ELOXX
Eloxx Pharmaceuticals

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

Canaccord Genuity Growth Conference

August 8, 2019

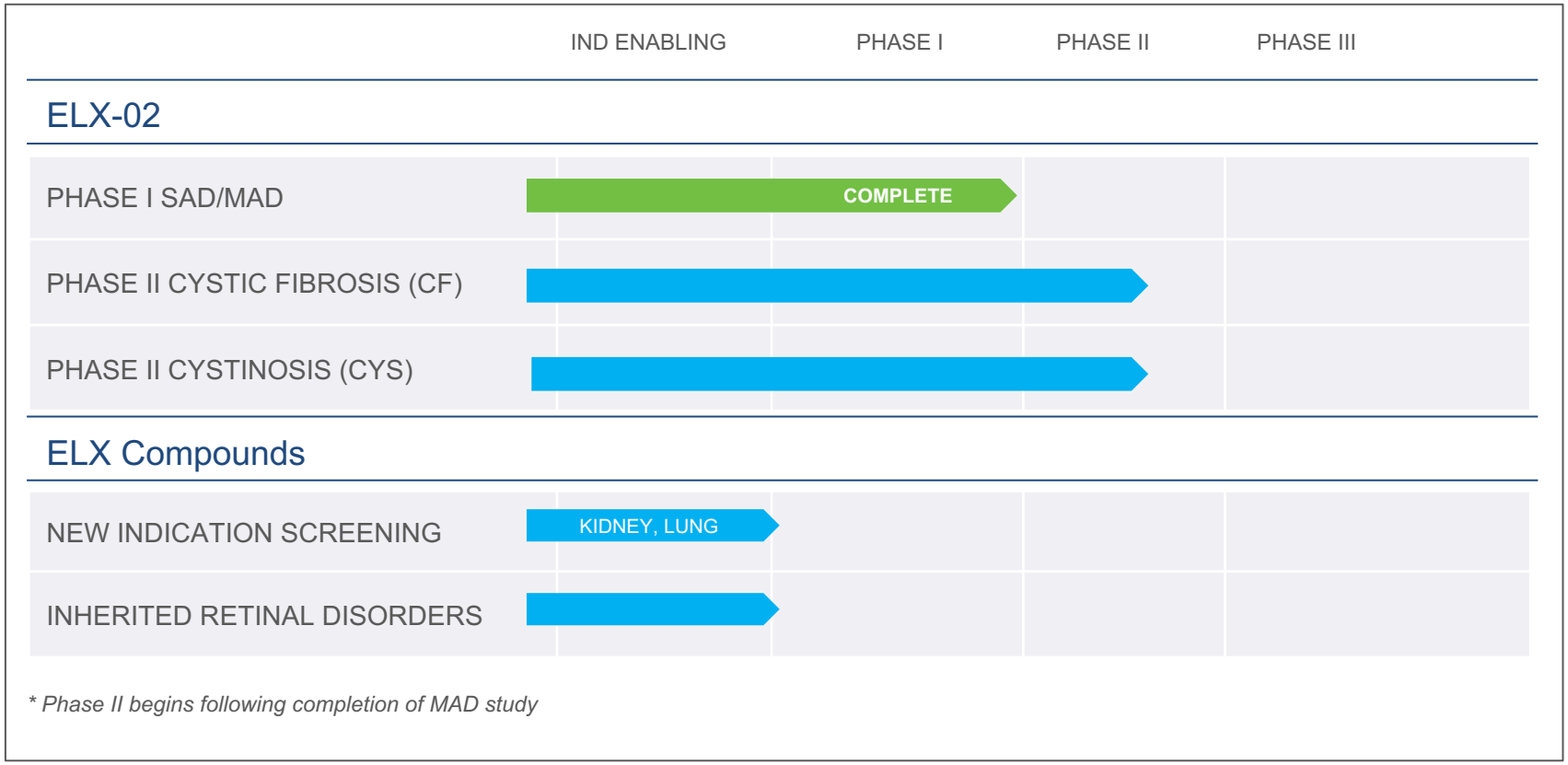
Forward-Looking Statements

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**
- **Phase 2 ELX-02 Clinical Trial in Cystic Fibrosis**
  - U.S. IND Open
  - Cystic Fibrosis Foundation Endorsed Protocol
  - Dr. Ahmet Uluer, Director of Adult Cystic Fibrosis Program, Boston Children's Hospital, Lead U.S. Investigator
  - Dr. Eitan Kerem, Head of Division of Pediatrics, Children's Hospital, Haddassah Medical Center, Global Lead Investigator
- **Phase 2 ELX-02 Clinical Trial in Cystinosis**
  - Dr. Paul Goodyer, Professor of Pediatrics, McGill University, Principal Investigator
  - Non-dilutive funding from Genome Quebec and Genome Canada
- **Topline ELX-02 Phase 2 Data in Cystic Fibrosis and Cystinosis in 2019**
- **New Indication Screening Ongoing (Eye, Kidney, Lung)**
- **Actively Developing Business Development Opportunities to Advance Full Pipeline and Expand Therapeutic Programs**

Eloxx Pipeline



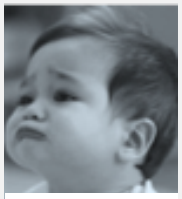
** Phase II begins following completion of MAD study*

The Potential for Read-Through of Rare Genetic Diseases

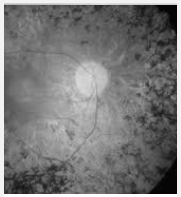
>1,800

Genetic diseases involve nonsense mutations

Cystic Fibrosis



Cystinosis



Retinitis Pigmentosa

Usher Syndrome

Primary Ciliary Dyskinesia

Polycystic Kidney Disease

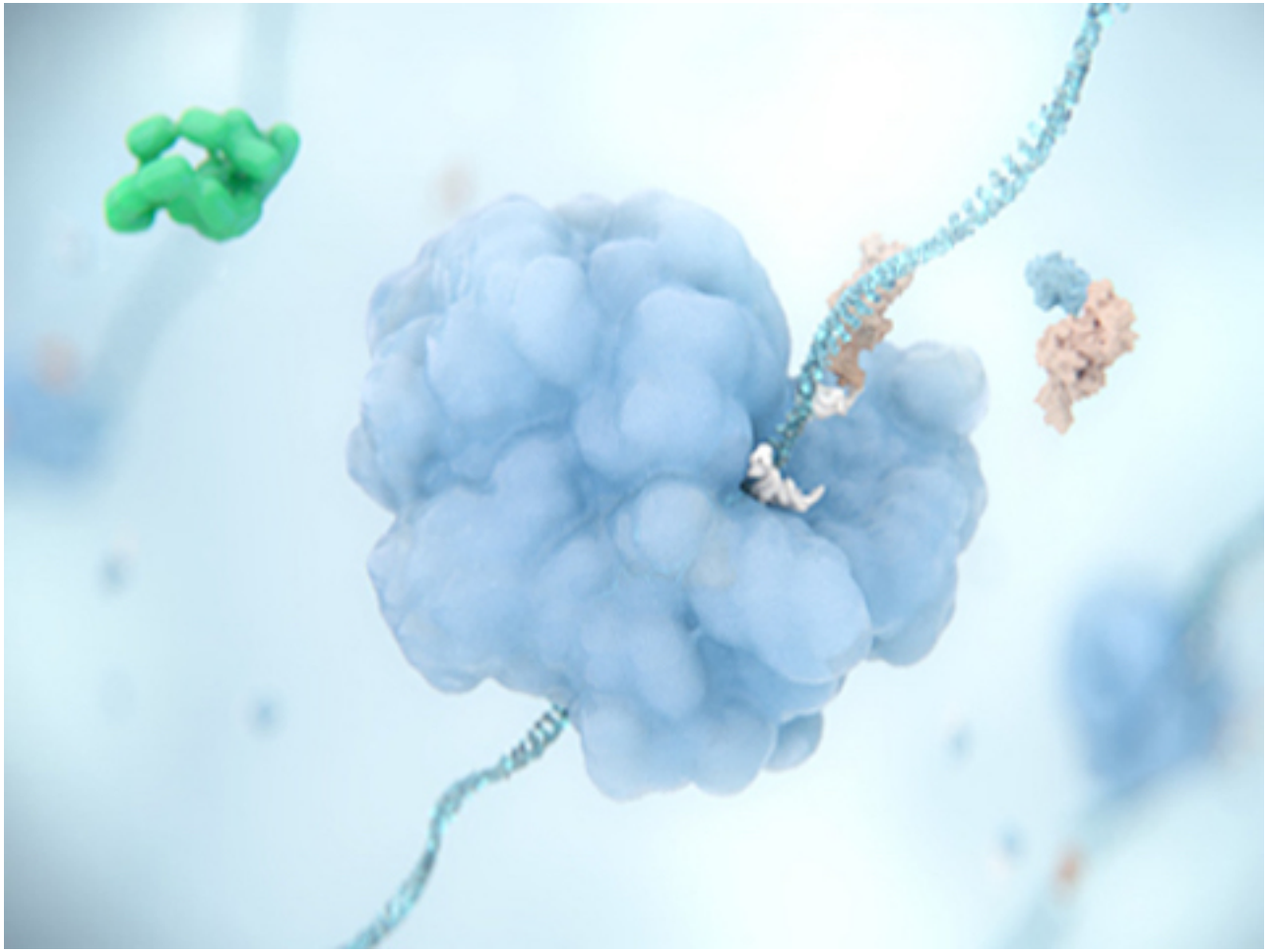
- In every genetic disease a subset of patients have nonsense mutations that impair the production of essential proteins
- Translational read-through is directed at restoring the production of full length proteins by overcoming the premature stop codon and nonsense mediated decay

