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): April 20, 2021

Eloxx Pharmaceuticals, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**001-31326**	**84-1368850**
(State or other jurisdiction of incorporation)	**(Commission File Number)**	**(I.R.S. Employer Identification No.)**

950 Winter Street **Waltham, MA**	**02451**
(Address of principal executive offices)	**(Zip Code)**

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

(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation 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))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.01 par value per share	ELOX	The Nasdaq Global Market

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. ☐

Cautionary Statement Regarding Forward-Looking Statements

This Current Report on Form 8-K 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 Company’s ability to execute and effect its restructuring program; 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; the impact of global health concerns, such as the COVID-19 global pandemic, on our ability to continue our clinical and preclinical programs and otherwise operate our business effectively; the successful integration of acquired companies, such as Zikani Therapeutics; 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.

Item 7.01. Regulation FD Disclosure.

We are furnishing this Current Report on Form 8-K in connection with the disclosure of information from a PowerPoint presentation for use, in whole or in part, at meetings with investors, analysts and other persons. This information may be amended, modified or updated at any time and from time to time through another Current Report on Form 8-K, a later Company filing, or other means, as management of the Company determines.

We do not have, and expressly disclaim, any obligation to release publicly any updates or any changes in our expectations or any change in events, conditions, or circumstances on which any forward-looking statement contained in the presentation is based.

The presentation included with this Current Report on Form 8-K is available on our website located at *www.eloxxpharma.com*, although we reserve the right to discontinue that availability at any time.

The information presented in Item 7.01 of this Current Report on Form 8-K and Exhibit 99.1 shall not be deemed to be "filed" for 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, unless the Company specifically states that the information is to be considered "filed" under the Exchange Act or specifically incorporates it by reference into a filing under the Securities Act of 1933, as amended, or the Exchange Act. By filing this Current Report on Form 8-K and furnishing the information contained herein, the Company makes no admission as to the materiality of any information in this report that is required to be disclosed solely by reason of Regulation FD.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits.

99.1	Presentation Slide Deck April 2021.

SIGNATURE

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: April 20, 2021

By: /s/ Neil S. Belloff
Name: Neil S. Belloff
Title: Chief Operating Officer, General Counsel and Corporate Secretary

Exhibit 99.1

RARE Thinking for RARE Solutions

Creating a World Leader in Ribosome Targeted Genetic Therapies

April 2021

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 readthrough 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; the impact of global health concerns, such as the COVID-19 global pandemic, on our ability to continue our clinical and preclinical programs and otherwise operate our business effectively; including successfully integrating the combined companies; 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 PHARMACEUTICALS

Eloxx + Zikani: Positioned to be the world leader in ribosome RNA-targeted genetic therapies

- **ELX-02:** in Phase 2 development for Cystic Fibrosis (CF)
  - **ELX-02 data readout in 2H 2021**
- Preclinical CF program from Zikani pipeline

- Expect to file IND for **RDEB* and JEB*** program in 2022
- Advance programs in **inherited and advanced colon cancer** targeted at restoring APC* tumor suppressor protein

- President and CEO: **Sumit Aggarwal**
- Head of R&D: **Dr. Vijay Modur**

- **Eukaryotic Ribosome Selective Glycosides (ERSGs):** Safer Aminoglycosides designed with human ribosome selectivity
- **TURBO-ZM™:** Proprietary synthetic chemistry platform to design novel macrolide- based oral Ribosome Modulating Agents (RMAs)

*RDEB and JEB: Recessive Dystrophic and Junctional Epidermolysis Bullosa; APC: Adenomatous Polyposis Coli

Meet the new Eloxx Leadership Team

Sumit Aggarwal
President and CEO

- 20+ years investing and transforming healthcare companies
- Raised >$150M
- Biotech Investor

progenity
McKinsey&Company
Adage Capital Management

Vijay Modur
Head of Research & Development

- 20+ years in translation and drug development
- Led Venglustat rare disease program at Sanofi

MERCK
NOVARTIS
SANOFI GENZYME

Neil Belloff
COO and General Counsel

- 30+ years business and legal experience
- GC and executive leadership roles

Celgene
Deutsche Telekom

Daniel Geffken
Interim Chief Financial Officer

- 30+ years building companies
- Closed $2B in equity and debt financings for public and private companies

Danforth Advisors
Apellis
HOMOLOGY Medicines, Inc.
TKT

Eloxx PHARMACEUTICALS

Transaction overview

Consideration

- Eloxx issued ~ 7.6 million shares
- Zikani stockholders have pro forma ownership ~ 16% of Eloxx

Board changes

- Silvia Noiman, Ph.D., and Martijn Kleijwegt stepped down from Eloxx Board
- Alan Walts, Ph.D., and Raj Parekh, Ph.D., current Zikani directors, appointed to fill vacancies and serve out remaining terms

Strong advisors and collaborators supporting programs

Key collaborators

CYSTIC FIBROSIS FOUNDATION

ecfs

Hubrecht Institute

Rina Arbesfeld
KOL **FAP/APC**
TEL AVIV UNIVERSITY

Mei Chen
KOL **DEB**
Keck School of Medicine of USC

Key advisors

Prof. Eitan Kerem
Cystic Fibrosis
Former Head of Pediatrics Hadassah Hospital
Former SAB member Vertex

Keith Flaherty
Oncology, Chairman SAB
Loxo Oncology co-founder
HARVARD MEDICAL SCHOOL

Pedro Huertas
Clinical Translation
ex-CMO Eloxx, Shire
Eloxx Shire

Dr. David Sidransky
Oncology
Advaxis, Champions Oncology
JOHNS HOPKINS MEDICINE

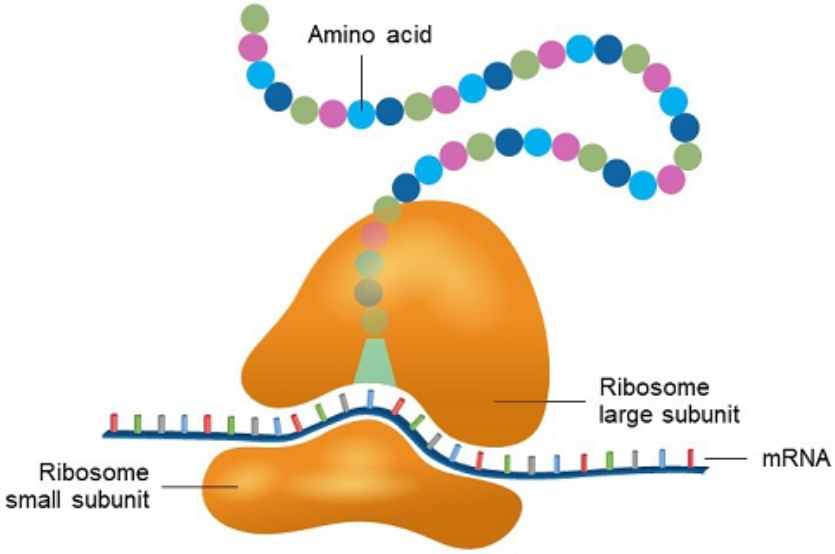
Dr. Andrew South
Epidermolysis Bullosa
Jefferson
Philadelphia University + Thomas Jefferson University

David Bedwell
Readthrough, Rare diseases
UAB THE UNIVERSITY OF ALABAMA AT BIRMINGHAM

Eloxx PHARMACEUTICALS

Complementary human ribosome targeting technologies that address defects in protein translation

