



Establishing a Translational Read-Through Approach for Inherited Retinal Disorders

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Forward-Looking Statements

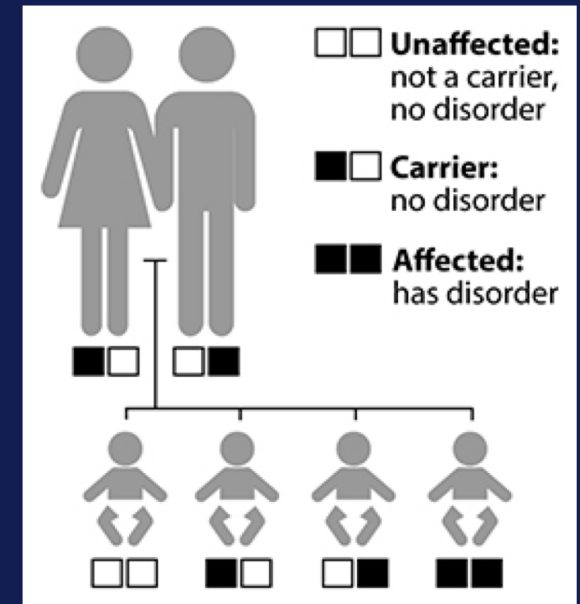
This presentation contains forward-looking statements, which are generally statements that are not historical facts. Forward-looking statements can be identified by the words "expects," "anticipates," "believes," "intends," "estimates," "plans," "will," "outlook" and similar expressions. Forward-looking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

Large Unmet Need in Inherited Retinal Disorders

	Incidence	US Prevalence	Nonsense Proportion
Usher Syndrome	3.2-6.2/ 100K	16.3K	20%
Retinitis Pigmentosa	1-5/10K	67K	15-50%
Choroideremia	1-9/100K	6.5K	36%
Stickler Syndrome	1-9/100K	66K	20%

What Causes Usher Syndrome ?

- Usher syndrome is inherited
- It passes from parents to a child through genes
- Sometimes genes are altered, or mutated



What are Genetic Mutations ?

- Genetic mutations are errors in our DNA code
- They cause cells in our body to behave abnormally