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

ELOXX proprietary library of novel read-through product candidates have been evaluated for activity in over 10 rare disease areas

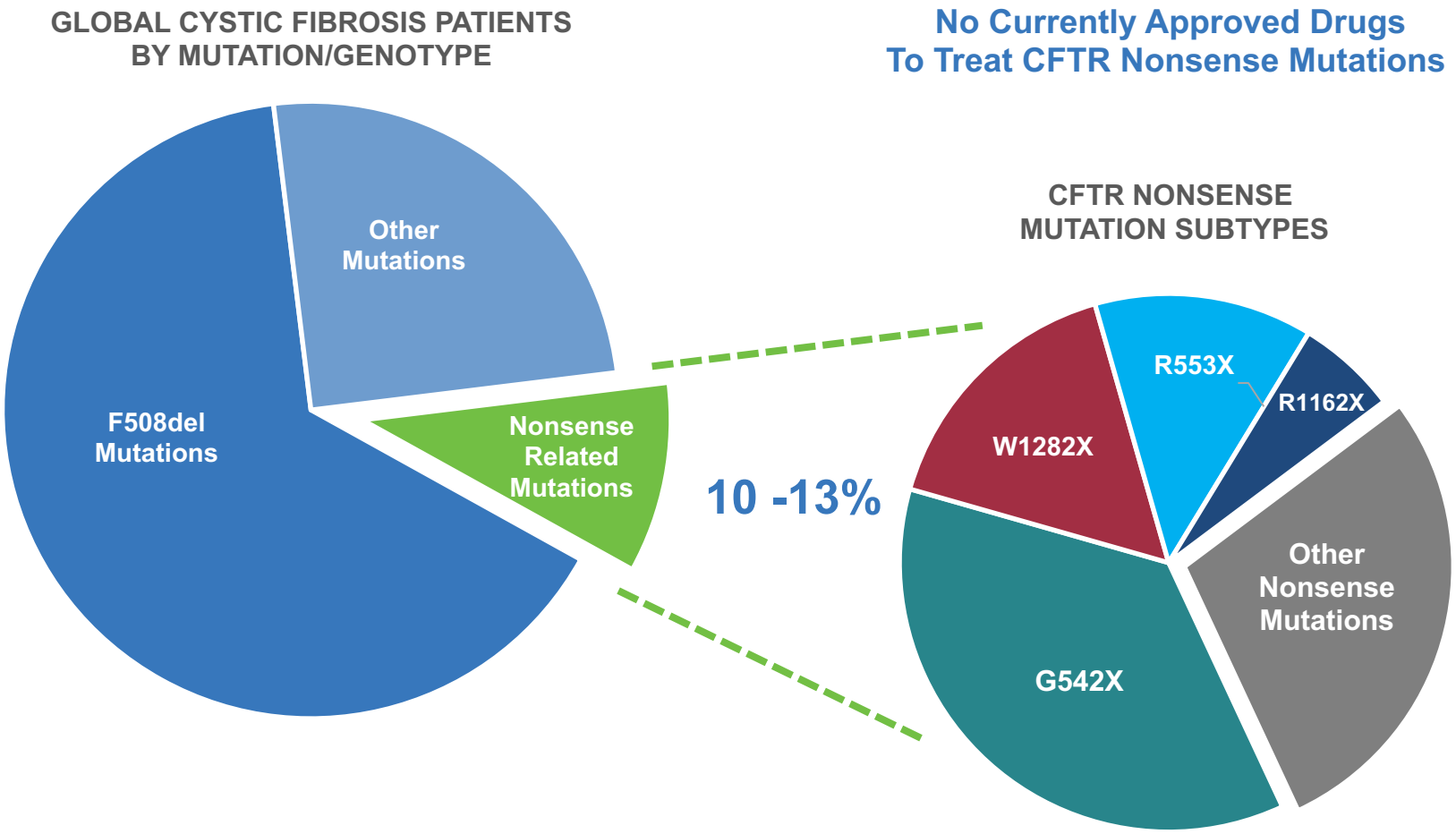
ELOXX is advancing molecules for subcutaneous and intravitreal dosing for diseases of the lung, kidney, eye and other target organs

Eukaryotic Ribosomal Selective Glycosides (ERSGs) Nonsense Mutation Suppressors Rescue Full-Length Protein



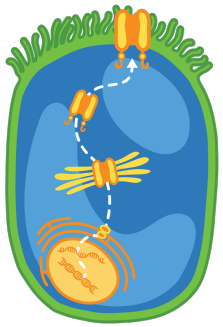
WATCH THE VIDEO: https://we.tl/t-WWxx4i3tzR

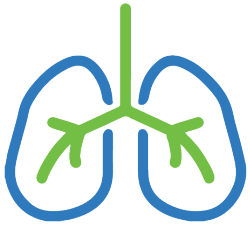
Cystic Fibrosis Nonsense Mutation Patients Represent a Key Unmet Need Population