Ribosome = "protein factory": Correcting mRNA and ribosomal mutations

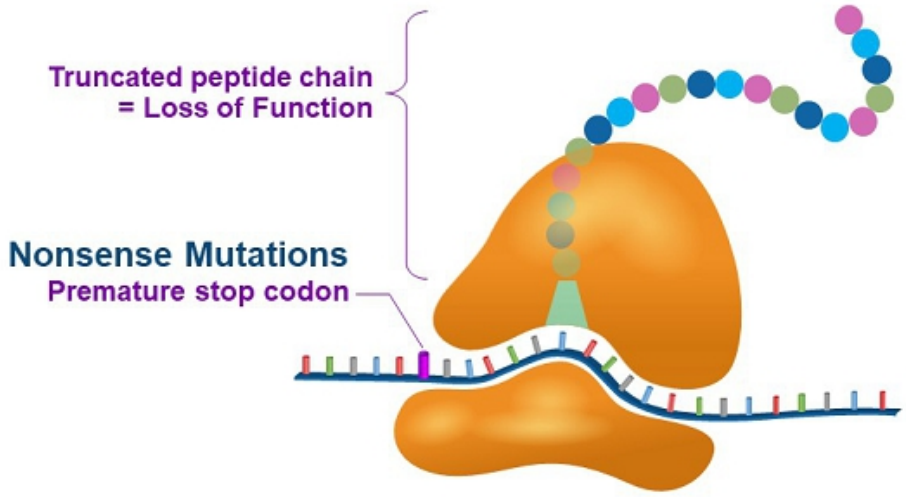


RMA: Ribosome Modulating Agent; **ERSG:** Eukaryotic Ribosome Selective Glycoside

Eloxx PHARMACEUTICALS

Complementary human ribosome targeting technologies that address defects in protein translation

Ribosome = "protein factory": Correcting mRNA and ribosomal mutations



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Eloxx PHARMACEUTICALS

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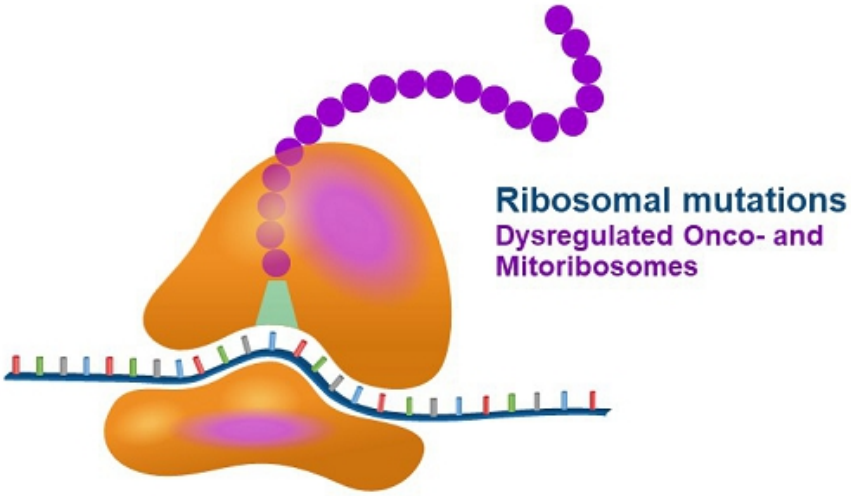

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Eloxx PHARMACEUTICALS

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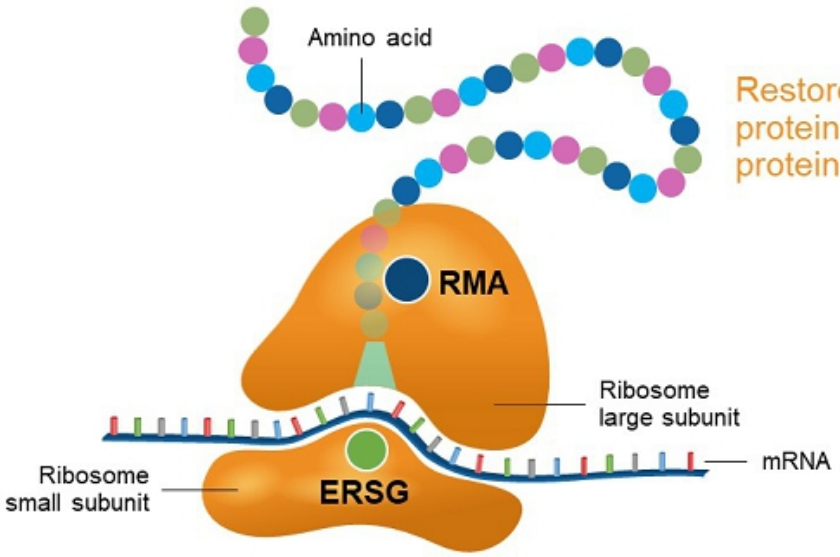


Restore full length proteins **and** inhibit protein translation

RMA: Ribosome Modulating Agent; **ERSG:** Eukaryotic Ribosome Selective Glycoside

Eloxx PHARMACEUTICALS

Strong evidence of readthrough activity with macrolides and aminoglycosides

Clinically relevant readthrough reported in over 36 different rare diseases

Diseases	Evidence	Readthrough Agent(s) Tested	
		Macrolides	Aminoglycosides
Familial Adenomatous Polyposis (FAP)	Clinical[1]	Ery, Tyl	Gen
Cystic Fibrosis Class 1	Clinical[2]	Tyl	Gen, G418
Duchenne Muscular Dystrophy	Clinical[3]		Gen
Dystrophic Epidermolysis Bullosa (RDEB)	Clinical[4]		Gen, G418
Lysosomal Storage Disorders, e.g., MPSI (Hurler), cystinosis	*ex vivo*[5]		Gen, G418
Rett Syndrome	*ex vivo*[5]	Ery	Gen
Spinal Muscular Atrophy (SMA)	*ex vivo*[5]	Azm, Ery	Gen
Ataxia-Telangiectasia (ATM)	*ex vivo*[5]	Ery	Gen
Usher syndrome/retinitis pigmentosa (RP)	*in vivo* Preclinical[6]		Gen, G418

Macrolides: Erythromycin (Ery); Tylosin (Tyl); Azithromycin (Azm)
Aminoglycosides: Gentamicin (Gen); Geneticin (G418)

[1]Kariv, R. Ann. Oncol. 2018, 29, suppl3; [2]Sermet-Gaudelus, I. BMC Med. 2007, 5, 5; [3]Malik, V. Ther. Adv. Neurol. Disord. 2010, 3, 379; [4]Woodley, D. J Clin Invest. 2017;127(8):3028; [5]Caspi, M., J Mol Med (Berl). 2016 Apr;94(4):469-82; [6]Goldmann, T, Hum Gene Ther. 2011 May;22(5):537-47.

Eloxx PHARMACEUTICALS

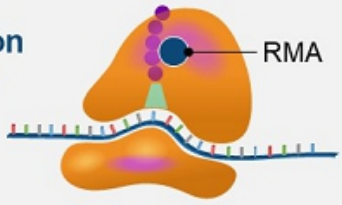
Large and broad applications for human ribosome targeted genetic therapies

Readthrough = "Functional Gene Therapy"

Stop codon readthrough in rare diseases and cancer

- 10–12% of patients across **>1800 rare diseases**
- **6–10% of cancer patients** have nonsense mutations in **tumor suppressor genes**
- **20–30% of neoantigens in cancer patients** have nonsense mutations

