



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

SVB Leerink Global Healthcare Conference

Forward-Looking Statements

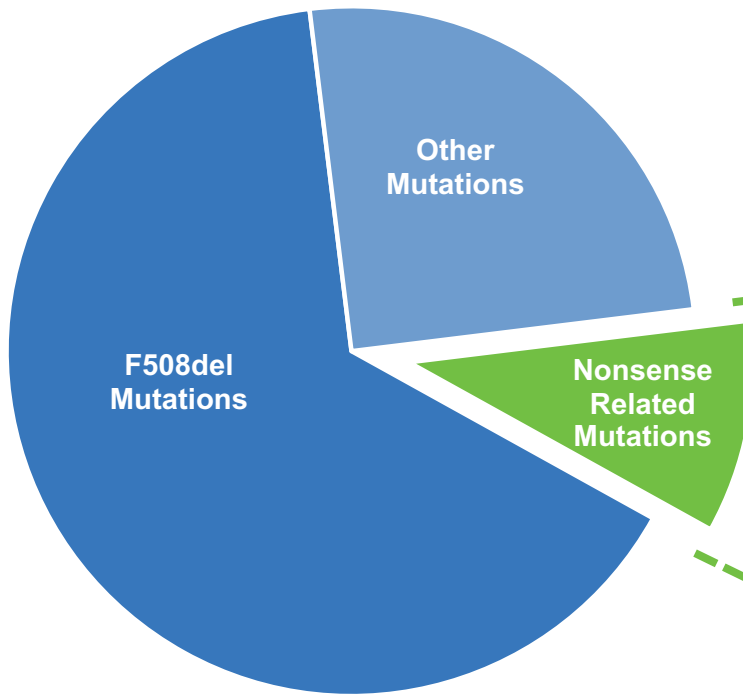
This presentation contains forward-looking statements, which are generally statements that are not historical facts. Forward-looking statements can be identified by the words "expects," "anticipates," "believes," "intends," "estimates," "plans," "will," "outlook" and similar expressions. Forward-looking 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

- **Experienced Leadership Team**
- **ELX-02 Clinical Progress**
 - Phase 1a SAD Published January 2019
 - Phase 1b MAD Ongoing
 - Final Approval CTA for Cystic Fibrosis Phase 2 in Belgium
 - Open IND for Cystinosis Phase 2 in US
- **Expect Topline Cystic Fibrosis Phase 2 Data in 2019**
- **Building Ophthalmology Inherited Retinal Disorder Program**
 - Existing Data Support Activity on Nonsense Mediated Ocular Orphan Targets
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 - Announced Partnership with Foundation Fighting Blindness
- **Actively Developing Opportunities for Collaboration to Advance Full Pipeline and Expand Therapeutic Programs**

