Stoke Therapeutics

NASDAQ: STOK

December 2022

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This presentation contains "forward-looking" statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995, including, but not limited to: the ability of our TANGO platform to design medicines to increase protein production and the expected benefits thereof; expectations regarding our aspirations to execute in the clinic with STK-001, advance to the clinic with STK-002, and expand our pipeline through internal discovery and collaboration; the ability of STK-001 to treat the underlying causes of Dravet syndrome and reduce seizures or show improvements in non-seizure comorbidities at the indicated dosing levels or at all; the ability of STK-002 to treat the underlying causes of Autosomal Dominant Optic Atrophy (ADOA); the preclinical data and study results regarding OPA1; our future operating results, financial position and liquidity; our expectations about timing and execution of anticipated milestones, responses to regulatory authorities, expected nomination of future product candidates and timing thereof; and our expectations, plans, aspirations and goals, including those related to the goals of our collaboration with Acadia. These forward-looking statements may be accompanied by such words as "aim," "anticipate," "believe," "could," "estimate," "expect," "forecast," "goal," "intend," "may," "might," "plan," "potential," "possible," "will," "would," and other words and terms of similar meaning. These forward-looking statements involve risks and uncertainties, as well as assumptions, which, if they do not fully materialize or prove incorrect, could cause our results to differ materially from those expressed or implied by such statements, including: our ability to develop, obtain regulatory approval for and commercialize STK-001, STK-002, and future product candidates, including any future product candidates nominated for SYNGAP1 or MECP2; the timing and results of preclinical studies and clinical trials; the risk that positive results in a clinical trial may not be replicated in subsequent trials or success in early stage clinical trials may not be predictive of results in later stage clinical trials, and that interim data readouts of ongoing trials may show results that change when such trials are completed; risks associated with clinical trials, including our ability to adequately manage clinical activities, unexpected concerns that may arise from additional data or analysis obtained during clinical trials, regulatory authorities may require additional information or further studies, or may fail to approve or may delay approval of our drug candidates; the occurrence of adverse safety events; failure to protect and enforce our intellectual property and other proprietary rights; failure to successfully execute or realize the anticipated benefits of our strategic and growth initiatives, including our collaboration with Acadia; risks relating to technology failures or breaches; our dependence on collaborators, including Acadia, and other third parties for the development, regulatory approval, and commercialization of products and other aspects of our business, which are outside of our full control; the direct and indirect impacts of the ongoing COVID-19 pandemic and its variants on our business, financial condition and operations, including on our expenses, supply chain, strategic partners, research and development costs, clinical trials and employees; risks associated with current and potential future healthcare reforms; risks relating to attracting and retaining key personnel; failure to comply with legal and regulatory requirements; risks relating to access to capital and credit markets; environmental risks; risks relating to the use of social media for our business; and the other risks and uncertainties that are described in the Risk Factors section of our most recent Annual Report on Form 10-K, our Quarterly Reports on Form 10-Q and in other reports we have filed with the U.S. Securities and Exchange Commission. These statements are based on our current beliefs and expectations and speak only as of the date of this presentation. We do not undertake any obligation to publicly update any forward-looking statements.

By attending or receiving this presentation you acknowledge that you are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date such statements are made; you will be solely responsible for your own assessment of the market and our market position; and that you will conduct your own analysis and be solely responsible for forming your own view of the potential future performance of Stoke.

Boldly Restoring Genetic Health

Addressing the underlying cause of severe diseases by upregulating protein expression with RNA-based medicines

Executing in the clinic with STK-001, the first potential disease-modifying approach for the treatment of Dravet syndrome

Advancing to the clinic with STK-002, the first potential disease-modifying approach for the treatment of Autosomal Dominant Optic Atrophy (ADOA)

Expanding our pipeline through internal discovery and collaboration

A Differentiated Platform for the Discovery and Development of Novel RNA-Based Medicines

Proprietary RNA therapeutics platform (TANGO)

Targets pre-mRNA splicing to restore target protein to near-normal levels

Disease-modifying approach

We aim to address the underlying cause of severe diseases

Clinical stage with emerging pipeline

Phase 1/2a studies ongoing with STK-001 for Dravet syndrome (DS). Preclinical development ongoing for STK-002 for autosomal dominant optic atrophy (ADOA)

Broad therapeutic potential

~1,200 monogenic disease genes and ~6,500 additional genes with TANGO target signatures