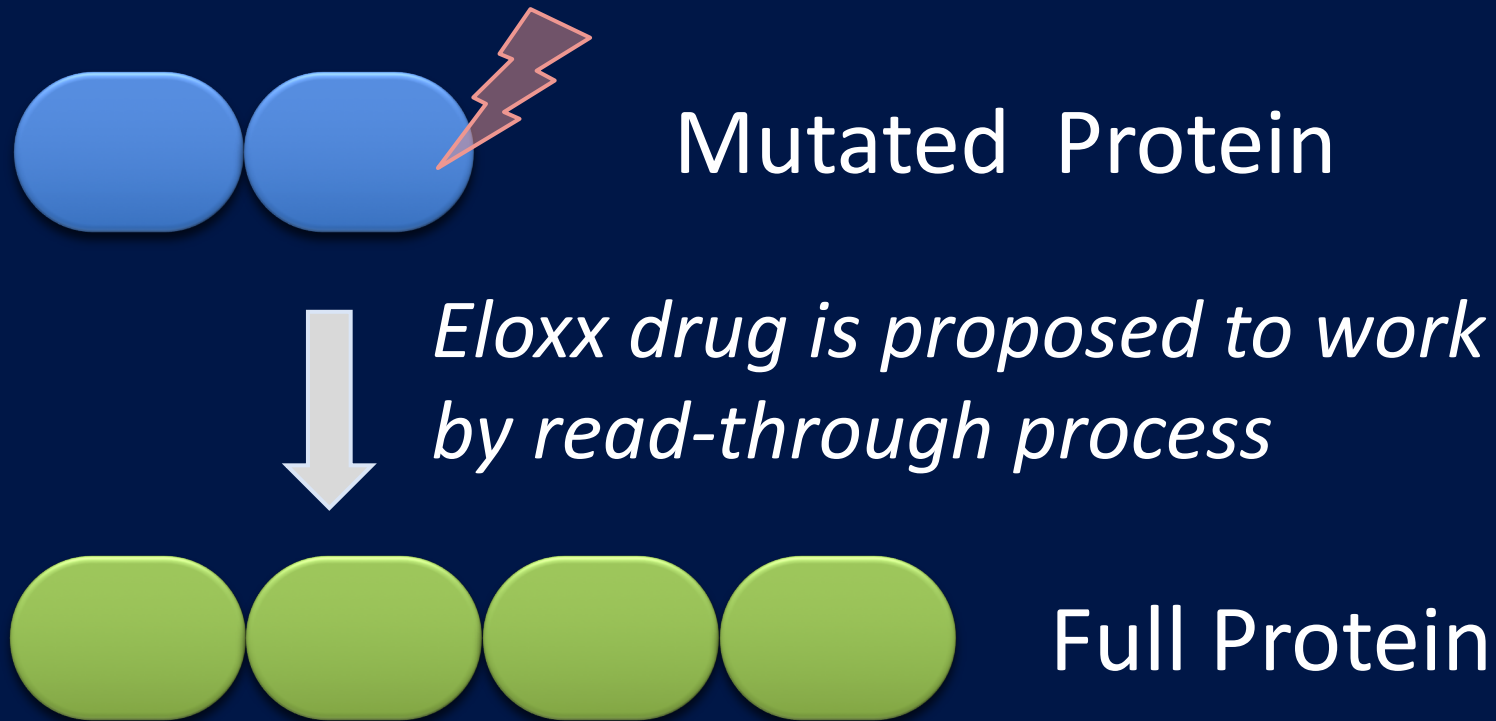


Mutations and Proteins

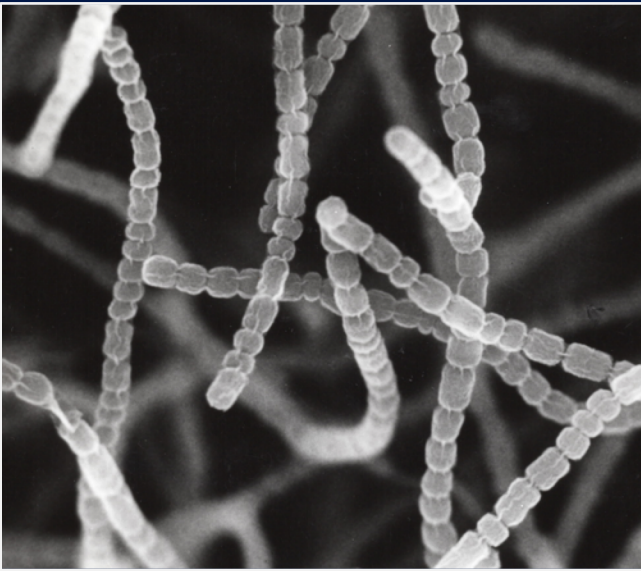
- Some mutations cause proteins to be shorter than usual
- These shorter proteins do not work properly
- Many patients have Usher Syndrome due to shorter proteins



Focus on Restoring Protein Function



Glycoside-Based Approach

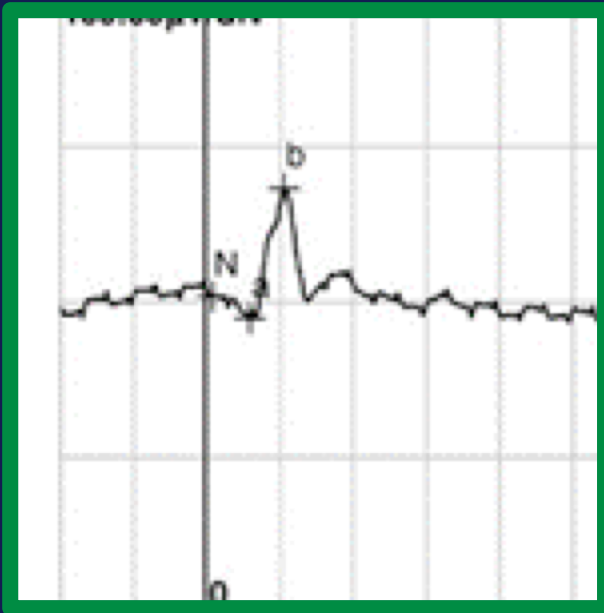


- Gentamicin is an antibiotic of the glycoside family
- Glycosides promote read-through some but have side effects
- Eloxx is developing specific glycosides, known as eukaryotic ribosomal selective glycosides (ERSGs) that are better tolerated and have greater ability to restore protein function, as a potential treatment for Usher Syndrome