Source: Eloxx Internal Research/CFTR2 database

ELX-02: ERSG In Development For Nonsense Mediated Cystic Fibrosis

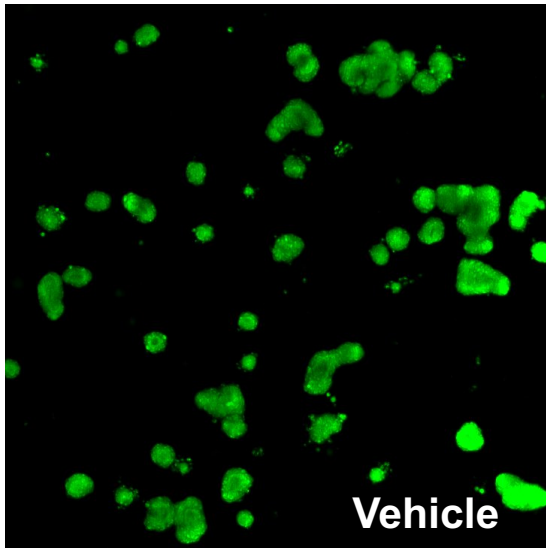




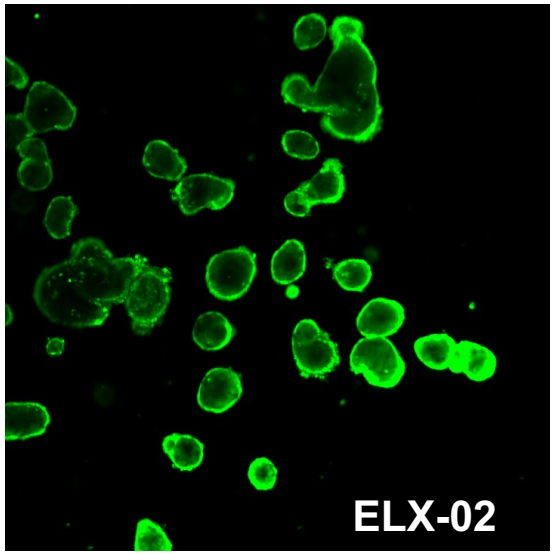
- ELX-02 is a small molecule that permits read-through of nonsense mutations
  - Defined interaction with the ribosome
  - High selectivity for the eukaryotic cytoplasmic ribosome relative to mitochondrial ribosome
- ELX-02 demonstrates read-through in multiple cystic fibrosis model systems
  - Pronounced *CFTR* read-through demonstrated in plasmid, HBE, FRT, transgenic mice and patient-derived organoids
  - Significant read-through demonstrated for *G542X*, *W1282X*, *R553X*, *R1162X*, and *E60X* alleles which represent the most common nonsense population
  - Defined MOA: Demonstrated significant increases in CFTR function, protein and *mRNA*
- ELX-02 is progressing to Phase 2 clinical studies
  - Phase 2 focus on patients with *G542X* genotype
  - Planned topline patient data will be reported in 2019

Organoid Swelling Assay Demonstrates CFTR Function

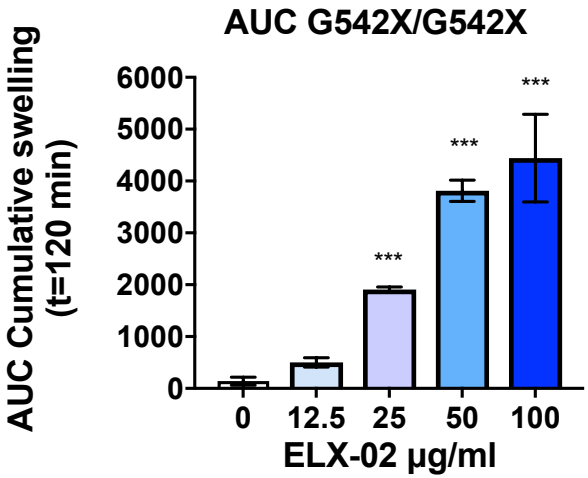
Cystic Fibrosis Organoid dose-responsive swelling assay response



Patient Organoid without drug treatment: No Swelling of Organoids

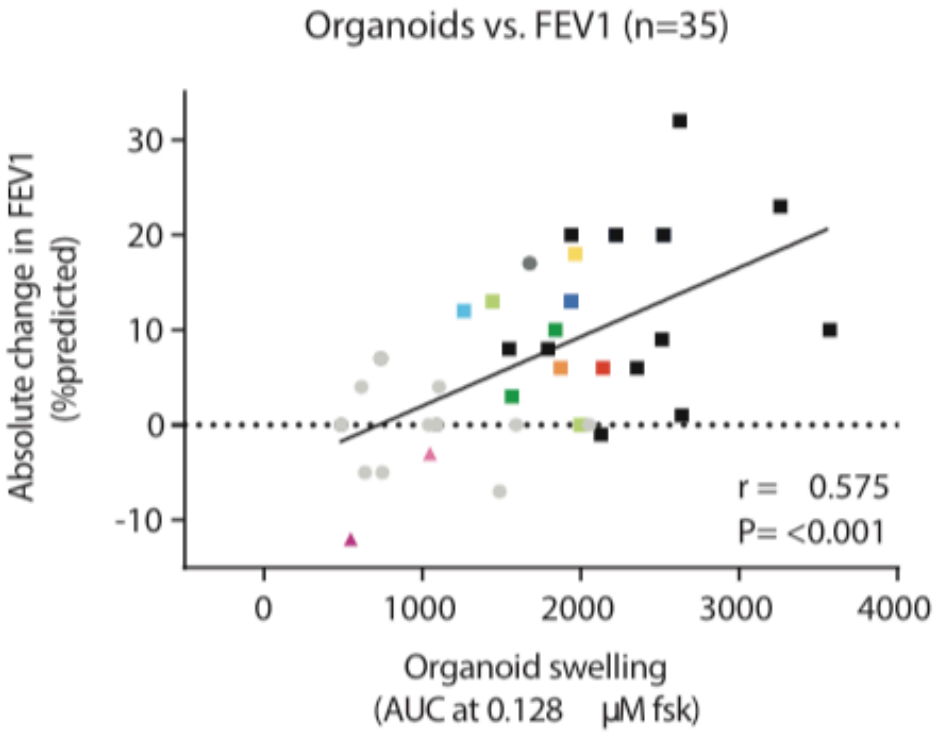


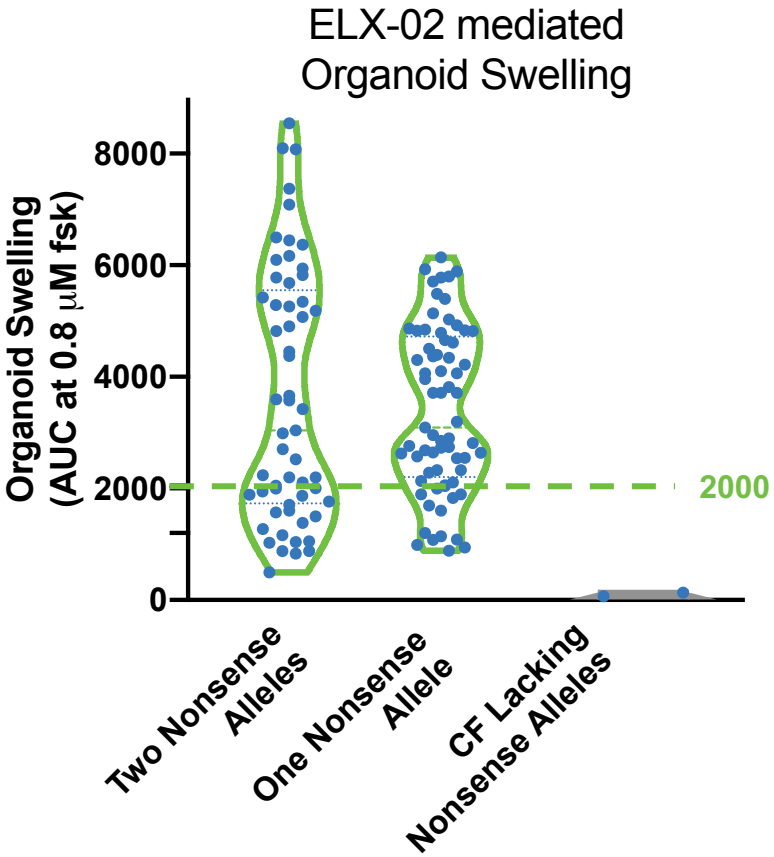
Patient Organoid with ELX-02 treatment: Swelling of Organoids



Swelling quantification of patient organoid with ELX-02 treatment.