Protein Translation Inhibition

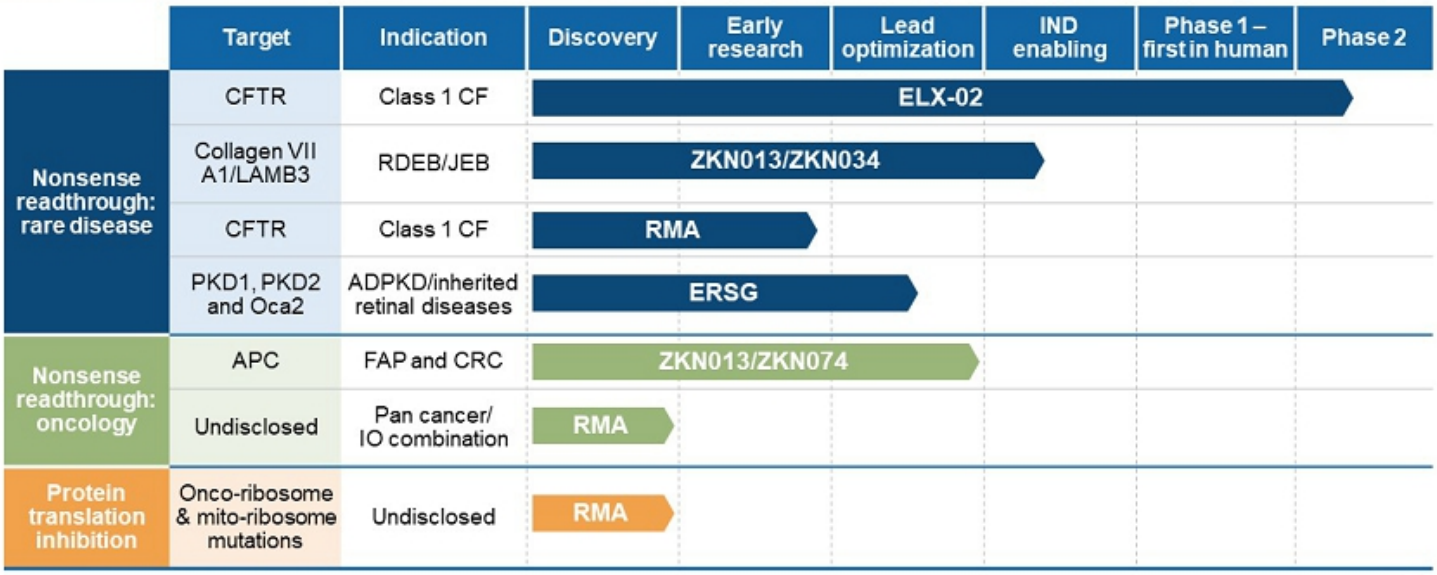


Onco- and mitoribosomal cancers:

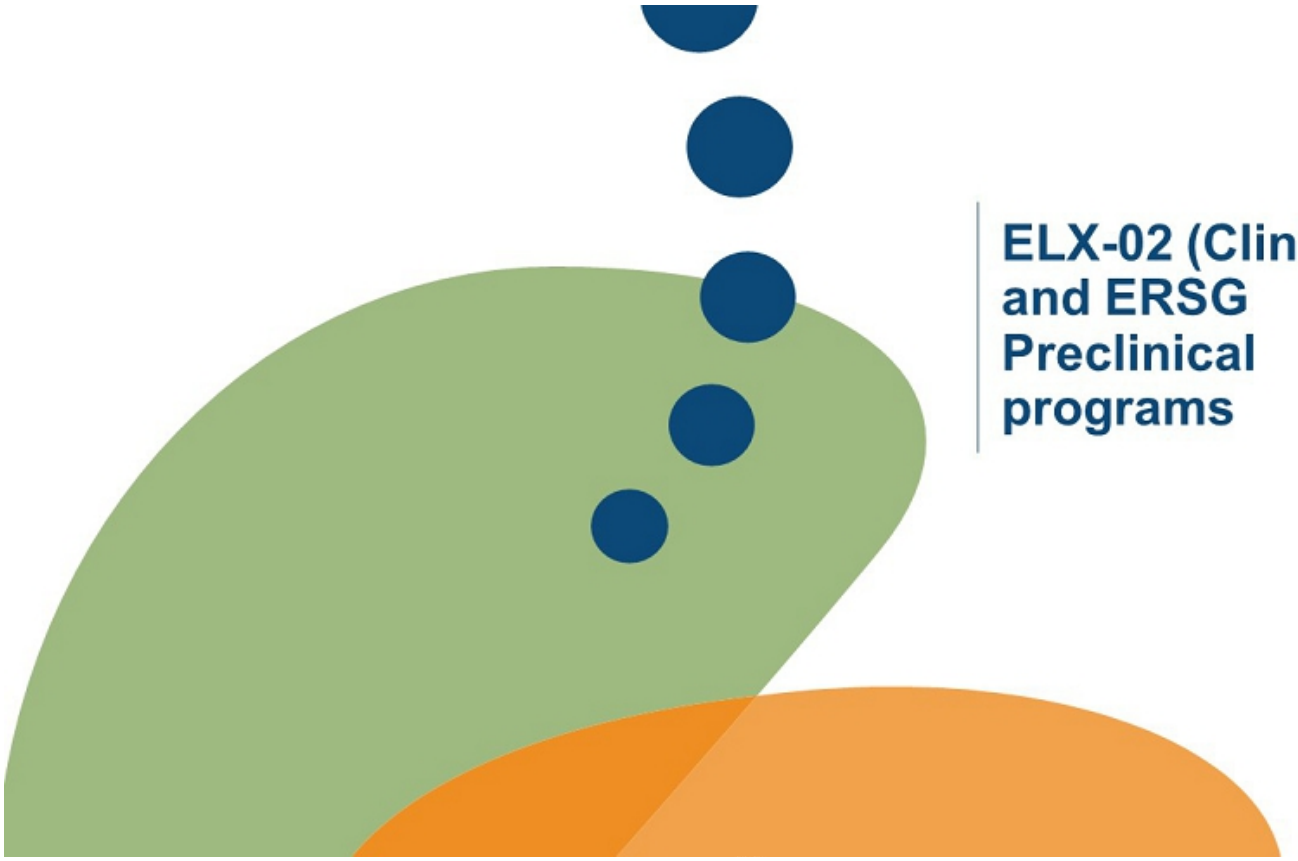
- Myc amplified lymphomas – (e.g., MALT, Burkitt, DLBCL)
- Hereditary Ribosomopathies
- HPV associated cancers (Head & Neck, Cervical)
- Triple Negative Breast, KRAS mutated Pancreatic Cancer, etc.

Eloxx PHARMACEUTICALS

Deep pipeline of synergistic potential first-in-class therapies



*Class 1 CF: Cystic fibrosis patients with class1 mutations; ADPKD: Autosomal dominant polycystic kidney disease; FAP: Familial adenomatous polyposis; CRC: Colorectal cancer

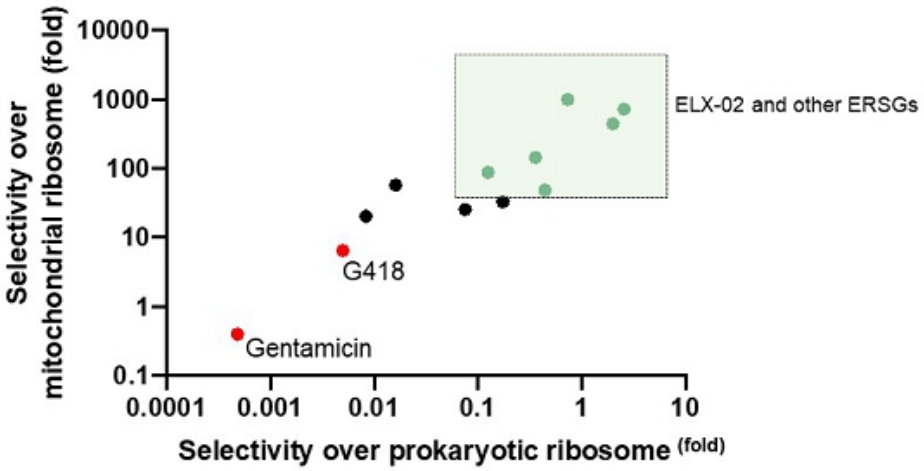


ELX-02 (Clinical) and ERSG Preclinical programs

Eloxx PHARMACEUTICALS

ERSGs like ELX-02 designed to expand human ribosome selectivity and therapeutic index over aminoglycosides