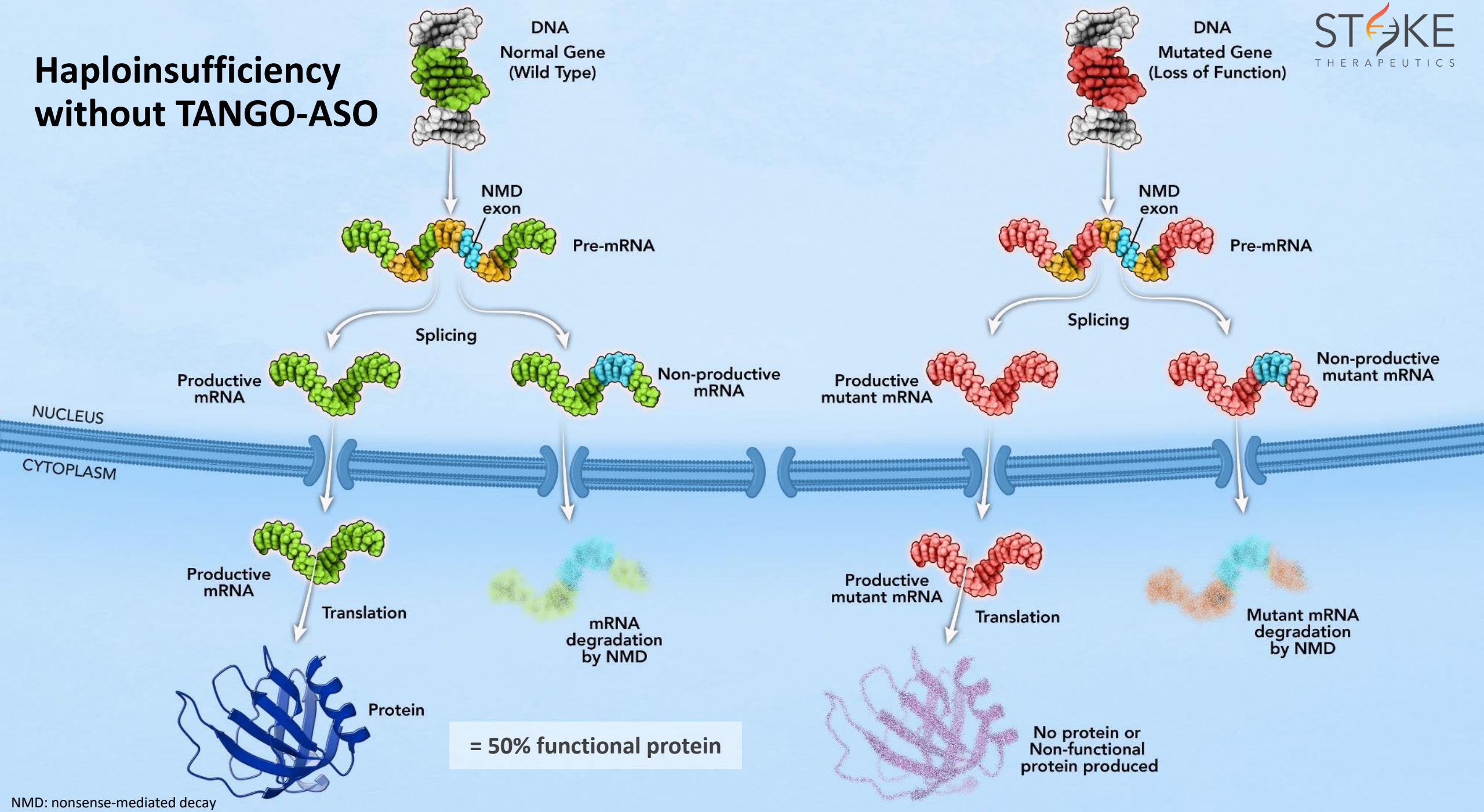
Source: Lim, K.H., Han, Z., Jeon, H.Y. et al. Antisense oligonucleotide modulation of non-productive alternative splicing upregulates gene expression. Nat Commun 11, 3501 (2020).

Targeted Augmentation of Nuclear Gene Output

Our compounds aim to restore protein levels by increasing protein production from the functional copy of a gene and:

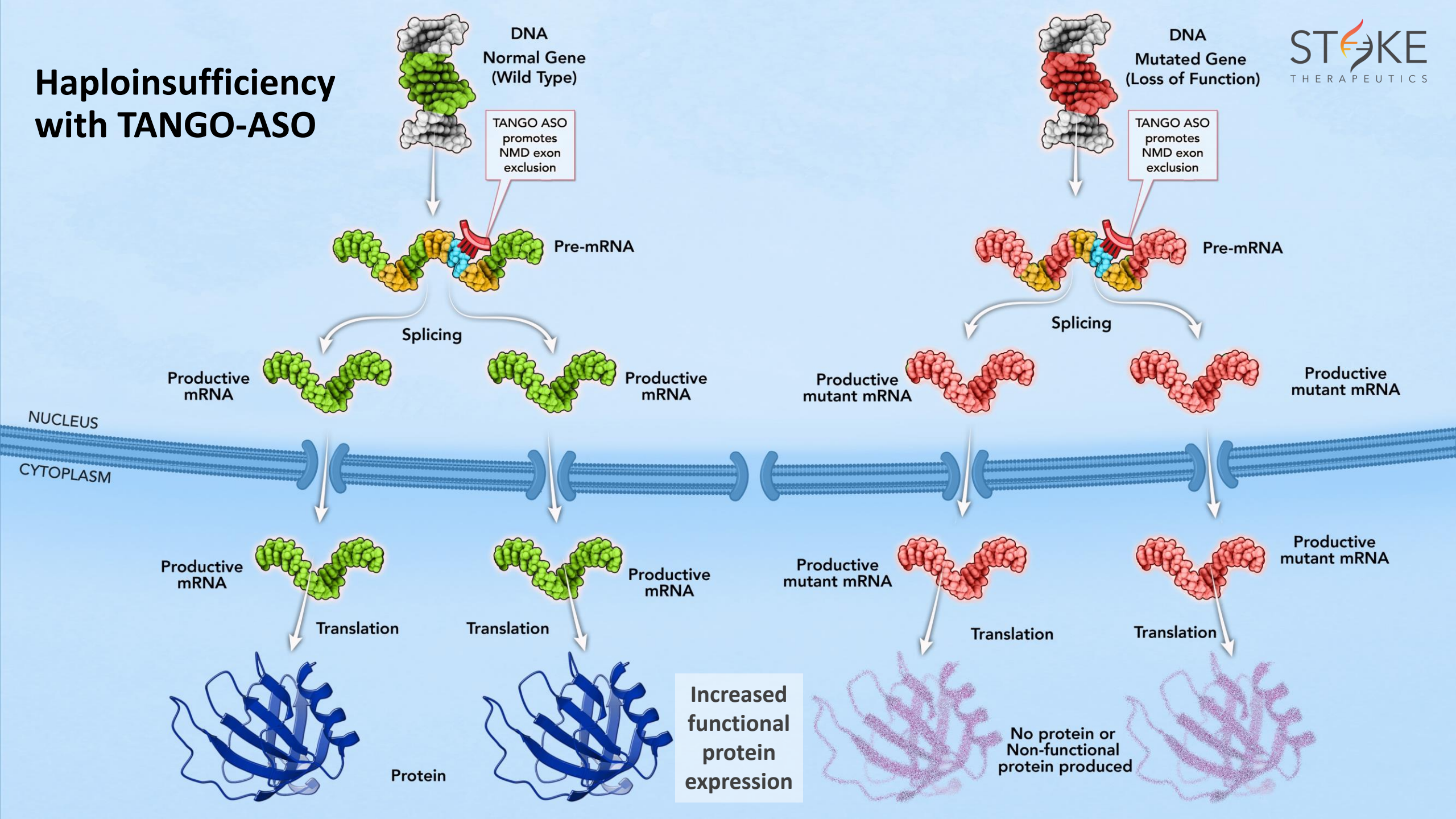
- Selectively boost expression only in tissues where the protein is normally expressed
- Offer one drug for diseases caused by many different loss-of-function mutations
- Apply to genes of diverse size: can be used to address small or large gene targets