Cystic Fibrosis Nonsense Mutation Patients Represent a Key Unmet Need Population

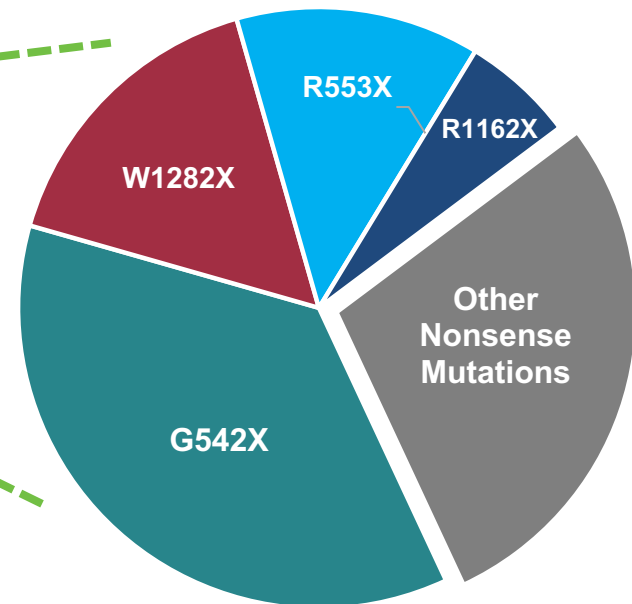
GLOBAL CYSTIC FIBROSIS PATIENTS
BY MUTATION/GENOTYPE



10 -13%

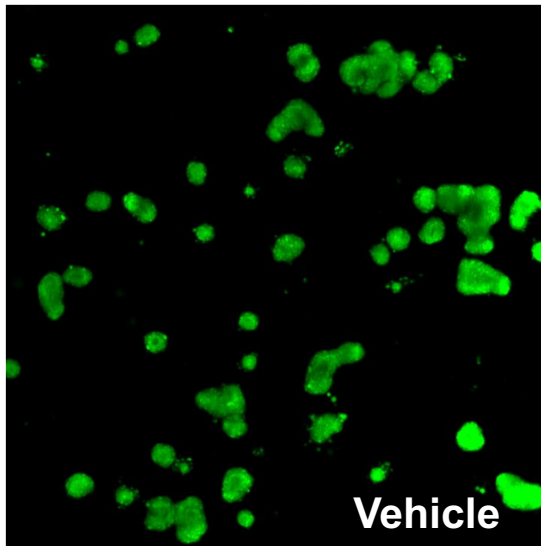
No Currently Approved Drugs
To Treat CFTR Nonsense Mutations

CFTR NONSENSE
MUTATION SUBTYPES

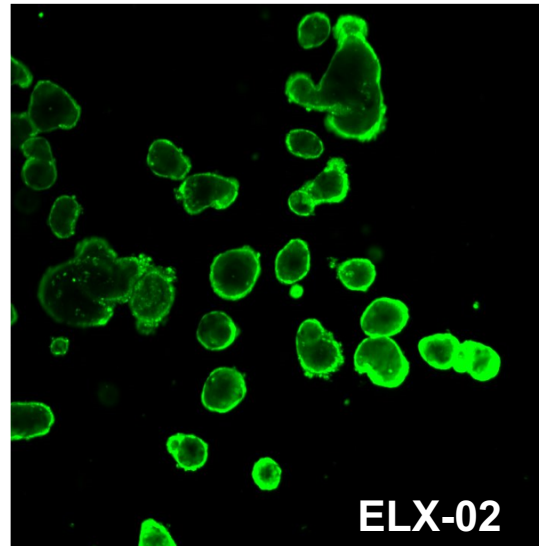


Organoid Swelling Assay Demonstrates CFTR Function

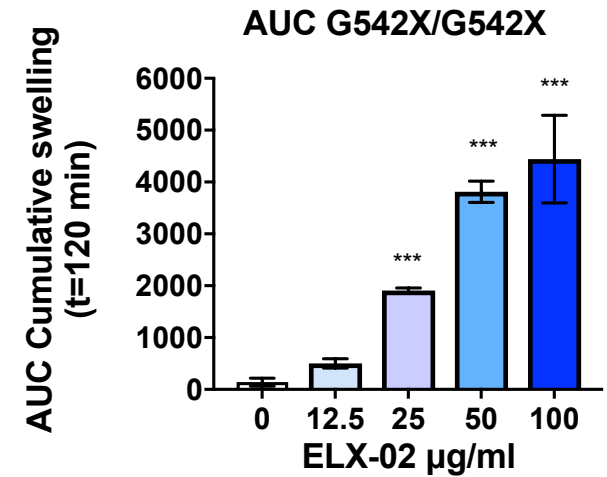
Cystic Fibrosis Organoid dose-responsive swelling assay response