Eukaryotic ribosome selectivity comparison



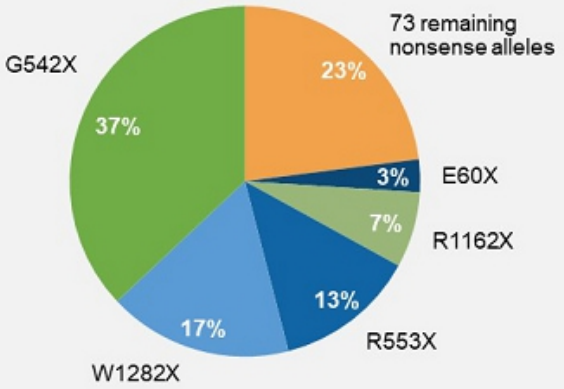
Data adapted from: Kandasamy. Increased Selectivity toward Cytoplasmic versus Mitochondrial Ribosome Confers Improved Efficiency of Synthetic Aminoglycosides in Fixing Damaged Genes: A Strategy for Treatment of Genetic Diseases Caused by Nonsense Mutations. *J. Med. Chem.* (2012)

Clinical validation for gentamicin supports ELX-02 use in treating Class 1 Cystic Fibrosis nonsense mutation patients

Class 1 CF opportunity and clinical rationale

N=12% of all CF patients[1]

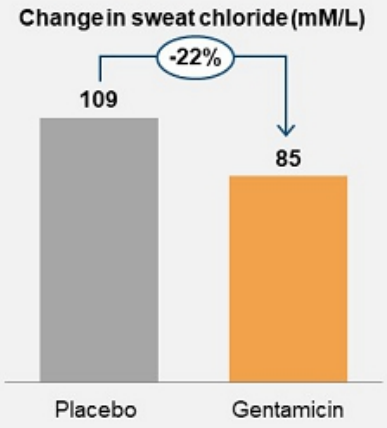
Nonsense allele frequency of global CF patient population



Class 1 CF

Gentamicin treatment 9 patients

(10 mg/kg iv, qd for 15 days)[2]

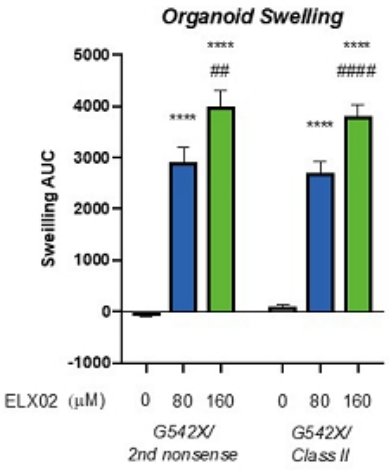


No currently approved drugs to treat CFTR nonsense mutations

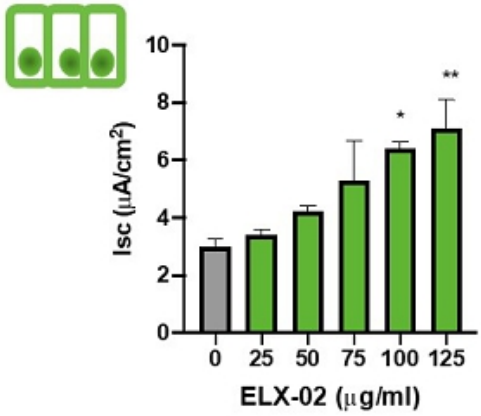
[1] Allelic frequency based on CFTR2 database (July 2020); CF population data based on 2019 Patient Registry Report.
[2] Sermet-Gaudelus, I. *BMC Med.* 2007, 5, 5;

High ELX-02 efficacy in organoid swelling and Ussing chamber experiments

Swelling response in G542X CF patient organoids[1]



Ussing chamber results heterozygous G542X/Fdel508 HBE cells



*p<0.05, **p<0.01
HBE cells were incubated for 2 days with ELX-02

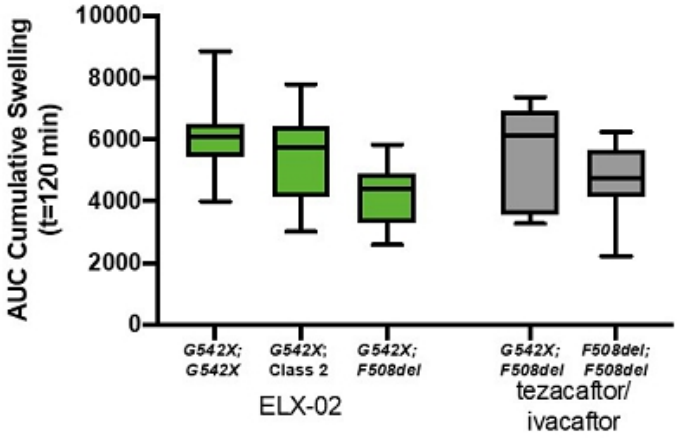
[1]Ordinary one-way ANOVA with Tukey's multiple comparison testing was used; **** p<0.0001 versus vehicle control, ## p<0.01 versus next lower concentration, #### p<0.0001 versus next lower concentration. Data represent >40 biological replicates per group from 2 or 4 independent patient organoids, respectively.

Eloxx PHARMACEUTICALS

Swelling response in Class 1 CF organoids with ELX-02 compares favorably with Symdeko in Class 2 organoids

Swelling response in Class 1 and Class 2 CF patient organoids when treated with ELX-02 vs. Symdeko

Organoid swelling (0.8 µM forskolin)

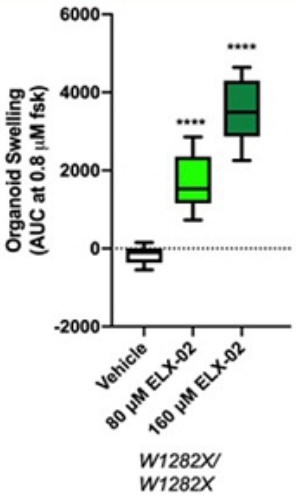


ClinicalTrials.gov Identifier: US Trial NCT04135495, EU/IL Trial NCT04126473
* From baseline to Day 7 of treatment periods 1-3, and Days 7 and 14 of treatment period 4
** Lancet Respir Med. 2014 Jul;2(7):527-38., *N Engl J Med.* 2010 Nov 18; 363(21): 1991–2003.

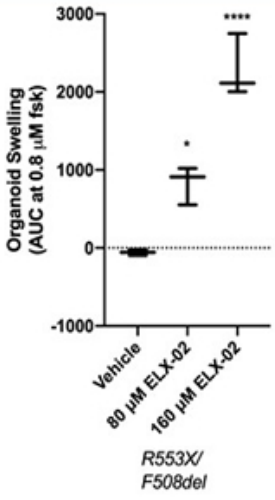
ELX-02 efficacy seen across CFTR nonsense mutations