Haploinsufficiency without TANGO-ASO



NMD: nonsense-mediated decay

Haploinsufficiency with TANGO-ASO



Dravet Syndrome: A Severe, Progressive Genetic Epilepsy

85%
of cases caused by a **HAPLOINSUFFICIENCY** of the *SCN1A* gene

RESULTING in

50%
$Na_V1.1$ protein expression

1 out of 16,000
babies are born with Dravet syndrome

Up to 20%
of children and adolescents with Dravet syndrome die before adulthood, due to SUDEP[1], prolonged seizures, seizure-related accidents or infections

Seizures are not adequately controlled in **90%** of people with Dravet syndrome

~35,000
people affected in the U.S., Canada, Japan, Germany, France and the UK

Dravet syndrome is not concentrated in a particular geographic area or ethnic group

[1] Sudden Unexpected Death in Epilepsy

Sources: *2018 Health Advances Report*; Djémié et al., *Molecular Genetics & Genomic Medicine,* 2016; Lagae et al., *Developmental Medicine & Child Neurology,* 2017; Nabbout et al., *Orphanet Journal of Rare Diseases,* 2013

No Approved Disease-Modifying Therapies for Dravet Syndrome

Non-Seizure Comorbidities of Dravet Syndrome Are Not Addressed by Current Therapies

- Intellectual disability
- Developmental delays
- Movement and balance issues
- Language and speech disturbances
- Growth defects
- Sleep abnormalities
- Disruptions of the autonomic nervous system
- Mood disorders

Dravet syndrome is classified as a developmental and epileptic encephalopathy due to the developmental delays and cognitive impairment associated with the disease

Non-Seizure Comorbidities of DS are Progressive and Measurable

Gap in overall intellectual development and adaptive function between patients and neurotypical children appears to widen with age

BUTTERFLY

An observational study of Dravet Syndrome patients

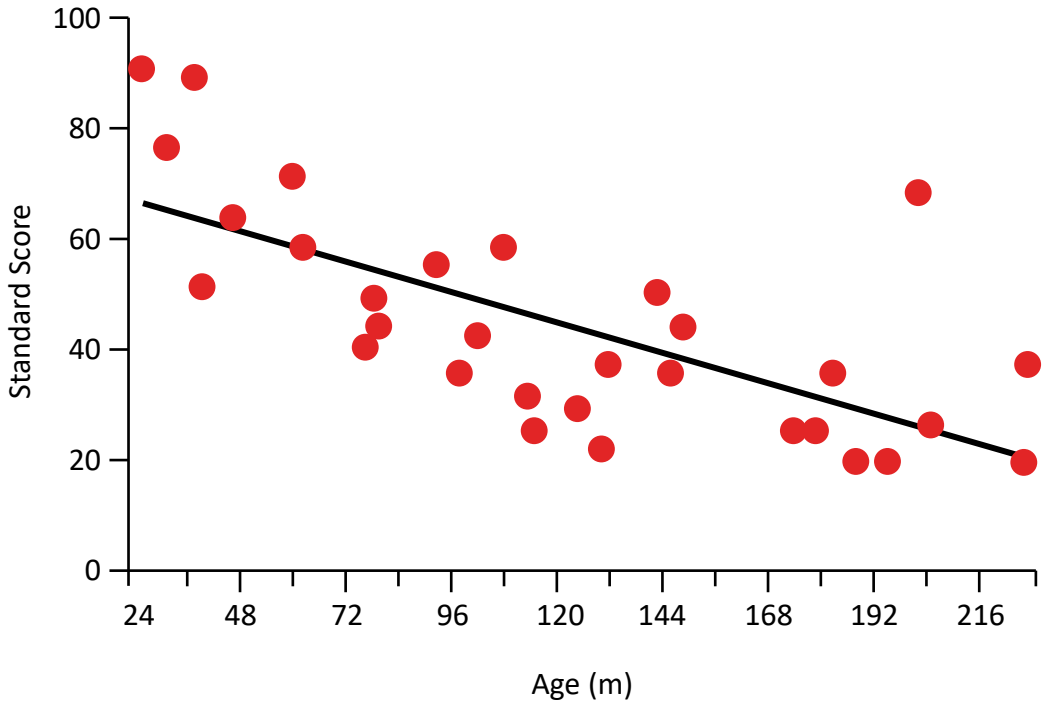
Initial findings showed:

- Validation of standard cognitive measures for use in DS patients
- Substantially decreased neurocognitive abilities despite the use of multiple anti-seizure medications
- A gap in adaptive functioning was observed in VABS* testing

(n=36, 2-18 year-olds). Study ongoing.