ELX-02 Response in Organoids Compares Favorably to Published Results



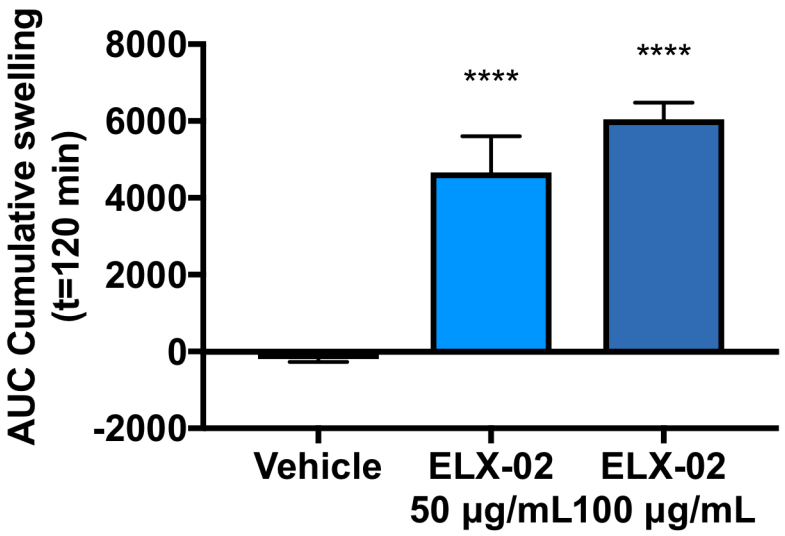


Berkers et al. Cell Reports (2019) Rectal Organoids Enable Personalized Treatment of Cystic Fibrosis.

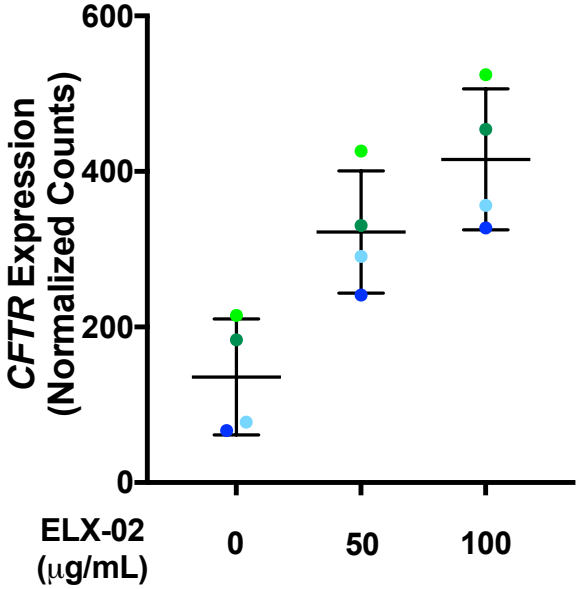
Eloxx data on file.

Homozygous Nonsense Mutation (G542X)

SWELLING (FUNCTION) ASSAY

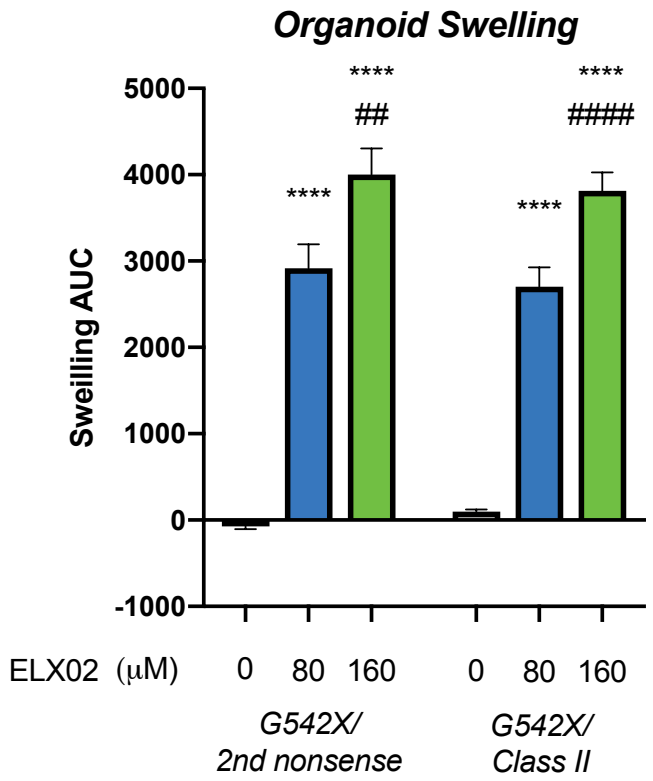


NANOSTRING (MRNA) ASSAY



ELX-02 Mediated Organoid Swelling Is Equivalent in Organoids With One or Two Nonsense Mutations

- Significant increase in organoid swelling is observed in both G542X organoids with a second nonsense mutation and heterozygous organoids.
- Experiments used 0.8 µM Forskolin

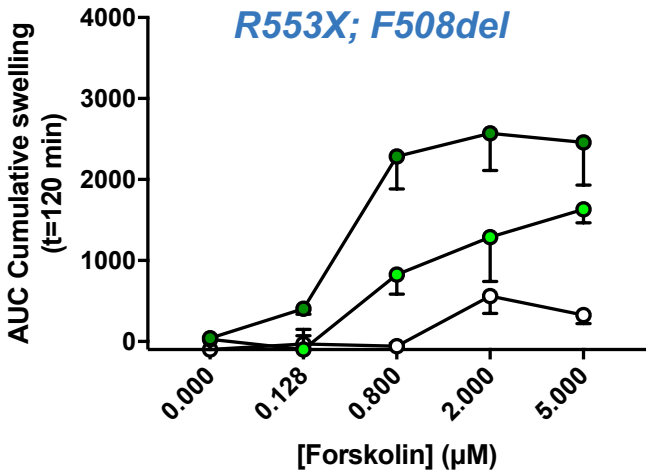


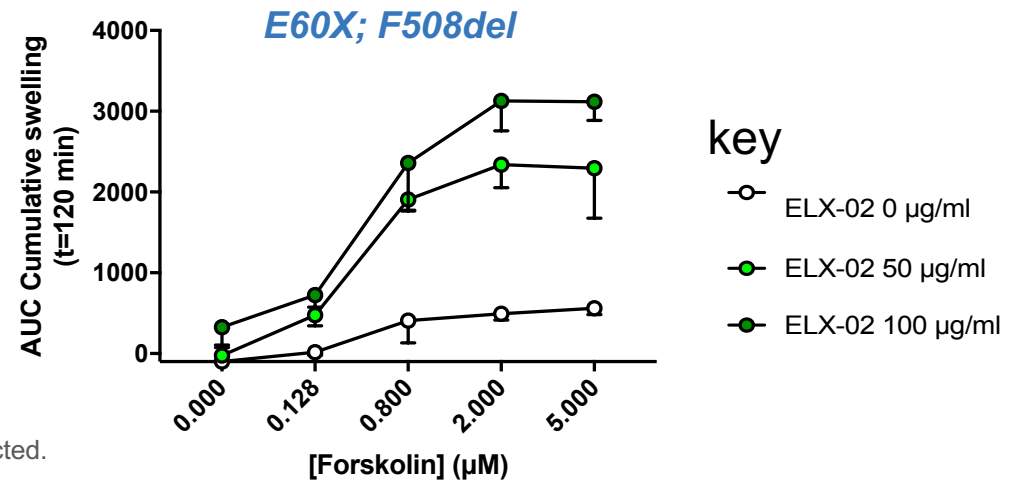
ordinary one-way ANOVA with Tukey's multiple comparison testing was used,, **** p<0.0001 versus vehicle control, ## p<0.01 versus next lower concentration, , #### p<0.0001 versus next lower concentration. Data represent >40 biological replicates per group from 2 or 4 independent patient organoids, respectively.

ELX-02 Organoid Response is Observed Across a Range of CF Genotypes

- *G542X; G542X*
- *G542X; F508del*
- *G542X; R1066C*
- *R1162X; F508del*
- *G542X; W1282X*
- *W1282X; W1282X*
- ***R553X; F508del***
- ***E60X; F508del***

New Genotypes





Eloxx data on file
Data from multiple individuals with the same genotypes also collected.