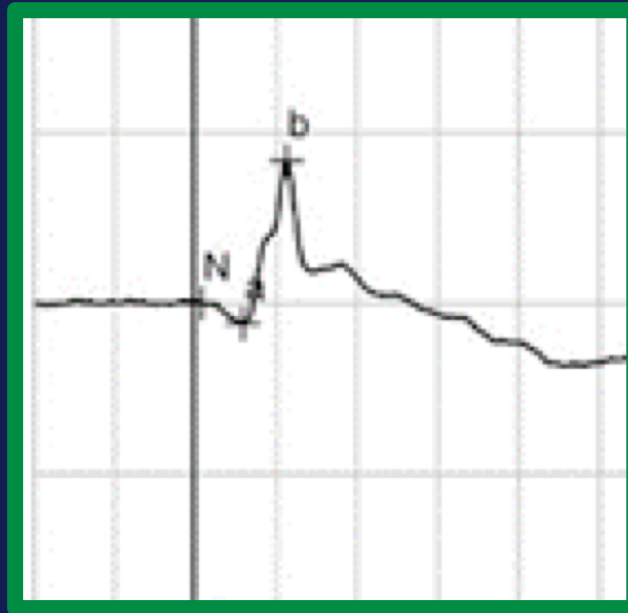
Safety Study



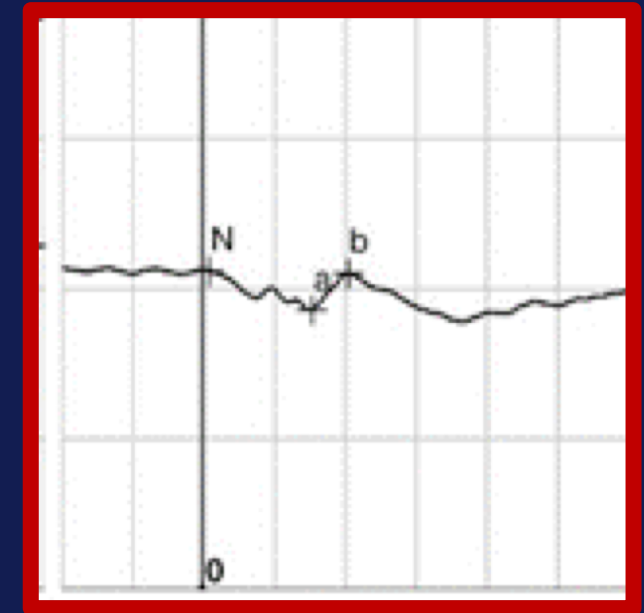
Normal



Eloxx Drug



Gentamicin



In this study, compared to gentamicin, Eloxx drug did not negatively affect photoreceptors – the light sensing cells in the retina