Results from the VABS Assessment

Adaptive Behavior Composite (ABC)*

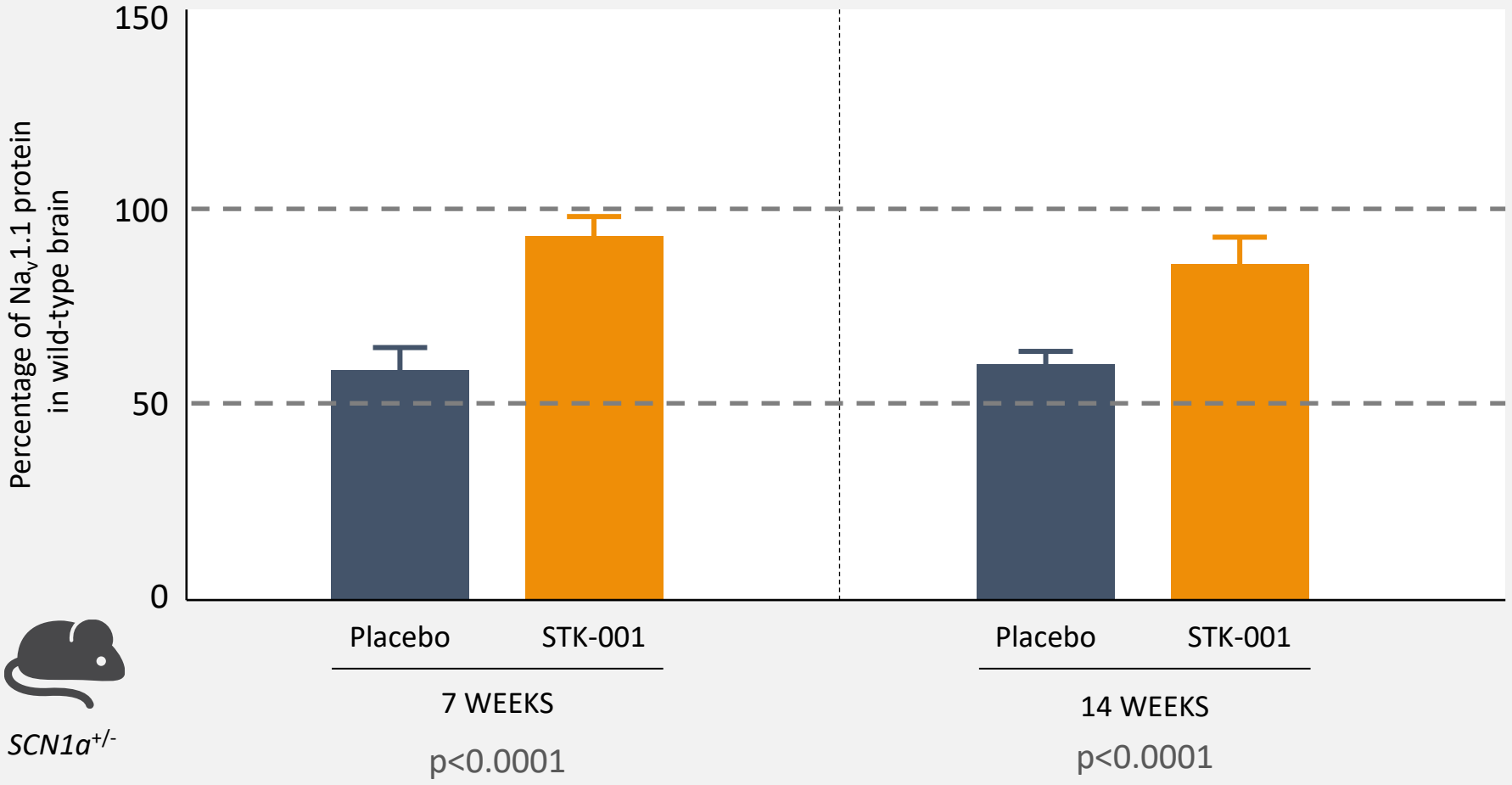


* VABS = Vineland Adaptive Behavior Scales

* ABC score based on Communication, Daily Living, and Socialization domains and expressed relative to normative mean of 100

Source: BUTTERFLY: An Observational Study to Investigate Cognition and Other Non-seizure Comorbidities in Children and Adolescents with Dravet Syndrome (DS) (AES 2021).

STK-001 Restores $Na_V1.1$ to Near Normal Levels for >3 Months in Dravet Syndrome (DS) Mice after a Single Dose