ELX-02 Completed Phase 1 Studies

ACCP
American College of Clinical Pharmacology
Advancing Clinical Care through Pharmacology

January 16, 2019

Safety, Tolerability, and Pharmacokinetics of Single Ascending Doses of ELX-02, a Potential Treatment for Genetic Disorders Caused by Nonsense Mutations, in Healthy Volunteers

CLINICALTRIALS.GOV
Identifier: NCT03292302

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

COMPLETED

We Believe Demonstrated Preclinical Results De-risk Phase 2 Cystic Fibrosis Program

- Pronounced CFTR read-through demonstrated in plasmid, HBE, FRT, transgenic mice and patient-derived organoids
- ELX-02 permits dose-dependent increases in *CFTR* mRNA
  - Nonsense mediated decay activity is detectable through 3'/5' binding ratios
- ELX-02 dose-dependently increases *CFTR* mRNA stability
  - Response is most pronounced in organoids bearing two nonsense alleles
  - Contributions of the NMD pathway are currently under evaluation
- ELX-02 increases CFTR function in organoids bearing nonsense alleles representing the most common of the cystic fibrosis nonsense genotype population

Phase 2 Cystic Fibrosis Top Line Data in 2019

- **Phase 2 Clinical Trial Program**
  - ✓ US IND Open
  - ✓ Protocol Endorsed by Cystic Fibrosis Foundation
  - ✓ Clinical Trial Application (CTA) approved, EU CFS “high priority” for Phase 2 Protocol
  - ✓ Dr. Ahmet Uluer, Director, Adult Cystic Fibrosis Program at Boston Children’s Hospital lead US investigator
  - ✓ Dr. Eitan Kerem, Head of Division of Pediatrics, Children’s Hospital, Haddassah Medical Center
- **Phase 2 Protocol**
  - Enrolling up to 24 patients with the G542X nonsense mutation on one or both alleles in the U.S., Europe and Israel
  - 4 increasing doses of ELX-02 ranging from 0.3 up to 3.0 mg/kg/day
  - Measuring changes in sweat chloride consistent with other Phase 2 trials for approved drugs

North American Cystic Fibrosis Conference (NACFC) October 31 - November 2, 2019

NORTH AMERICAN CYSTIC FIBROSIS CONFERENCE
CYSTIC FIBROSIS FOUNDATION

"Investigational Drug ELX-02 Mediates CFTR Nonsense Mutation Read-through To Increase *CFTR m*RNA CFTR Protein Translation and CFTR Function" **November 1, 2019 2:15 p.m. -3:50 p.m. CDT ORAL Workshop: New & Emerging Therapies to Correct the Basic Defect"**

"Pharmacokinetics, Safety, and Tolerability of Multiple Ascending Doses of ELX-02 in Healthy Volunteers, a Potential Treatment for Cystic Fibrosis Caused by Nonsense Mutations" **October 31, 2019 11:15 a.m. – 1:45 p.m. CDT POSTER Session 1**

"Pharmacokinetics, Safety, and Tolerability of Single Ascending Doses of ELX-02 in Healthy Volunteers, a Potential Treatment for Cystic Fibrosis Caused by Nonsense Mutations" **11:15 a.m. – 1:45 p.m. CDT POSTER Session 1**

Phase 2 Cystinosis Top Line Data in 2019

- **Phase 2 Clinical Trial Program**
  - ✓ Renal Impairment Study Completed
  - ✓ Enrolling Phase 2 Clinical Trial in Canada
  - ✓ Non-dilutive funding from Genome Quebec and Genome Canada
  - ✓ Cystinosis Research Foundation provided funding for preclinical phase
  - ✓ Dr. Paul Goodyer, Professor of Pediatrics at McGill University is the Prinicipal Investigator
- **Phase 2 Protocol**
  - Enrolling 6 patients with nephropathic cystinosis with at least 1 nonsense mutation in the cystinosis gene
  - Three increasing doses of ELX-02 ranging from 0.3 up to 2.0 mg/kg/day
  - Measuring the dose-dependent effect of ELX-02 on cysteine levels in white blood cells

ELX-02 Completed Phase 1 Studies

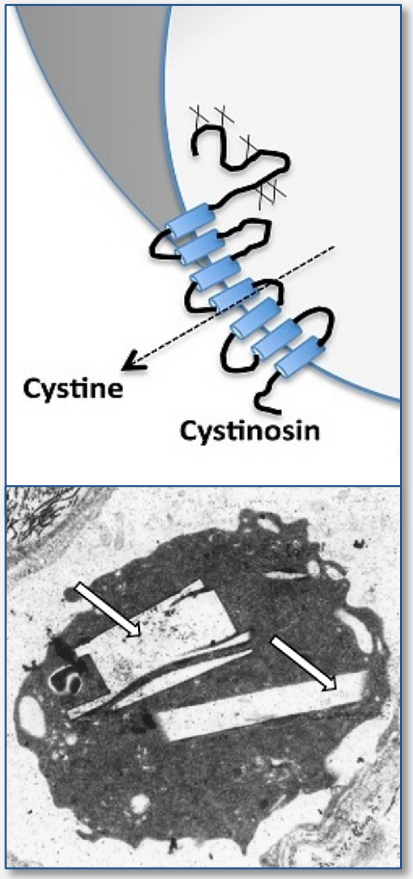
CLINICALTRIALS.GOV
Identifier: NCT0 NCT03776539

A Study to Evaluate the Effects of Renal Impairment on the Pharmacokinetics of ELX-02

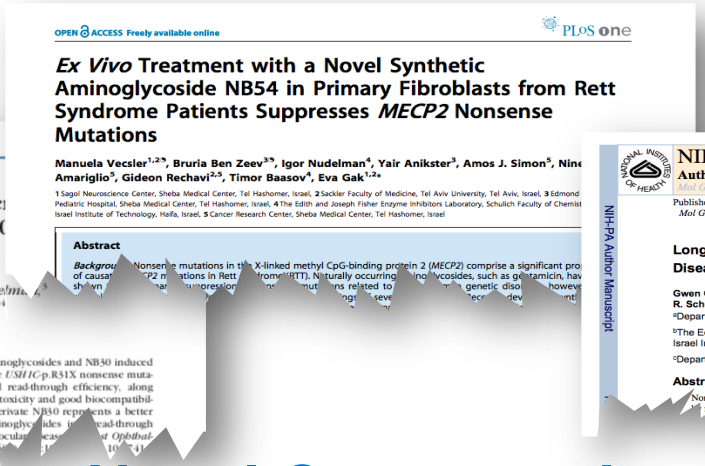
- ✓ Mild, Moderate, Severe Renal Impairment Patient Cohort dosing complete
- ✓ Data analysis ongoing
- ✓ Preliminary results consistent with pK model and generally supportive of continued development including indications where individuals may have renal impairment as a component of ongoing renal disease

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



Novel Compound Library has Demonstrated Activity across Multiple Orphan Diseases


OPEN ACCESS Freely available online

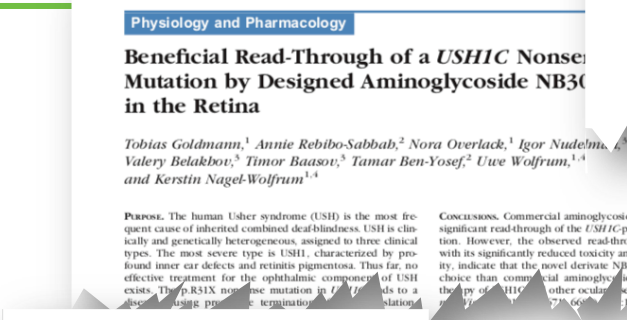
PLoS one

***Ex Vivo* Treatment with a Novel Synthetic Aminoglycoside NB54 in Primary Fibroblasts from Rett Syndrome Patients Suppresses *MECP2* Nonsense Mutations**

Manuela Vecsler[1,2¤], Bruria Ben Zeev[3¤], Igor Nudelman[4], Yair Anikster[3], Amos J. Simon[5], Nine Amariglio[5], Gideon Rechavi[2,5], Timor Baasov[4], Eva Gak[1,2*]