Patient Organoid without drug treatment:
No Swelling of Organoids

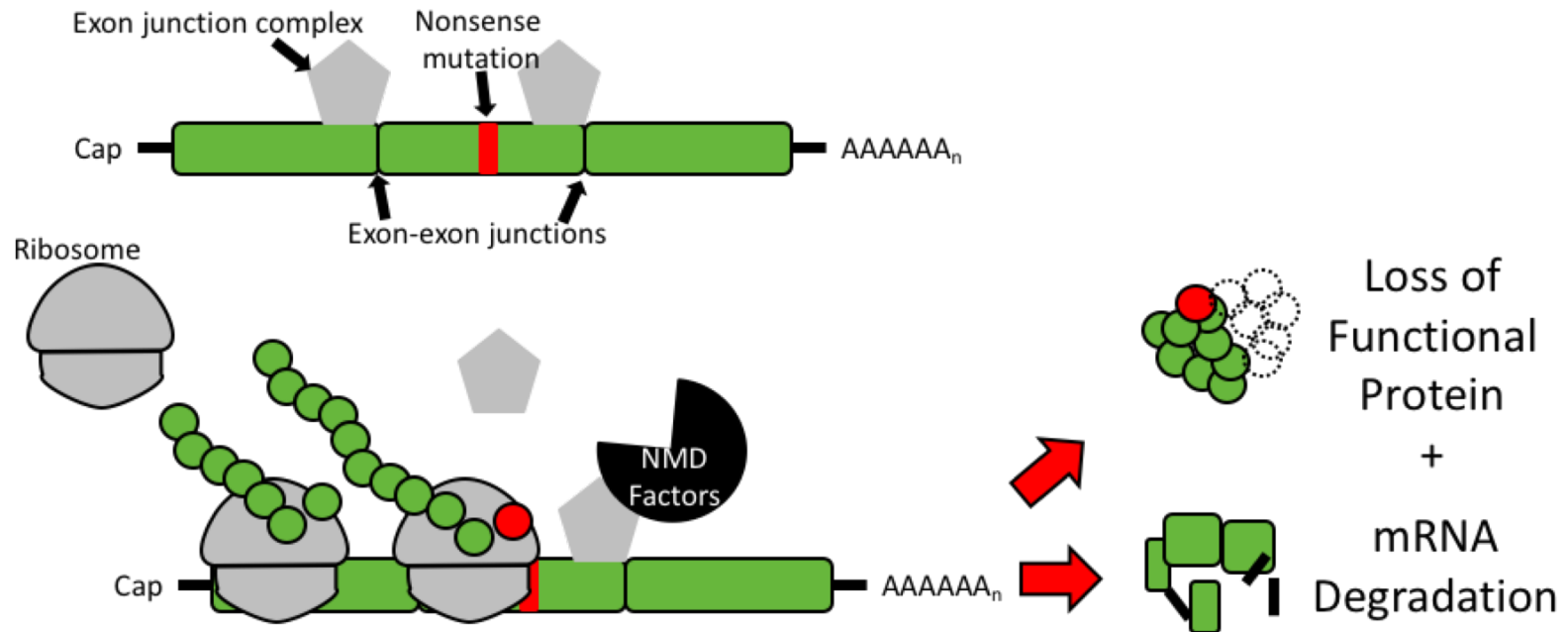


Patient Organoid with ELX-02 treatment:
Swelling of Organoids



Swelling quantification of patient organoid with ELX-02 treatment.