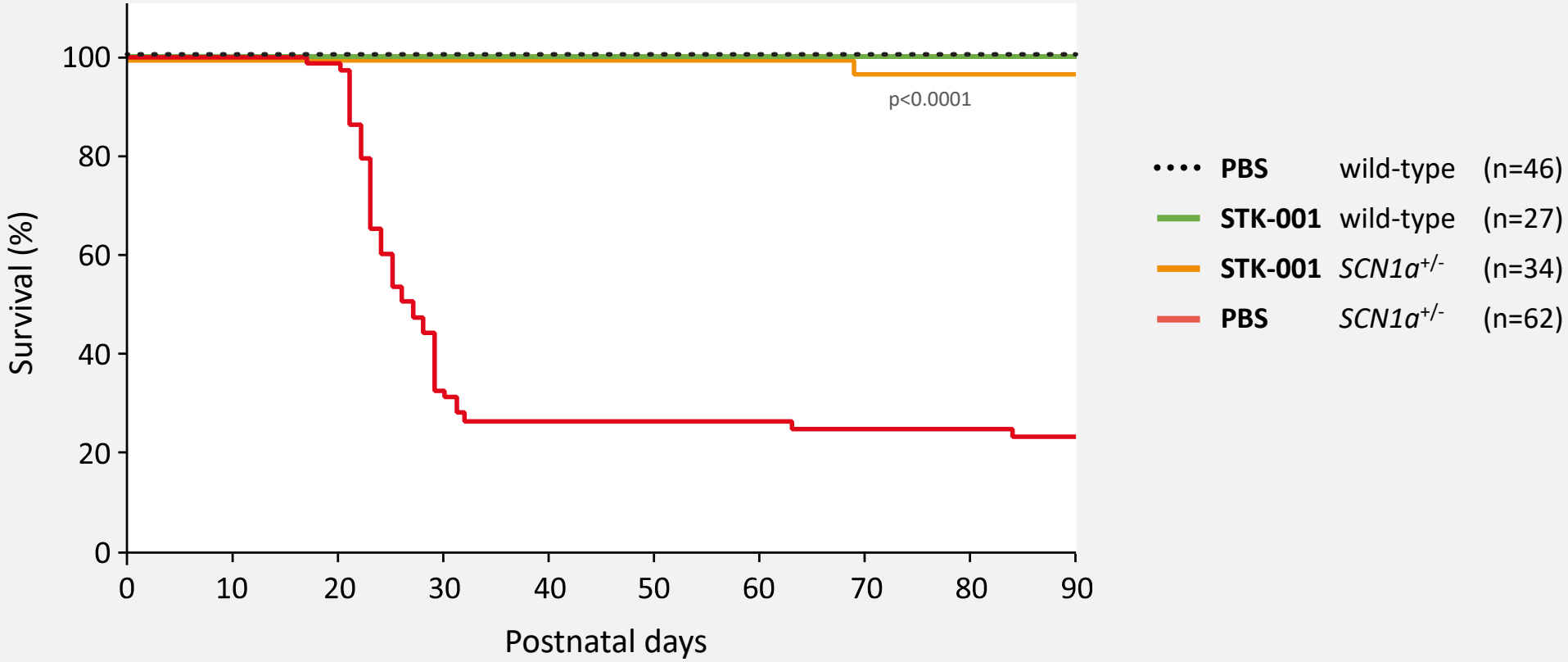


Science Translational Medicine
AAAS

Sources: Z. Han, C. Chen, A. Christiansen, S. Ji, Q. Lin, C. Anumonwo, C. Liu, S. C. Leiser, I. Aznarez, G. Liau, L. L. Isom, Antisense oligonucleotides increase Scn1a expression and reduce seizures and SUDEP incidence in a mouse model of Dravet syndrome. *Sci. Transl. Med.* 12, eaaz6100 (2020).

STK-001 Significantly Reduces Premature Mortality in DS Mice After a Single Dose

Significant improvements in survival after STK-001 administration at postnatal day 2



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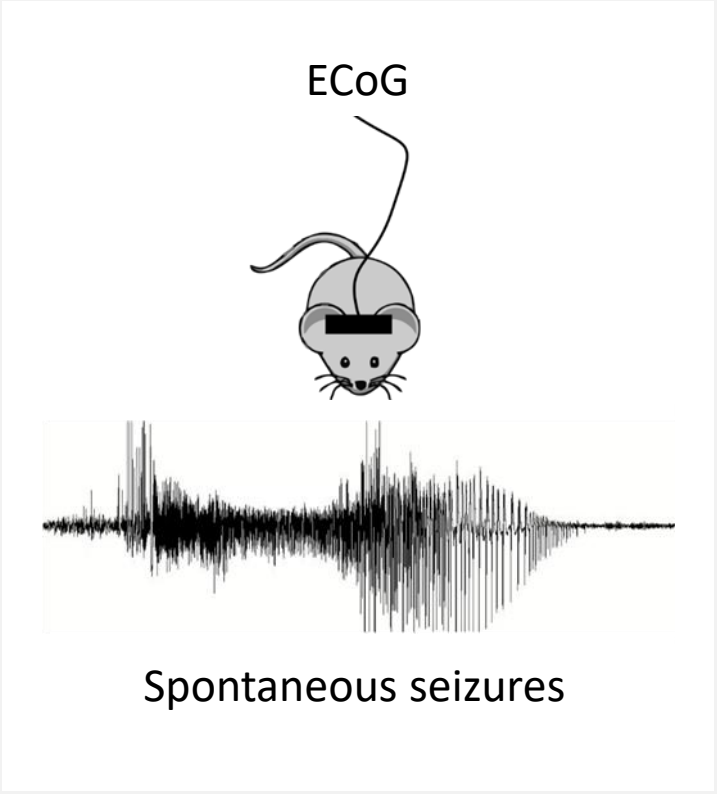
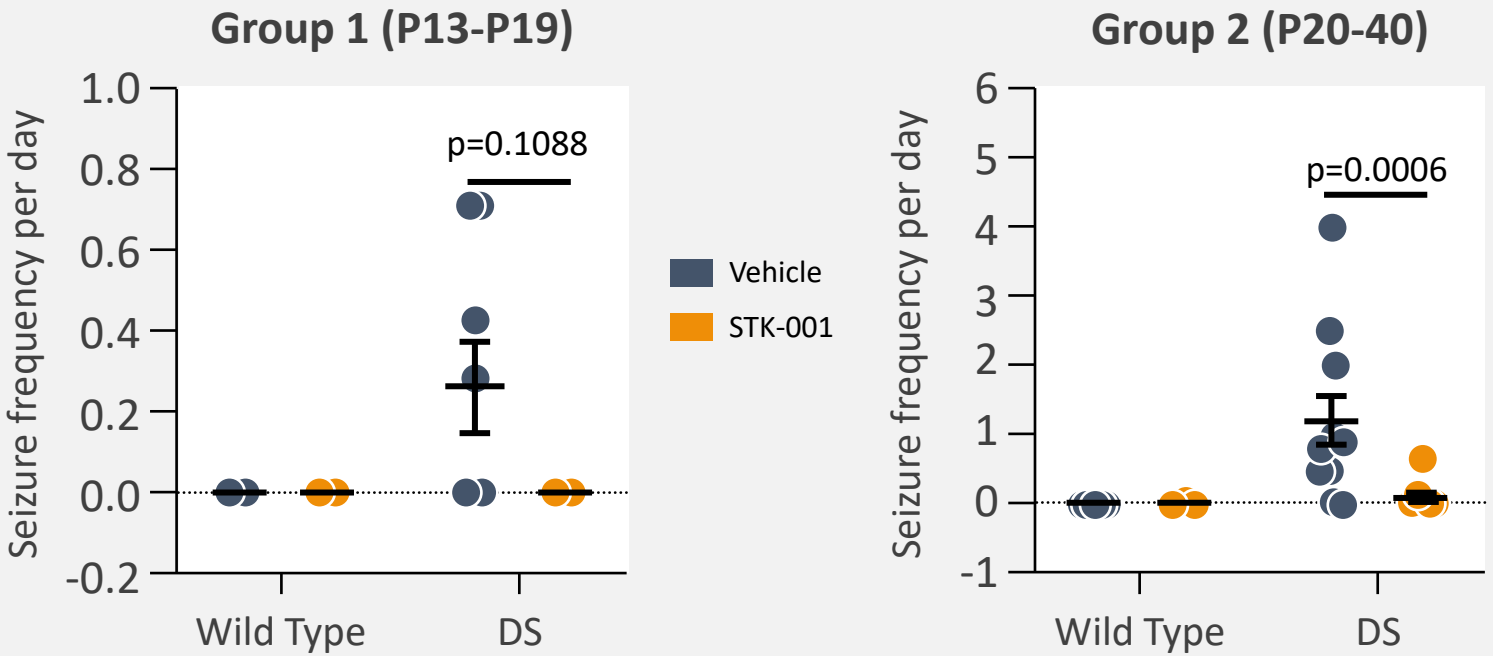
Sources: Z. Han, C. Chen, A. Christiansen, S. Ji, Q. Lin, C. Anumonwo, C. Liu, S. C. Leiser, I. Aznarez, G. Liau, L. L. Isom, Antisense oligonucleotides increase Scn1a expression and reduce seizures and SUDEP incidence in a mouse model of Dravet syndrome. *Sci. Transl. Med.* 12, eaaz6100 (2020).