1 Sagol Neuroscience Center, Sheba Medical Center, Tel Hashomer, Israel, 2 Sackler Faculty of Medicine, Tel Aviv University, Tel Aviv, Israel, 3 Edmond Pediatric Hospital, Sheba Medical Center, Tel Hashomer, Israel, 4 The Edith and Joseph Fisher Enzyme Inhibitors Laboratory, Schulich Faculty of Chemist Israel Institute of Technology, Haifa, Israel, 5 Cancer Research Center, Sheba Medical Center, Tel Hashomer, Israel

Abstract


Physiology and Pharmacology

Beneficial Read-Through of a *USH1C* Nonse Mutation by Designed Aminoglycoside NB3(in the Retina

Tobias Goldmann,[1] Annie Rebibo-Sabbah,[2] Nora Overlack,[1] Igor Nudelm Valery Belakhov,[5] Timor Baasov,[5] Tamar Ben-Yosef,[2] Uwe Wolfrum,[1,4] and Kerstin Nagel-Wolfrum[1,4]

Purpose. The human Usher syndrome (USH) is the most frequent cause of inherited combined deaf-blindness. USH is clinically and genetically heterogeneous, assigned to three clinical types. The most severe type is USH1, characterized by profound inner ear defects and retinitis pigmentosa. Thus far, no effective treatment for the ophthalmic component of USH exists. The p.R31X nonsense mutation in *USH1C* leads to a

Conclusions. Commercial aminoglycosides and NB30 induced significant read-through of the *USH1C*-p.R31X nonsense mutation. However, the observed read-through efficiency, along with its significantly reduced toxicity and good biocompatibility, indicate that the novel derivate NB30 represents a better choice than commercial aminoglycosides in read-through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Mol Genet Metab. Author manuscript; available in PMC 2015 March 01.

Published in final edited form as:

Mol Genet Metab. 2014 March ; 111(3): 374–381. doi:10.1016/j.ymgme.2013.12.007.

Long-Term Nonsense Suppression Therapy Moderates MPS I-H Disease Progression

Gwen Gunn[a], Yanying Dai[a], Ming Du[a], Valery Belakhov[b], Jeyakumar Kandasamy[b], Trenton R. Schoeb[c], Timor Baasov[b], David M. Bedwell, and Kim M. Keeling[a]

[a]Department of Microbiology, University of Alabama at Birmingham, Birmingham, AL, USA.

[b]The Edith and Joseph Enzyme Inhibitors Laboratory, Schulich Faculty of Chemistry, Technion-Israel Institute of Technology, Haifa, Israel

[c]Department of Genetics, University of Alabama at Birmingham, Birmingham, AL, USA

Abstract

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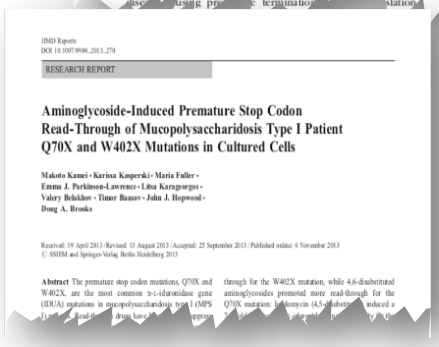
HMD Reports

DOI 10.1007/8904_2013_270

RESEARCH REPORT

Aminoglycoside-Induced Premature Stop Codon Read-Through of Mucopolysaccharidosis Type I Patient Q70X and W402X Mutations in Cultured Cells

Makoto Kanei · Karissa Kasperski · Maria Fuller · Emma J. Parkinson-Lawrence · Litsa Karagiorgos · Valery Belakhov · Timor Baasov · John J. Hopwood · Doug A. Brooks

Received: 19 April 2013 / Revised: 13 August 2013 / Accepted: 25 September 2013 / Published online: 6 November 2013

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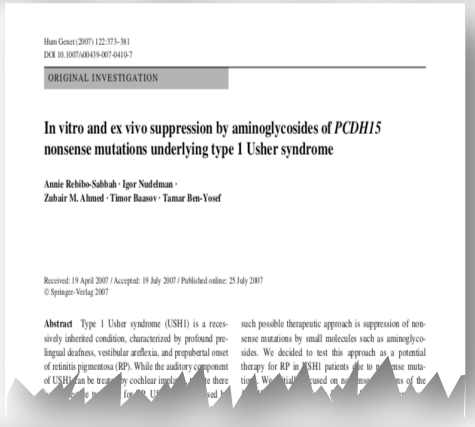
Abstract The premature stop codon mutations, Q70X and W402X, are the most common α-l-iduronidase gene (IDUA) mutations in mucopolysaccharidosis type I (MPS I)

through for the W402X mutation, while 4,6-disubstituted aminoglycosides promoted more read-through for the Q70X mutation. Gentamicin (4,5-disubstituted


Hum Genet (2007) 122:373–381

DOI 10.1007/s00439-007-0410-7

ORIGINAL INVESTIGATION

In vitro and ex vivo suppression by aminoglycosides of *PCDH15* nonsense mutations underlying type 1 Usher syndrome

Annie Rebibo-Sabbah · Igor Nudelman · Zubair M. Ahmed · Timor Baasov · Tamar Ben-Yosef

Received: 19 April 2007 / Accepted: 19 July 2007 / Published online: 25 July 2007

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Abstract Type 1 Usher syndrome (USH1) is a recessively inherited condition, characterized by profound prelingual deafness, vestibular areflexia, and prepubertal onset of retinitis pigmentosa (RP). While the auditory component of USH can be treated by cochlear implantation, there

such possible therapeutic approach is suppression of nonsense mutations by small molecules such as aminoglycosides. We decided to test this approach as a potential therapy for RP in USH1 patients due to nonsense muta-


Aminoglycoside antibiotics restore dystrophin function to skeletal muscles of *mdx* mice

Elisabeth R. Barton-Davis,[1] Laurence Cordier,[1] Daria I. Shoturma,[1] Stuart E. Leland,[2] and H. Lee Sweeney[1]

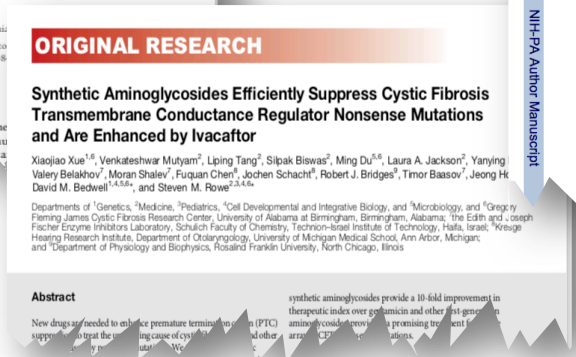
[1]Department of Physiology, and

[2]Institute for Human Gene Therapy, University of Pennsylvania School of Medicine, Philadelphia, Pennsylvani

Address correspondence to: H. Lee Sweney, A700 Richards Building, Department of Physiology, 3700 Hamilto University of Pennsylvania School of Medicine, Philadelphia, Pennsylvania 19104-6085, USA. Phone: (215) 89 Fax: (215) 898-0475; E-mail: Lsweeney@mail.med.upenn.edu.

Received for publication July 14, 1999, and accepted July 16, 1999.