Swelling response in CF patient organoids

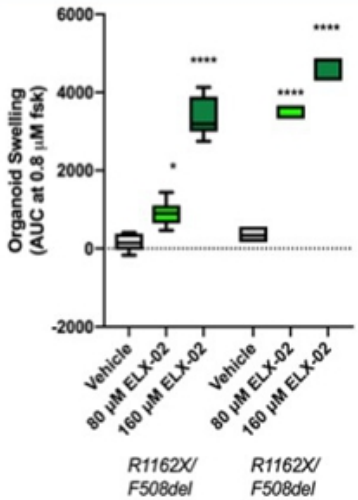
W1282X response example



R553X response example



R1162X response example

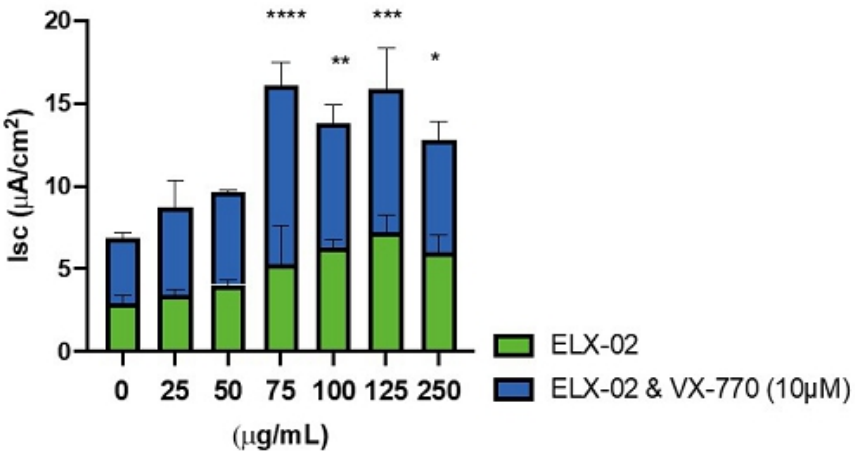


Eloxx PHARMACEUTICALS

ELX-02 shows synergy with Kalydeco (VX770- a potentiator)

Ussing Chamber experiment: Treatment of Het G542X HBE cells

G542X/F508del



ELX-02 well tolerated in Phase 1 and 2 clinical studies

>100 subjects exposed to ELX-02 to date with NO treatment-related serious adverse events or off target effects reported

PHASE 1: Healthy volunteers and subjects with various severities of renal dysfunction

- Single and Multiple ascending doses studied from 0.3 to 7.5 mg/kg. **Generally well tolerated**
- No dose limiting toxicities, SAEs or off target effects
  - No nephrotoxicity or vestibular toxicity
- Most common AE was mild injection site reaction
- 5 transient and reversible cases of high frequency audiometry shift
- Highly reproducible PK over the dose range studied

PHASE 2: Nephropathic cystinosis with homozygous CTNS W138X who previously received kidney transplant

- **Generally well tolerated:** No treatment-related serious adverse events, nephrotoxicity, or ototoxicity
- Mild injection site reactions reported
- No meaningful changes in eGFR or serum creatinine
  - Consistent with preservation of kidney function
- Pharmacokinetics consistent with previous studies in healthy volunteers
- **Safety Review Committee approval to enroll patients ages 12 years and older**

Data presented at scientific meetings in 2019 and published in peer reviewed journals in 2021

Phase I trial records: NCT03776539, NCT03309605, NCT03292302,
Phase II trial record: NCT04069260

Eloxx PHARMACEUTICALS

ELX-02 Phase 2 cystic fibrosis trial designed to evaluate safety and short term sweat chloride reduction

ELX-02 Phase 2 program principally designed for safety and dose finding in CF patients, with sweat chloride as PD biomarker

Population
- Up to 24 CF patients with a *G542X* mutation on one or both alleles

Primary outcome measures
- Safety, tolerability, and pharmacokinetics

Secondary outcome measures
- Change from baseline in sweat chloride concentration*
- Change from baseline in percent predicted forced expiratory volume (ppFEV1)*

Locations
- Europe, Israel & USA, opening additional sites in Canada and Australia

DESIGN: Intra-patient dose escalation through 4 increasing doses of ELX-02

Dose 1
0.3 mg/kg
SC QD for 7 days

Dose 2
0.75 mg/kg
SC QD for 7 days

Dose 3
1.5 mg/kg
SC QD for 7 days

Dose 4 (individualized)
Up to 3 mg/kg
SC QD for 14 days

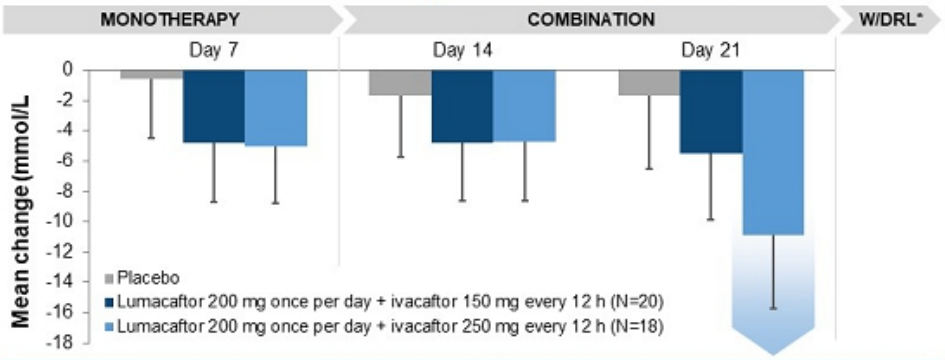
ClinicalTrials.gov Identifier: US Trial NCT04135495, EU/IL Trial NCT04126473
* From baseline to Day 7 of treatment periods 1-3, and Days 7 and 14 of treatment period 4

Eloxx PHARMACEUTICALS

ORKAMBI confirmed sweat chloride reduction, but no FEV_1 change in similar small P2 safety trial

Orkambi Ph 2 (Cohort 1) trial – Changes in sweat chloride

Treatment in homozygous delta F508 CF patients



Sweat chloride change at day 21 is decreased (–10·9 mmol/L) for high dose group

FEV_1 – Ph2

FEV_1 improvement is not significant for any dose group in small short trial

FEV_1 – Ph3

Larger and longer ORKAMBI Ph3 trial showed +2.6% FEV_1 change leading to its approval

P2 trials for Kalydeco and Orkambi found changes in sweat chloride to be less variable in small studies than FEV_1/ppFEV_1, which were not significant in many cohorts

* **W/DRL** = Withdrawal period

Lancet Respir Med. 2014 Jul;2(7):527-38. doi: 10.1016/S2213-2600(14)70132-8

Eloxx PHARMACEUTICALS

ELX-02: Potential for transformative efficacy in Class 1 CF patients

Demonstrated efficacy in clinically relevant pre-clinical models	• Swelling response in Class 1 CF patient organoids • Induces CFTR activity of up to 30% of normal; confirmed in Ussing Chamber • Active across broad range of mutations
Safety demonstrated in clinical studies	• Generally well-tolerated for chronic dosing, with no serious adverse events in over 100 subjects exposed to ELX-02 to date • Consistent pharmacokinetics across both single and multiple-dose accumulations
Phase 2 CF trials designed for rapid clinical signal	• Study designed to confirm safety and biological activity via changes in sweat chloride • Funding provided by Cystic Fibrosis Foundation (CFF), sanctioned by CFF-TDN & ECFS-CTN (high priority ranking)

Expect to complete enrollment in Phase 2 clinical trials by midyear and report data in 2H 2021

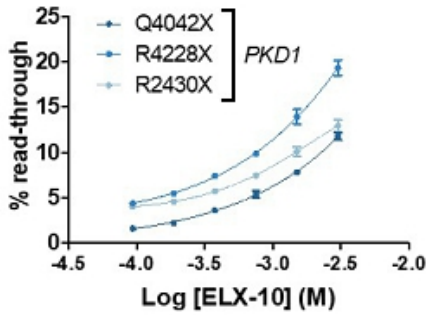
CYSTIC FIBROSIS FOUNDATION
ADDING TOMORROWS

Eloxx PHARMACEUTICALS

Readthrough shown in preclinical models with other ERSG's in rare kidney and ocular diseases

Polycystic Kidney Disease (ADPKD)