Increased Full-Size Protein Production in Cells With an Usher Syndrome Mutation

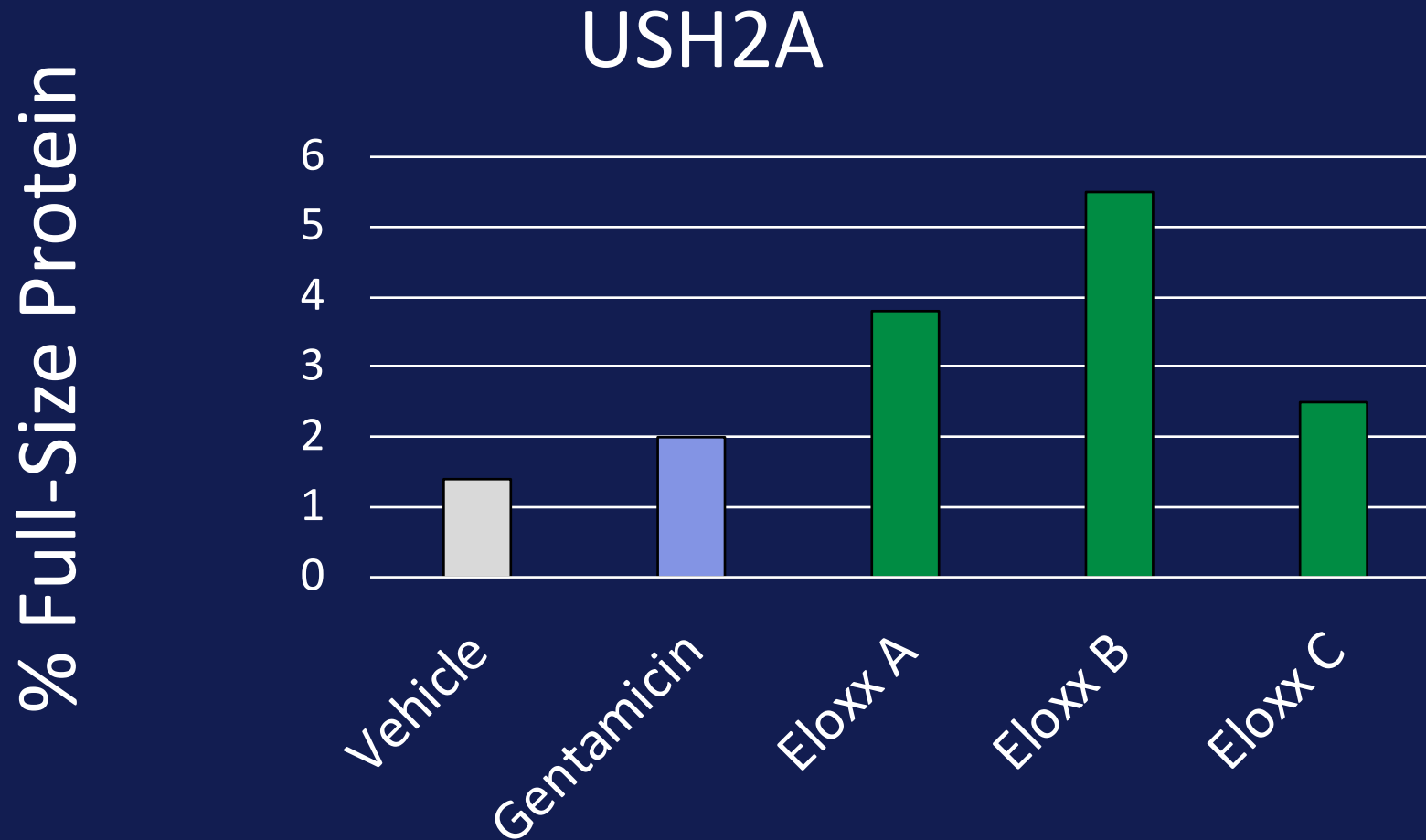
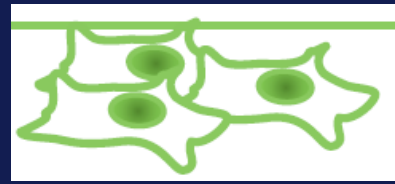
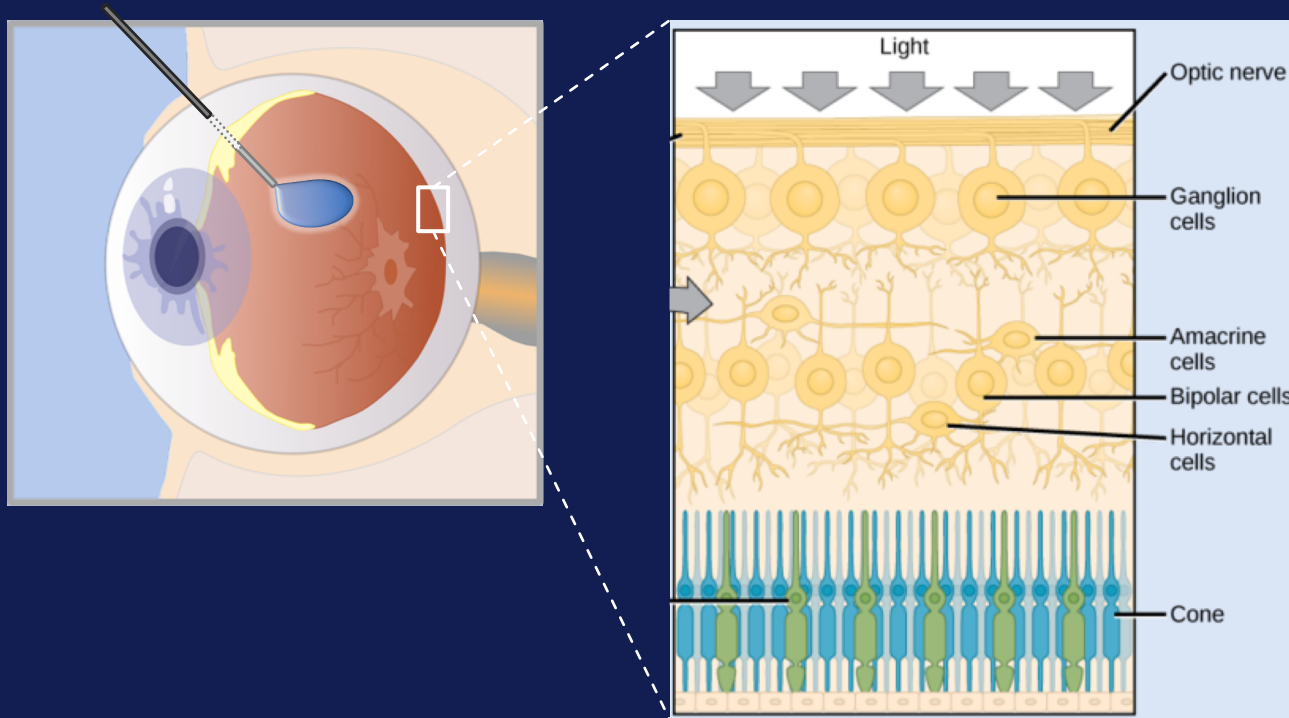