Duchenne muscular dystrophy (DMD) is caused by mutations in the dystrophin gene absence of the dystrophin protein in muscle. A significant number of these mu


ORIGINAL RESEARCH

Synthetic Aminoglycosides Efficiently Suppress Cystic Fibrosis Transmembrane Conductance Regulator Nonsense Mutations and Are Enhanced by Ivacaftor

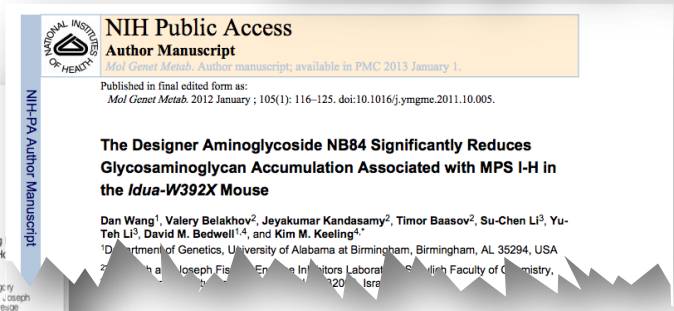
Xiaojiao Xue[1,6], Venkateshwar Mutyam[2], Liping Tang[2], Silpak Biswas[2], Ming Du[5,6], Laura A. Jackson[2], Yanying Valery Belakhov[7], Moran Shalev[7], Fuquan Chen[8], Jochen Schacht[8], Robert J. Bridges[9], Timor Baasov[7], Jeong Ho David M. Bedwell[1,4,5,6*], and Steven M. Rowe[2,3,4,6*]

Departments of [1]Genetics, [2]Medicine, [3]Pediatrics, [4]Cell Developmental and Integrative Biology, and [5]Microbiology, and [6]Gregory Fleming James Cystic Fibrosis Research Center, University of Alabama at Birmingham, Birmingham, Alabama; [7]the Edith and Joseph Fischer Enzyme Inhibitors Laboratory, Schulich Faculty of Chemistry, Technion–Israel Institute of Technology, Haifa, Israel; [8]Kresge Hearing Research Institute, Department of Otolaryngology, University of Michigan Medical School, Ann Arbor, Michigan; and [9]Department of Physiology and Biophysics, Rosalind Franklin University, North Chicago, Illinois

Abstract

New drugs are needed to enhance premature termination codon (PTC) suppression to treat the underlying cause of cystic fibrosis (CF) and other

synthetic aminoglycosides provide a 10-fold improvement in therapeutic index over gentamicin and other first-generation aminoglycosides. Ivacaftor (VX-770) is a promising treatment for CF patients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The Designer Aminoglycoside NB84 Significantly Reduces Glycosaminoglycan Accumulation Associated with MPS I-H in the *Idua-W392X* Mouse

Dan Wang[1], Valery Belakhov[2], Jeyakumar Kandasamy[2], Timor Baasov[2], Su-Chen Li[3], Yu-Teh Li[3], David M. Bedwell[1,4], and Kim M. Keeling[4,*]

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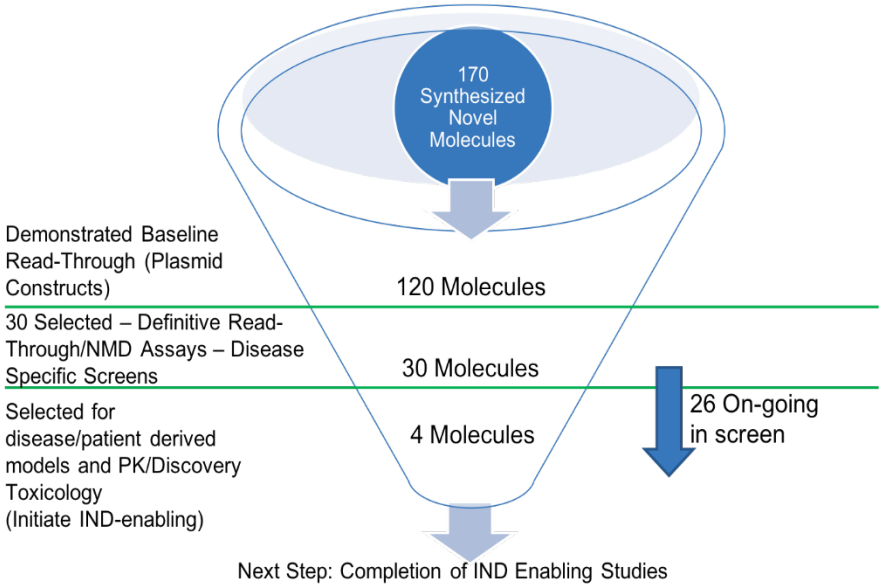
NIH-PA Author Manuscript

Advancing Our Portfolio of Novel ERSGs

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

Rare Kidney Diseases

- > 150 identified Rare Kidney Diseases
- Overall Prevalence: 60-80 cases per 100,000
- Fifth most common cause of ESRD

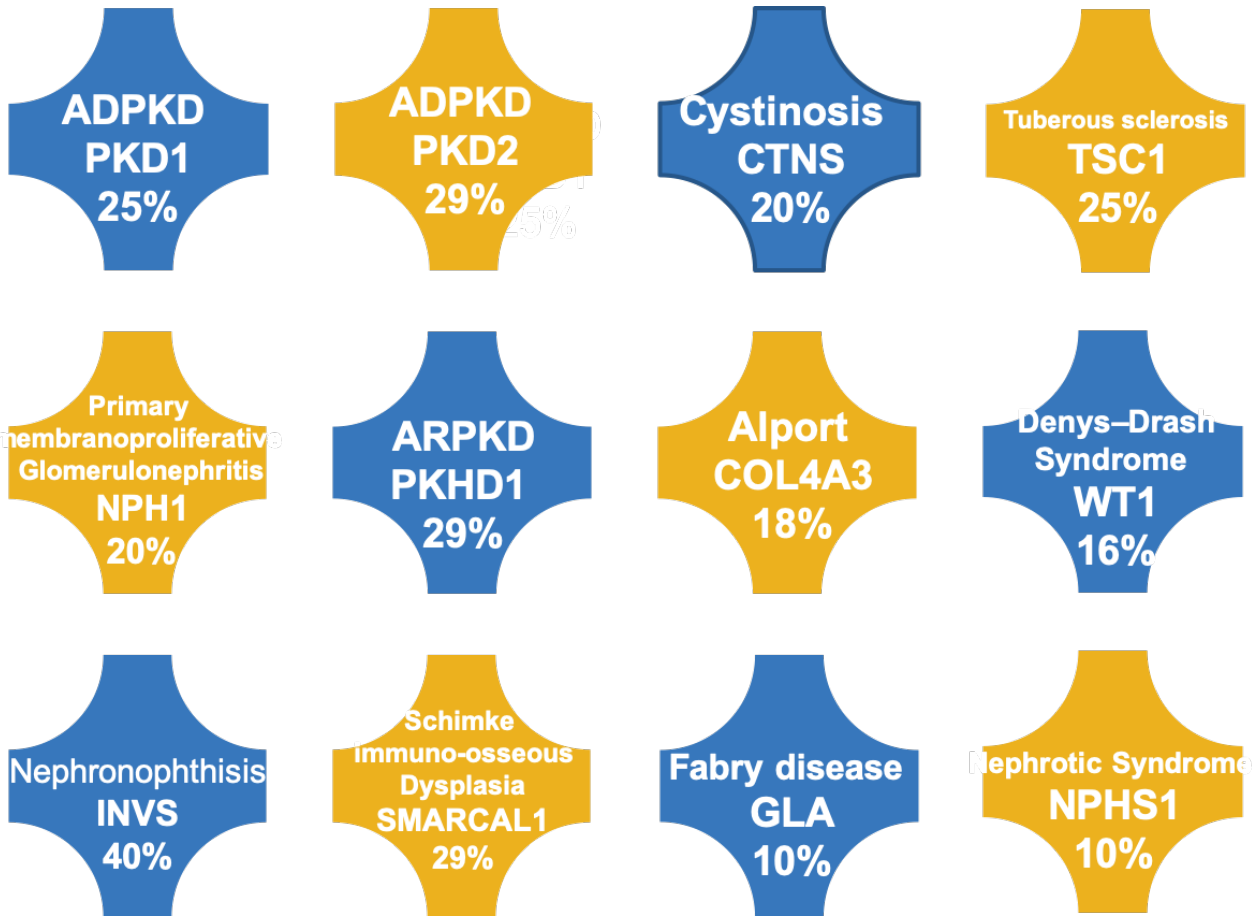
Patients with inherited kidney disorders rarely die (many progress to renal transplantation)
Results in poor health, low quality of life, multisystemic complications. *Devuyst & Gauy-Woodford - KDIGO Conference (2016)*

Nephropathic cystinosis is an autosomal recessive storage disease caused by defective transport of cystine out of lysosomes .The renal tubular damage of cystinosis, begins at 6 to 12 months of age, glomerular damage generally becomes apparent by 2 to 5 years of age and results in end-stage renal disease by 9 to 10 years of age unless cystine-depleting therapy is initiated early in life. Renal transplantation, often by age of 20, has transformed cystinosis from an exclusively pediatric disease to one that affects individuals up to (and potentially beyond) 50 years of age.