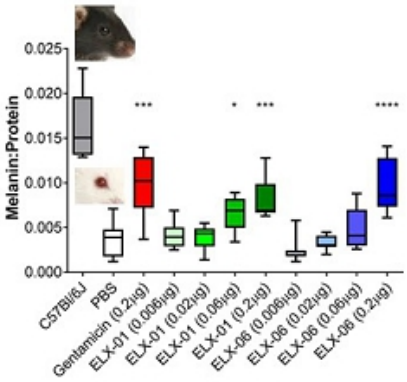
Readthrough in nonsense mutations in PKD1 gene with ELX-10 in dual luciferase assay



- Mutations in PKD1 or PKD2 lead to cyst growth
- **Affects 6–10% of patients on dialysis** and renal transplant in the US

Inherited Retinal Diseases (IRDs)

Single dose study in SJL/J mouse with R262X mutation in OCA2 gene 1



- Usher syndrome and Retinitis Pigmentosa are most common diseases
- **Approx. 2000 patients in the US**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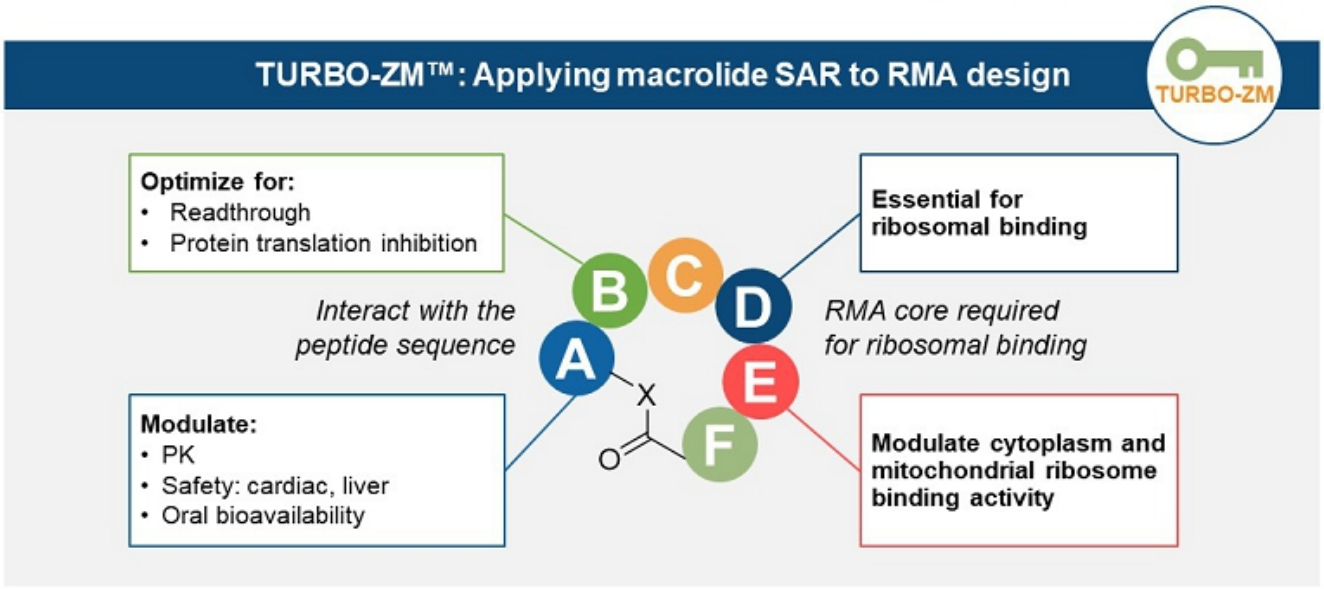
TURBO-ZM™ (TUning the RiBOsome with Zikani Molecules) platform fully unlocks the potential of macrolides



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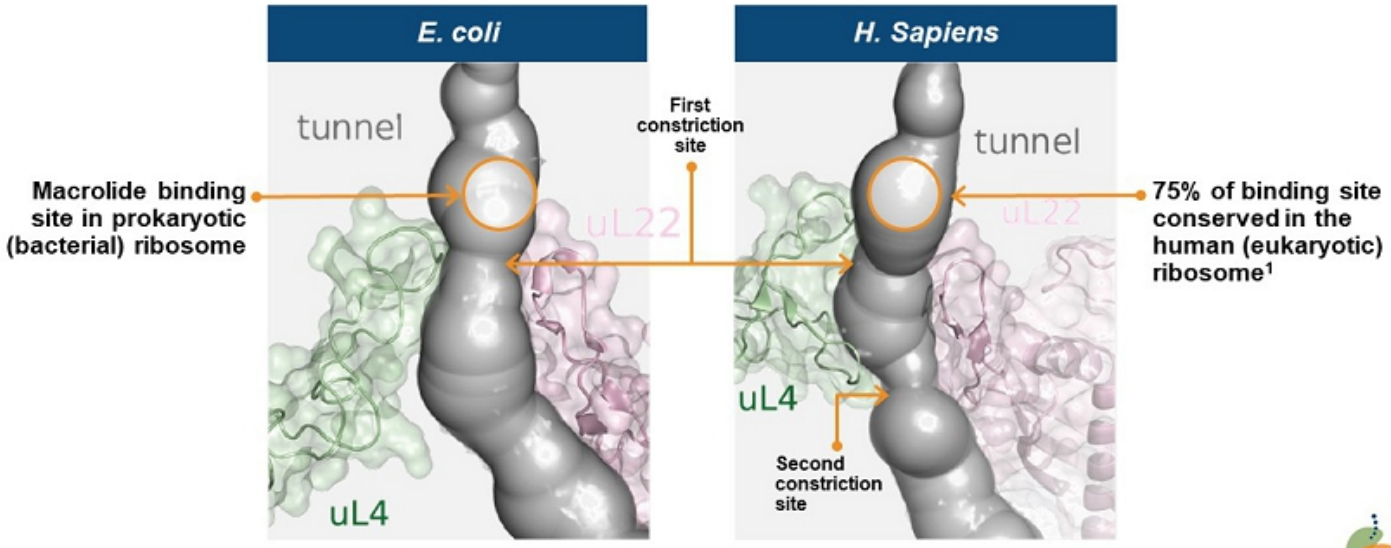
Strong rationale for macrolides to bind to the human ribosome

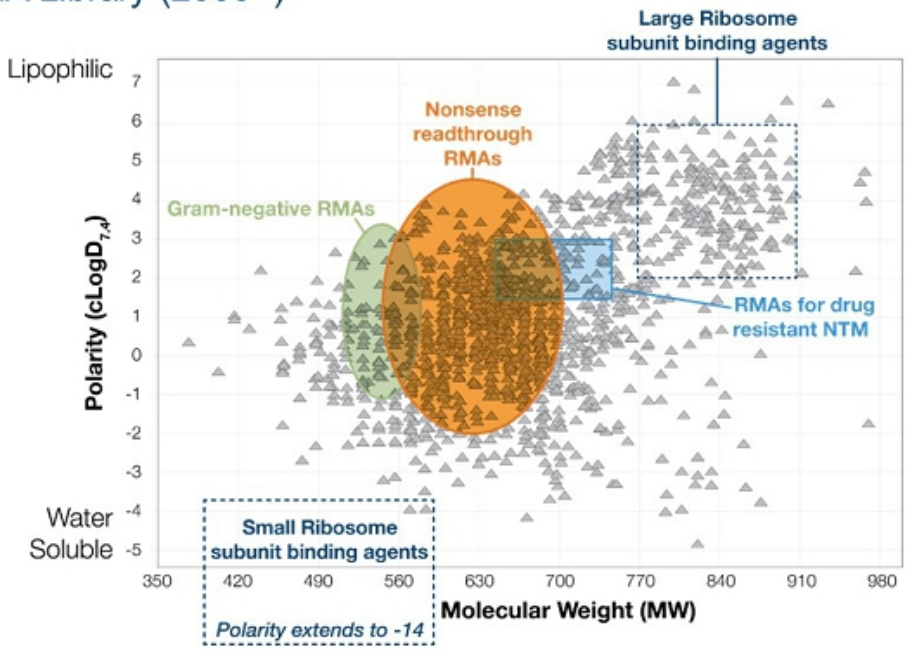
Nascent peptide exit tunnel in *E. coli* vs. human ribosomes[1]