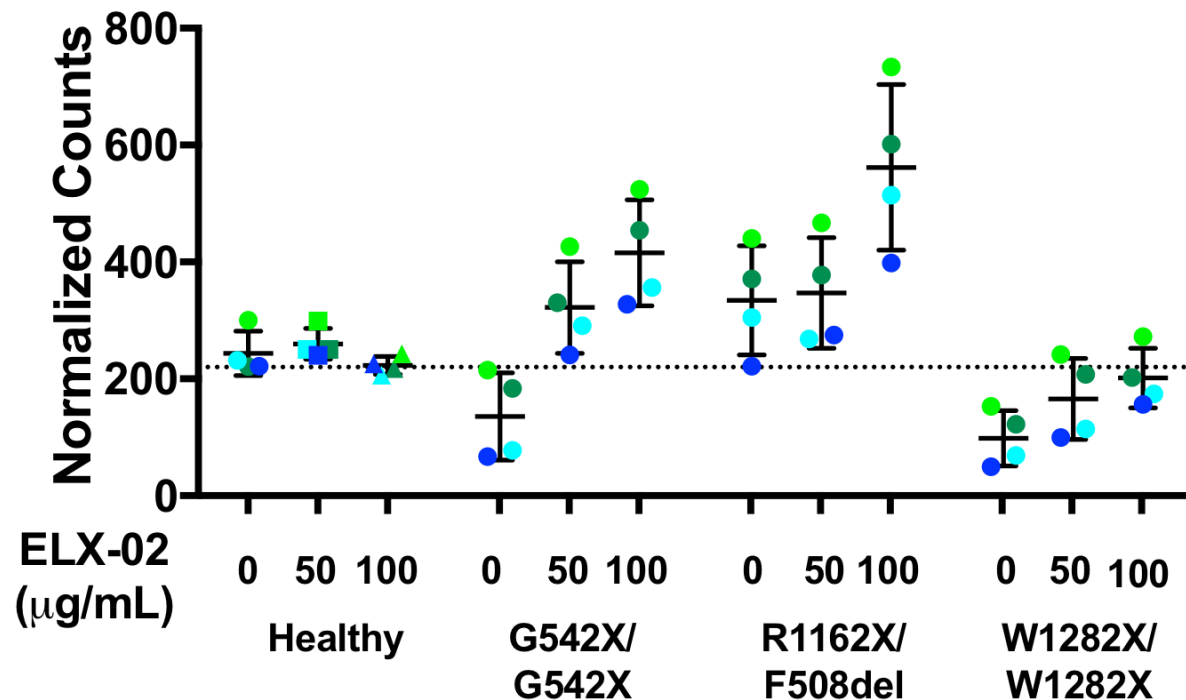
Nonsense Mutations Can Cause a “Double-Hit”, Loss of mRNA and Functional Protein



For more information: Kurosaki & L.E. Maquat. Nonsense-mediated mRNA decay in humans at a glance. *J. Cell Sci.* 1;129(3):461-7 (2016).

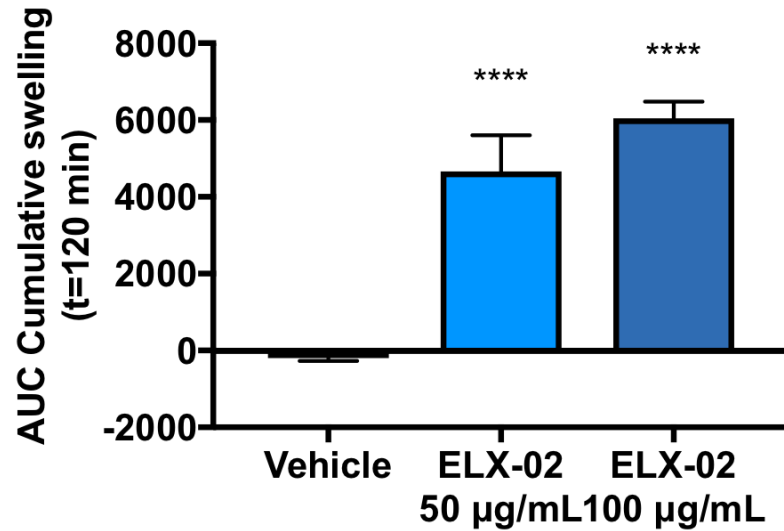
CFTR mRNA Comparisons to Healthy, Wild-type Control

Cystic Fibrosis Organoid Responsive to ELX-02 demonstrates elevations above wild-type

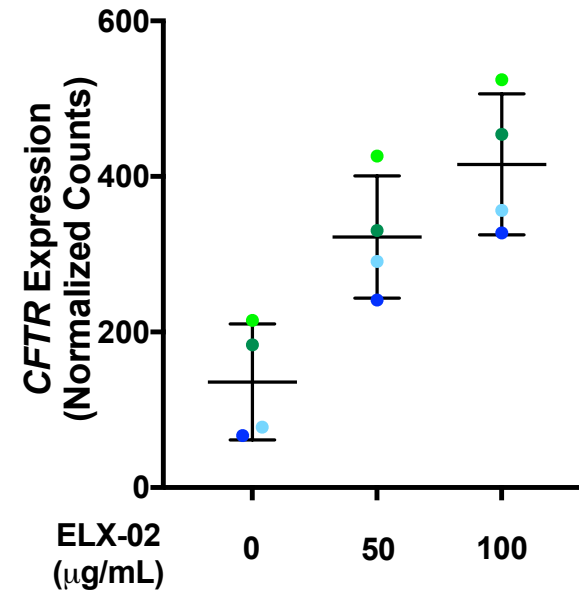


Homozygous Nonsense Mutation (G542X)