STK-001 Administration Reduces Seizure Frequency in DS Mice



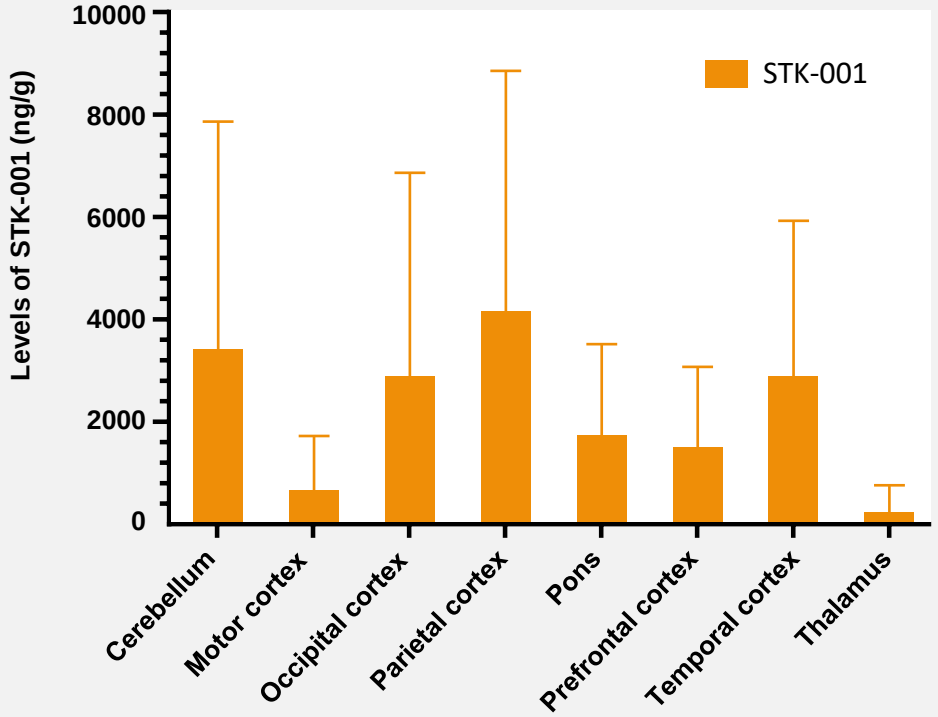
A single dose of STK-001 completely stopped seizure events early (P13-19) and substantially reduced seizure frequency late (P20-40)



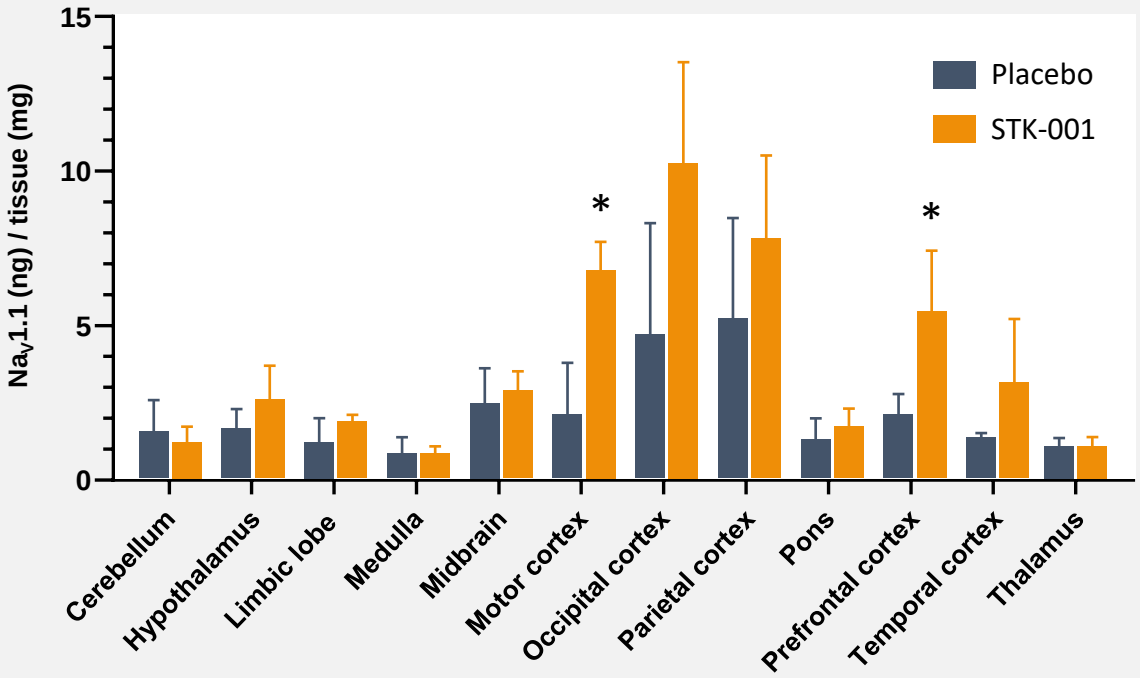
Sources: Targeted Augmentation of Nuclear Gene Output (TANGO) of SCN1A reduces seizures and rescues parvalbumin positive interneuron firing frequency in a mouse model of Dravet syndrome (AES 2020). Wengert ER, Wagley PK, Strohm SM, Reza N, Wenker IC, Gaykema RP, Christiansen A, Liau G, Patel MK. Targeted Augmentation of Nuclear Gene Output (TANGO) of Scn1a rescues parvalbumin interneuron excitability and reduces seizures in a mouse model of Dravet Syndrome. Brain Res. 2022;1775:147743.