[1]*Nucleic Acids Res.* 2019;47(8):4198-4210

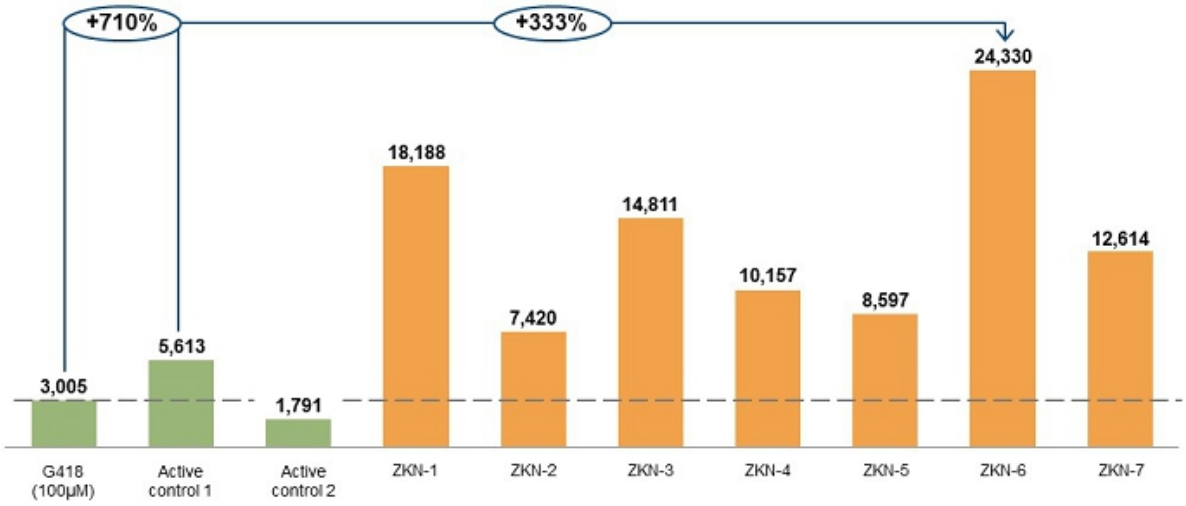
Growing library of RMAs with drug-like properties

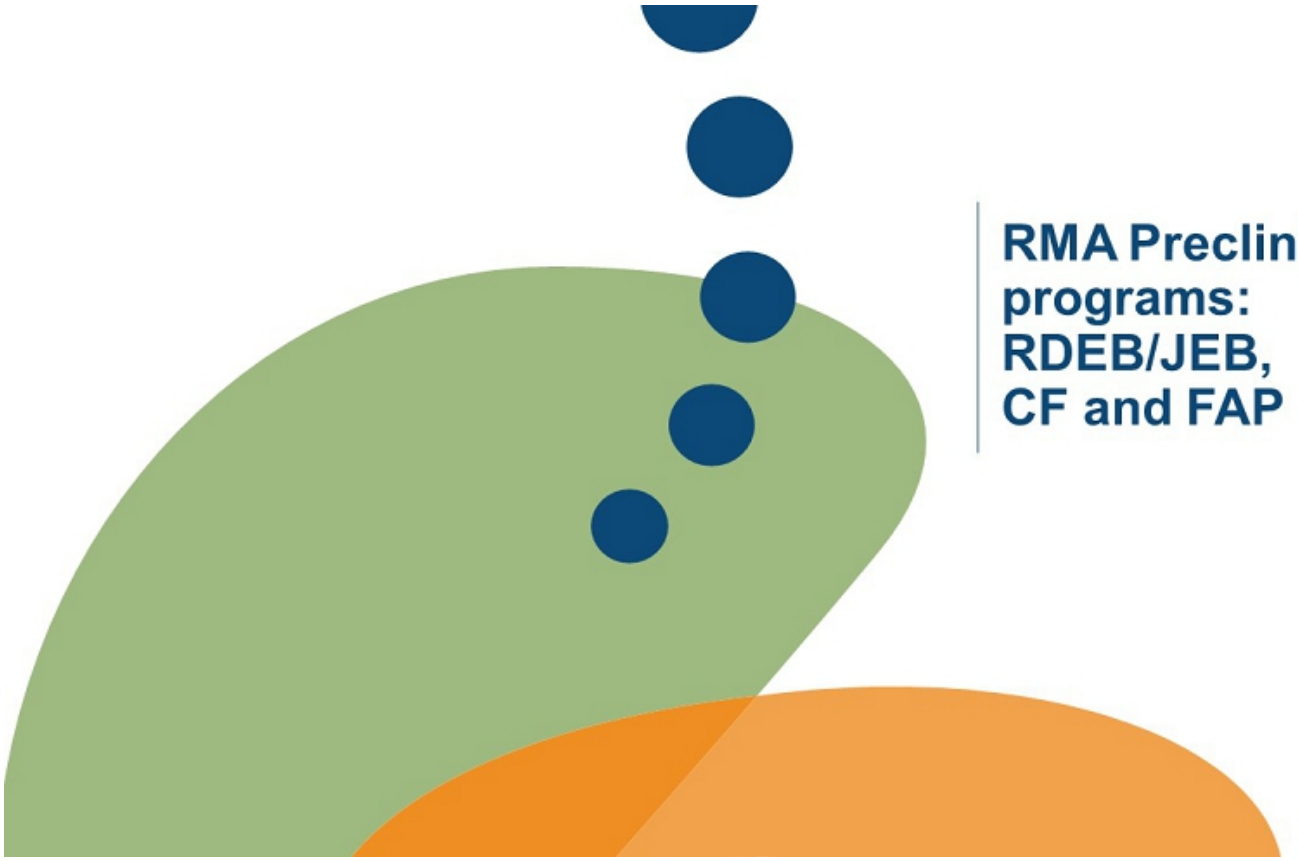
Zikani RMA Library (2000+)



RMAs show superior readthrough to alternatives

Readthrough Emax of selected RMA hits relative luciferase units compared to DMSO in W134X Nanoluc reporter assay





RMA Preclinical programs: RDEB/JEB, CF and FAP

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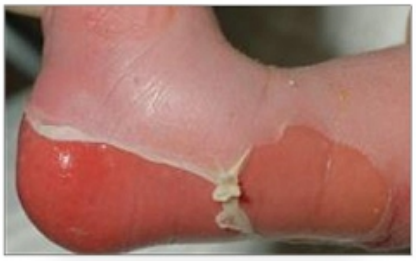
RDEB/JEB: Clinically validated path for RMAs in rare skin disease targeting patients with nonsense mutations

RDEB and JEB



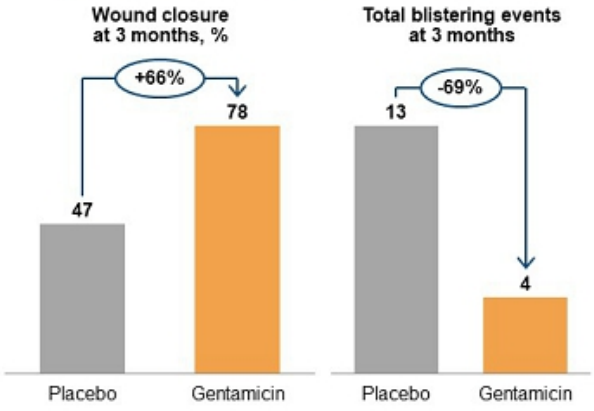
- Mutations in COL7A1 gene (Collagen) and LAMB3 (Laminin)
- Most RDEB patient develop skin cancer by age 35
- Average mortality of JEB patients is 18 months