SWELLING (FUNCTION) ASSAY

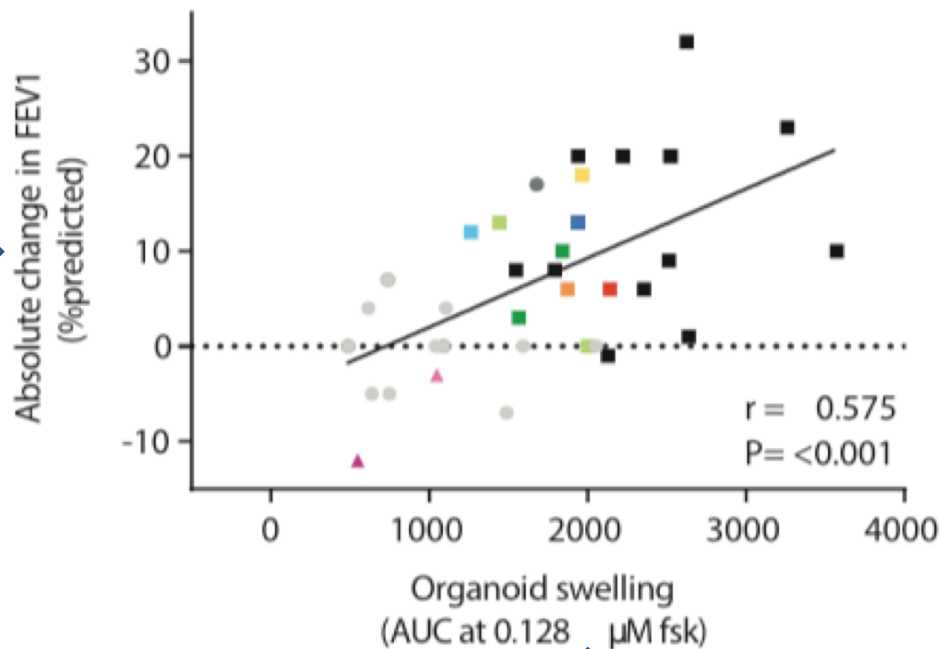


NANOSTRING (MRNA) ASSAY



Organoid data correlates with potential clinical response

Organoids vs. FEV1 (n=35)



FEV1 response correlates with organoid swelling in samples collected across multiple clinical trials.