STK-001 Achieves Broad Distribution and Increases $Na_v1.1$ Protein Expression in NHPs

Study 1: Exposure of STK-001 observed in all brain regions



Study 2: $Na_v1.1$ protein levels increased up to 3-fold



* = p<0.05

NHP = Non-human primate
Source (left graph): Stoke data
Source (right graph) TANGO oligonucleotides for the treatment of Dravet Syndrome: Safety, biodistribution and pharmacology in the non-human primate (AES 2019)

Preclinical Findings Support Clinical Development of STK-001

- Single dose restores $Na_V1.1$ to near-normal levels for >3 months in DS mice
- Significantly reduces mortality and seizure frequency in DS mice
- Achieves broad distribution and increases $Na_V1.1$ protein expression in NHPs
- NHP toxicology studies support current clinical dosing

Sources: Targeted Augmentation of Nuclear Gene Output (TANGO) of SCN1A reduces seizures and rescues parvalbumin positive interneuron firing frequency in a mouse model of Dravet syndrome (AES 2020). Wengert ER, Wagley PK, Strohm SM, Reza N, Wenker IC, Gaykema RP, Christiansen A, Liau G, Patel MK. Targeted Augmentation of Nuclear Gene Output (TANGO) of Scn1a rescues parvalbumin interneuron excitability and reduces seizures in a mouse model of Dravet Syndrome. Brain Res. 2022;1775:147743. Stoke data. TANGO oligonucleotides for the treatment of Dravet Syndrome: Safety, biodistribution and pharmacology in the non-human primate (AES 2019)

Phase 1/2a Trials of STK-001 for Dravet Syndrome are Ongoing

Parallel studies in the US & UK evaluating children and adolescents ages 2 to 18 years old

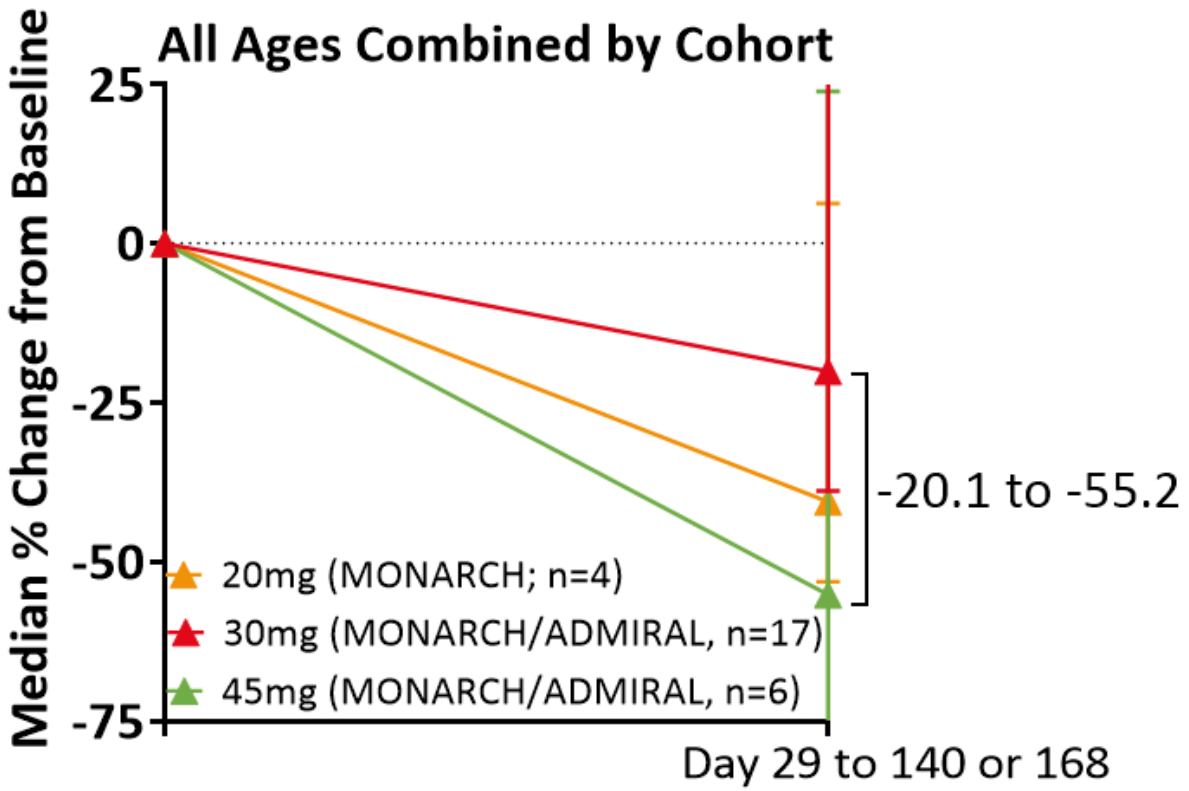
	monarch	admiral
Design	Evaluation of STK-001 (up to 45mg*)	Evaluation of STK-001 (up to 70mg)
Status	MAD @45mg: Dosing ongoing	MAD @70mg: Dosing ongoing
Primary Endpoint	Safety and tolerability of SAD and MAD dose levels	Safety and tolerability of MAD dose levels
	Characterize human pharmacokinetics (PK) and cerebrospinal fluid (CSF) drug exposure	
Secondary Endpoint	Change in seizure frequency, overall clinical status, and quality of life	
Open-Label Extension	Enrollment and dosing ongoing (30mg) swallowtail	Enrollment and dosing ongoing (45mg) Longwing

*Doses >45mg remain on FDA partial clinical hold

Sources: Interim Safety, PK, and CSF Exposure Data from the Phase 1/2a MONARCH Study of STK-001, an Antisense Oligonucleotide (ASO), in Children and Adolescents with Dravet Syndrome (AES 2021). ADMIRAL: A UK Study of the Safety and Pharmacokinetics of Antisense Oligonucleotide STK-001 in Children and Adolescents with Dravet Syndrome (AES 2021). Interim Analysis of STK-001 for the Treatment of Dravet Syndrome, Presented on November 14, 2022.