~4,000 patients, $1.5B TAM

RDEB

Gentamicin treatment of RDEB patients

(0.1% gentamicin ointment tid for 2 weeks; n=5)[1]



RDEB and JEB: Recessive Dystrophic and Junctional Epidermolysis Bullosa

[1]Woodley, DT. *J Clin Investig* 2017, 127, 3028-3038; [2] Hammersen, Johanna et al. *Dermatology* May 2019

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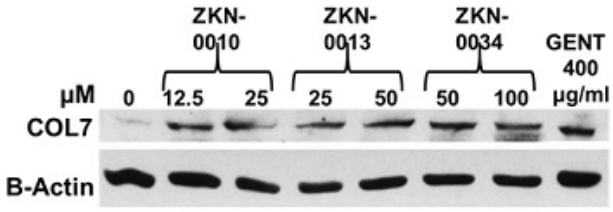
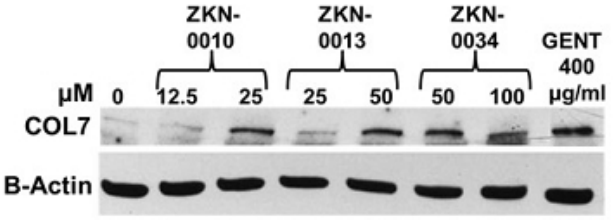
RDEB: RMAs restore functional collagen protein in primary patient cells comparable to high dose gentamicin

COL7 with 48 hr. exposure in RDEB patient derived **primary** fibroblasts*

Full length protein in Hom R578X COL7A Fibroblasts**



Full length protein in R613X/R1683X COL7A Fibroblasts**



Data generated in collaboration with academic partner

- **Assay with proven translation to clinic**
- **30 to 60-day Col7 protein half-life**
- **RMAs compounds exceed clinical efficacy threshold of 10% Gentamicin 845uM**

* Fibroblasts isolated from patients two and five in gentamicin clinical trial. *J Clin Invest* 2017, 127, 3028-3038
** 48 hours treatment with media and compounds replaced and refreshed at 24 hours. Study repeated twice with equivalent results.

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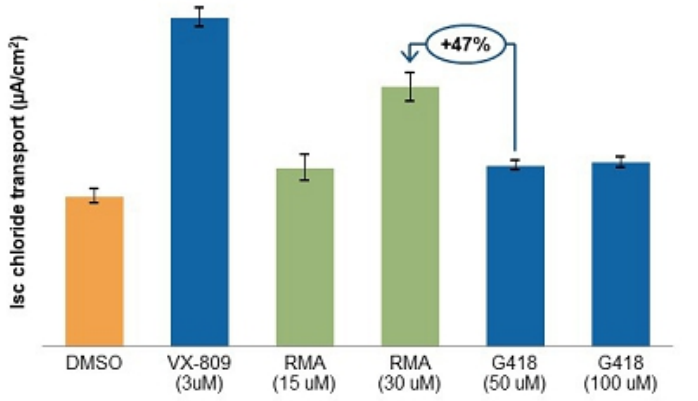
Class 1 CF: RMA lead showed highest ever readthrough preclinical Ussing chamber assay

Summary of Class 1 CF data

Het G542X Human Broncho Epithelial (HBE) cells Ussing Chamber steady state modulator response measurement**



Data generated at Chantest

CYSTIC FIBROSIS FOUNDATION

Never seen before impressive single agent activity from non aminoglycoside class – need to advance this program

– CF Foundation

Encouraged to apply for "Path to Cures"

Submitted $2.5M grant to CF Foundation to support through development candidate

*Forskolin 10 μM/1μM VX-770 - both chambers
**VX 809 and RMA data averaged from 2 separate Ussing chamber results

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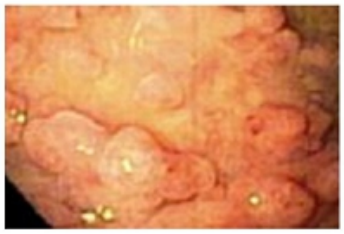
APC readthrough: Supported by positive prior clinical success of Erythromycin in FAP

APC mutant Familial Adenomatous Polyposis (FAP) and CRC

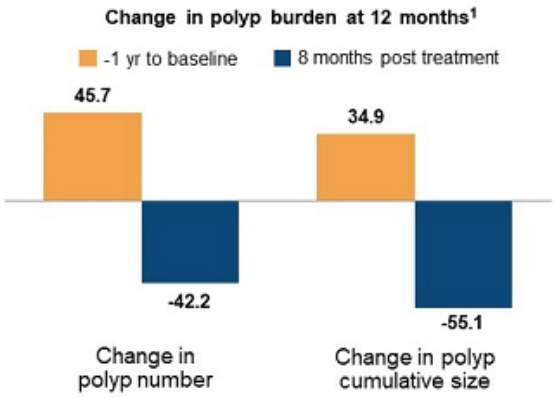


- Mutations in the Adenomatous Polyposis Coli (APC) gene (tumor suppressor gene)
- FAP patients develop CRC by age 40
- 80% of CRC patients have an APC mutation

8,000–12,500 FAP patients in the US/EU; 210,000 CRC patients WW

Clinical trial success in FAP with Erythromycin

Erythromycin treatment
(250 mg/day po for 4 months)

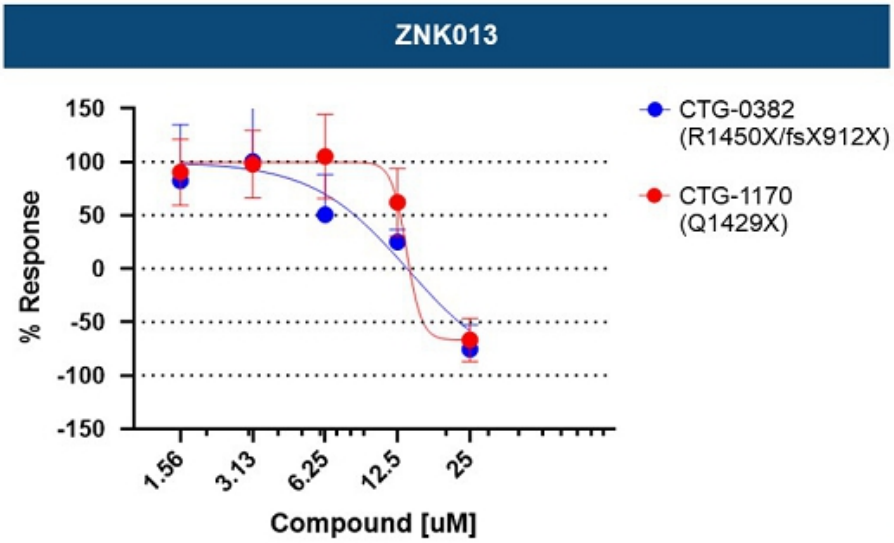


[1]Kariv, R. *Int J Cancer*. 2019 doi: 10.1002/ijc.32557

Clear path treating FAP supported by efficacy in APC mutant cancer patient tumor grafts

Efficacy of ZN013 in colorectal cancer patient derived tumor grafts ex-vivo

- Ex-vivo sensitivity assessment in tumor grafts
- Potent tumor growth inhibition
  - GI50<15uM
- Cancer xenograft studies planned in 2021



Positioned to be the world leader in ribosome targeted genetic therapies

- Proprietary ribosome targeted small molecule platforms targeting rare diseases and oncology
- Deep pipeline led by clinical stage program to treat class 1 nonsense mutations in Cystic Fibrosis
- Expect to file first IND for first-in-class oral RDEB/JEB program expected in 2022; expect to file 1 IND per year after 2022
- Right leadership, team, and advisors

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