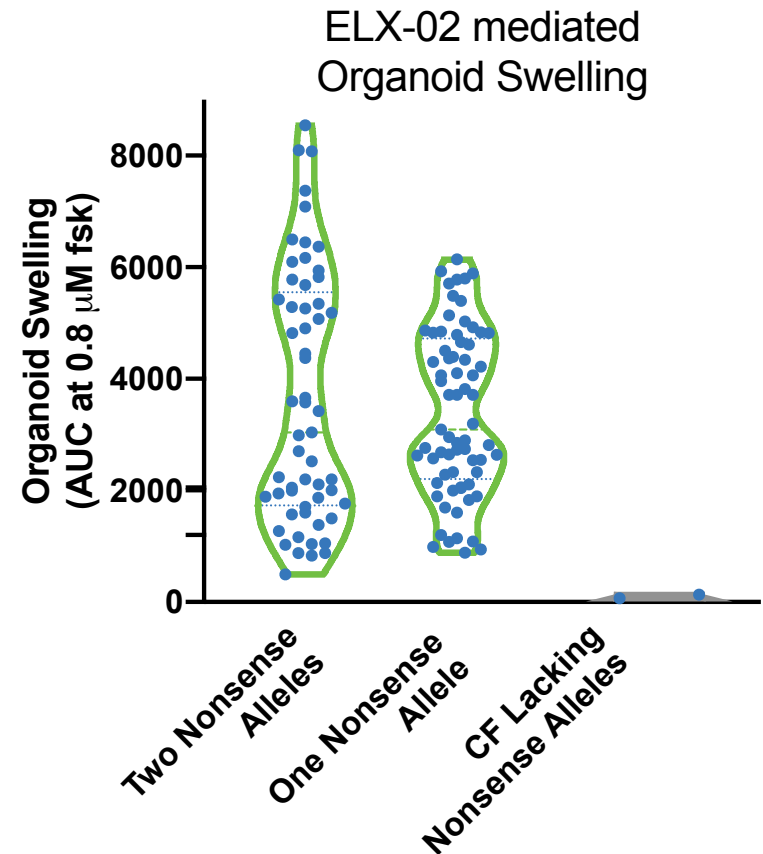
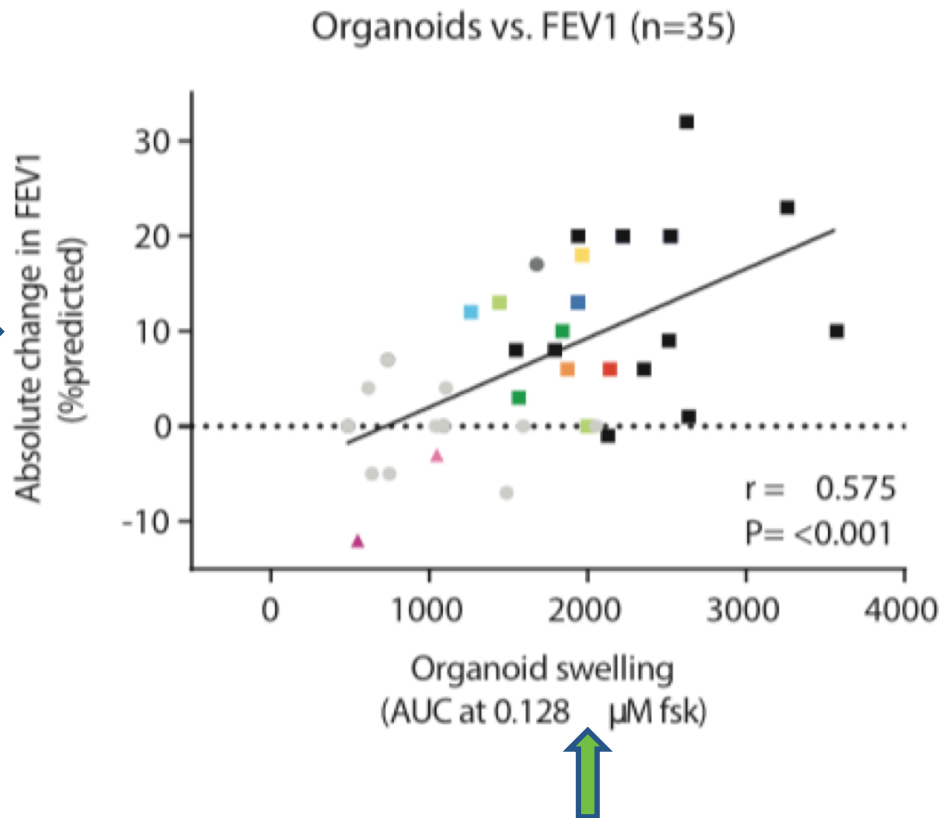
- Genistein & Curcumin (circles)
- Ivacaftor (squares)
- Lumacaftor & Ivacaftor (triangles)

Organoid results correlate with both sweat chloride change

($r = -0.708$, $p < 0.001$)

Berkers et al. Cell Reports (2019) Rectal Organoids Enable Personalized Treatment of Cystic Fibrosis.

ELX-02 response in organoids compares favorably to published results



Berkers et al. Cell Reports (2019) Rectal Organoids Enable Personalized Treatment of Cystic Fibrosis.

Eloxx data on file.

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