Nonsense mutations are an important genetic variation in a wide range of rare kidney disorders including: Nephropathic Cystinosis, Autosomal Dominant Polycystic Kidney Disease, Cystinuria, and others

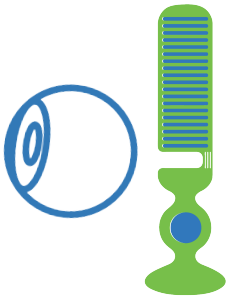
Nephrology NSM mediated disease opportunities

Inherited Disease Target Gene NSM %



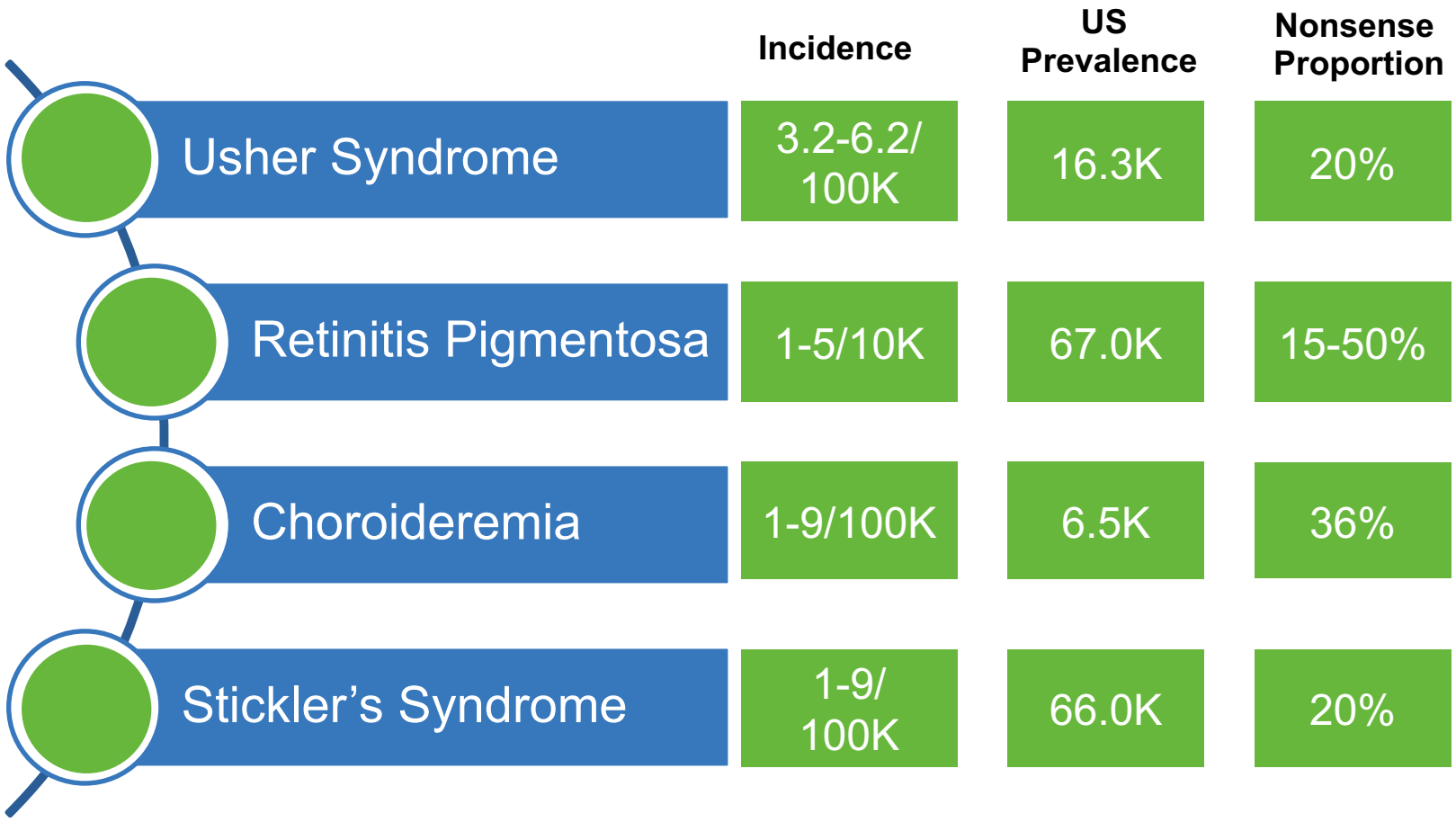
Source: Torra et al, UGA hopping: a sport for nephrologists too? Nephrol Dial Transplant (2010) 25: 2391–2395

Ocular Program Development



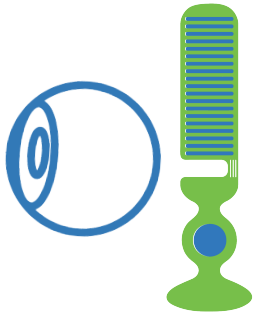
- High unmet medical need and prevalence of nonsense mutations across inherited retinal diseases (IRDs)
- Screened multiple compounds from ERSG library for read-through and tolerability
- IND-enabling studies are progressing
- Emerging Profile Across Models
  - Compounds are appropriate for intravitreal administration
  - Compounds show retinal tolerability at doses 10-fold greater than anticipated efficacy range in sensitive species
    - No adverse ELX compound-related retinal anatomic or functional changes observed to date by histopathology and ERG
  - Dose-dependent read-through of Usher mutations greater than gentamicin reference
  - Encouraging PK demonstrating retina exposure
- Growing team of ophthalmic expertise
- Support from the FFB and Research Community

Substantial Opportunities in Inherited Retinal Diseases



Population values from National Organization for Rare Disorders (NORD) and Orphanet
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

Emerging Profile of Read-through For Usher Syndrome



✓

Tolerability
At doses >10-fold over anticipated treatment level

✓

Read-through
of Usher mutations >gentamicin

✓

Pharmacokinetics
demonstrate substantial retinal exposure

✓

Focus
on developing therapies to prevent blindness

Eloxx Pharmaceutical Highlights

- **Experienced Leadership Team**
- **Phase 2 ELX-02 Clinical Trial in Cystic Fibrosis**
  - U.S. IND Open
  - Cystic Fibrosis Foundation Endorsed Protocol
  - Dr. Ahmet Uluer, Director of Adult Cystic Fibrosis Program, Boston Children's Hospital, Lead U.S. Investigator
  - Dr. Eitan Kerem, Head of Division of Pediatrics, Children's Hospital, Haddassah Medical Center, Global Lead Investigator
- **Phase 2 ELX-02 Clinical Trial in Cystinosis**
  - Dr. Paul Goodyer, Professor of Pediatrics, McGill University, Principal Investigator
  - Non-dilutive funding from Genome Quebec and Genome Canada
- **Topline ELX-02 Phase 2 Data in Cystic Fibrosis and Cystinosis in 2019**
- **Reported Cash & Cash Equivalents at June 30, 2019 of $76.3M funded through top-line data and into 2021**

Eloxx Pharmaceuticals

Thank you.

Canaccord Genuity Growth Conference

August 8, 2019