Figure: Effects of gentamicin (4 mM) and Eloxx drugs (1.5 mM) on read-through activity in cells with the Ush2A R626X mutation assessed using a plasmid-based dual-luciferase assay

Delivery of Drug to the Retina



Drug Levels in Eye Tissues

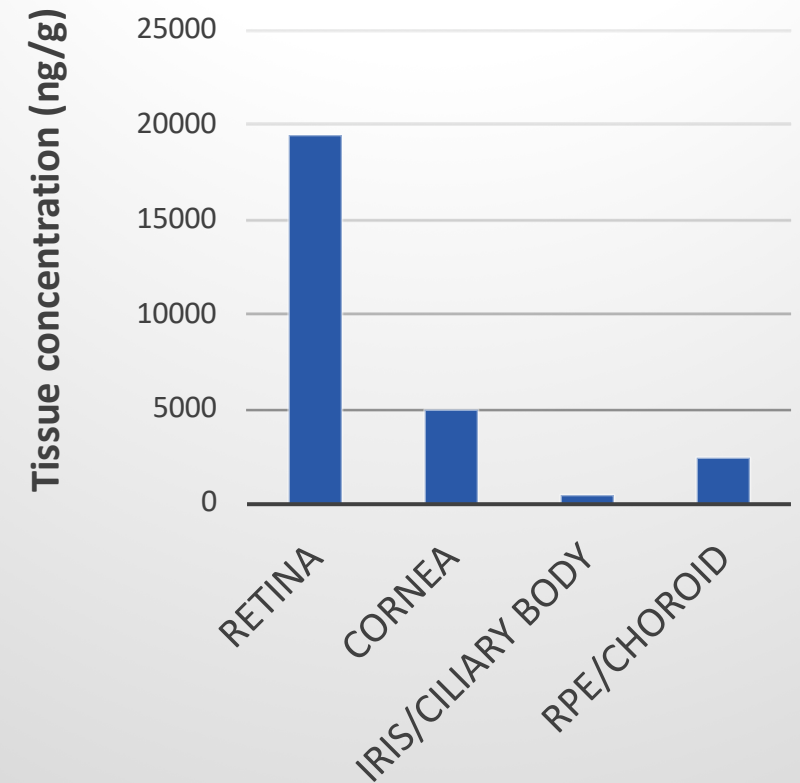
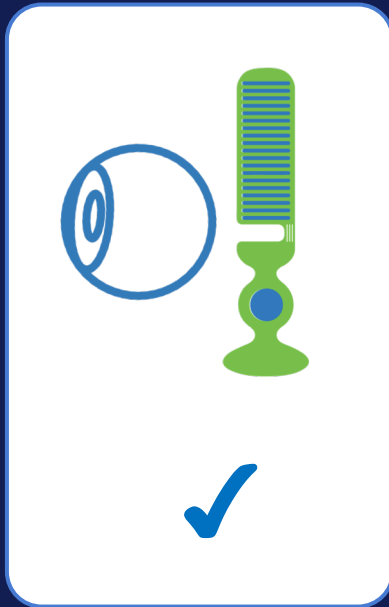


Figure: Ocular tissue concentrations of Eloxx drug three hours after intravitreal injection in rabbits

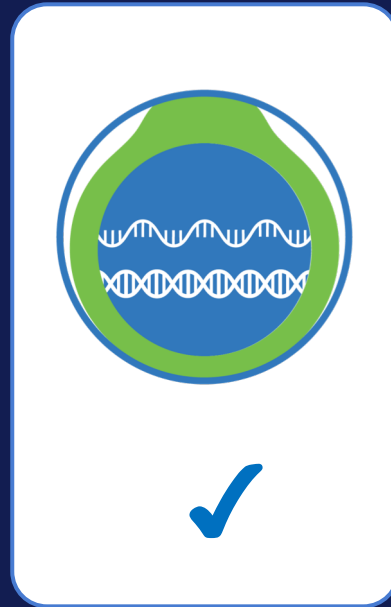
Emerging Profile of Read-through For Usher Syndrome



SAFETY:

Tolerability

At doses >10-fold
over anticipated
treatment level



EFFECT:

Read-through

of Usher
mutations
>gentamicin



DISEASE

LOCATION:

Pharmacokinetics
demonstrate
substantial retinal
exposure



GOAL:

Focus

on developing
therapies to
prevent blindness



THANK YOU