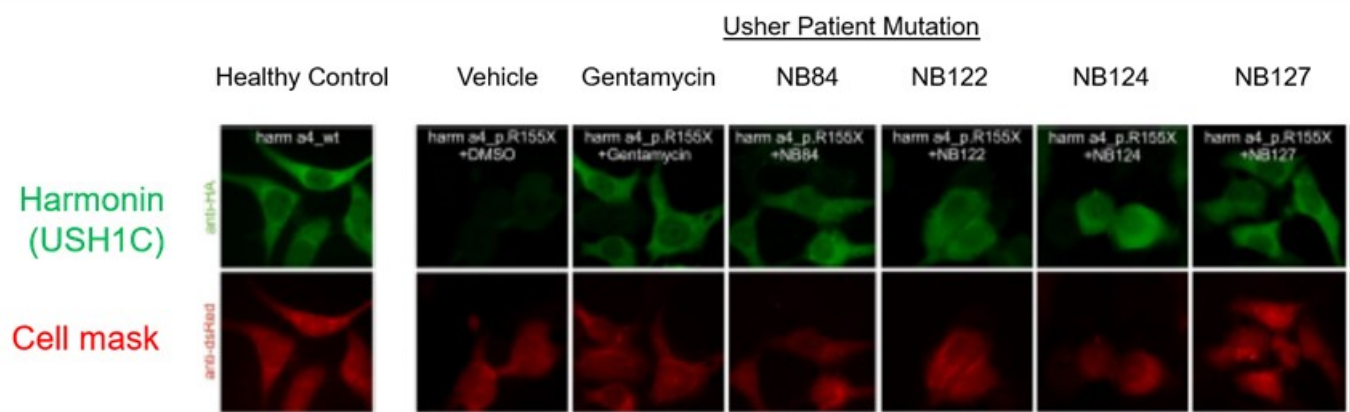
- Studies in retinal culture have demonstrated distinct biocompatibility profile, ie, no change in retinal staining or structure<sup>1</sup>
- Multiple Library Compounds<sup>2</sup> have been evaluated to date
- Additional supportive preclinical studies ongoing



<sup>1</sup>Goldmann et al. EMBO Mol Med 2012, 4(11):1186-1199

<sup>2</sup> ELX compounds, NB84, NB122, NB124 and NB127, but not gentamicin and G418, elicit comparable biocompatibility in vitro effect [Möller et al. 2016 The Annual Meeting of the Association for Research in Vision and Ophthalmology (ARVO)]

# Demonstration of Usher Protein Production



Goldmann et al. EMBO Mol Med 2012, 4(11):1186-1199

- Collaborations with the Ben-Yosef and Nagel-Wolfrum labs demonstrate potential:
  - ✓ Favorable safety profile in the retina
  - ✓ Readthrough of relevant eye-disorder mutations
  - ✓ Production of missing protein

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Thank you.

**Cantor Global Healthcare, October 2, 2018**