55% Median Reduction in Convulsive Seizure Frequency Observed in Patients Treated With Three Doses of STK-001 (45mg)

Across the multiple dose cohorts (20mg, 30mg, 45mg), 74% (20/27) of patients experienced reductions in seizures

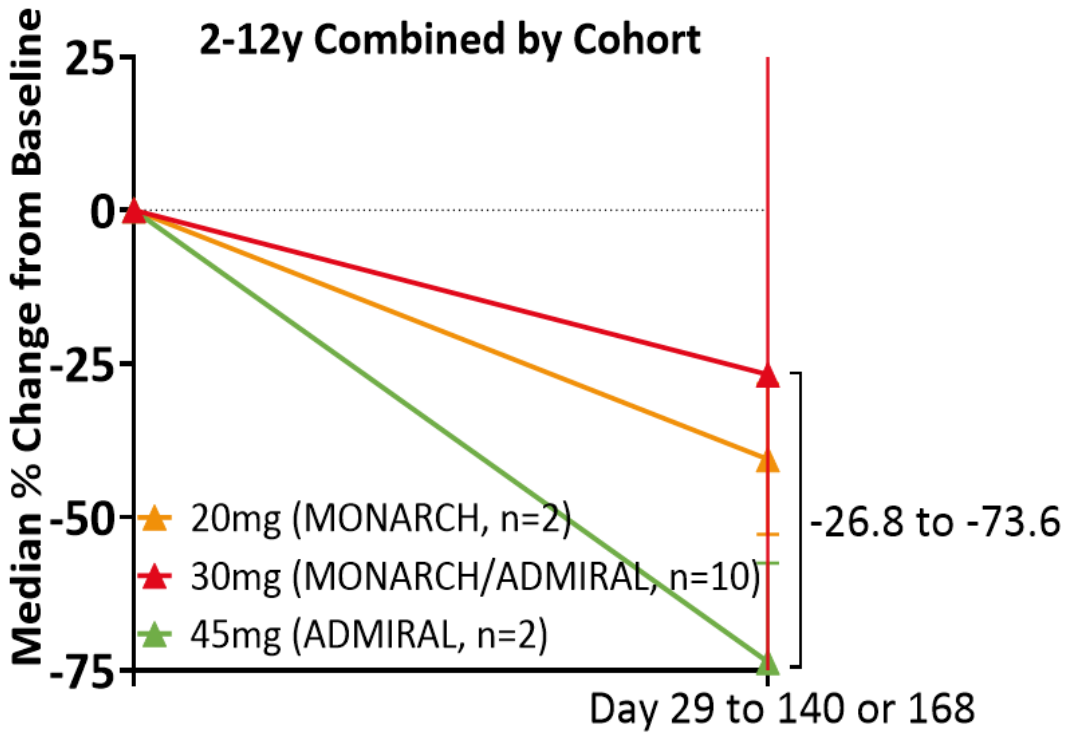


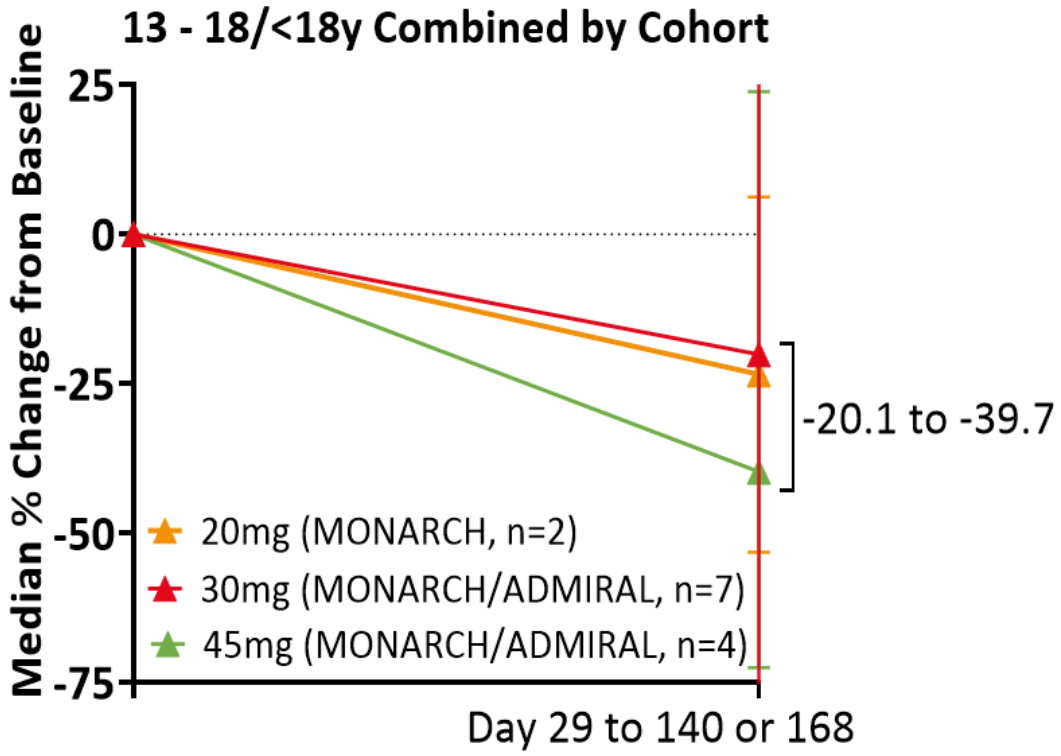
Similar seizure reduction was observed among patients taking or not taking concomitant fenfluramine (>50% of patients were taking concomitant fenfluramine)

Source: Preliminary Interim Analysis of STK-001 for the Treatment of Dravet Syndrome from the Phase 1/2a MONARCH and ADMIRAL Studies, Presented on November 14, 2022.

Reductions in Convulsive Seizure Frequency Observed Across Age Groups

Seizure reductions more evident among patients ages 2-12

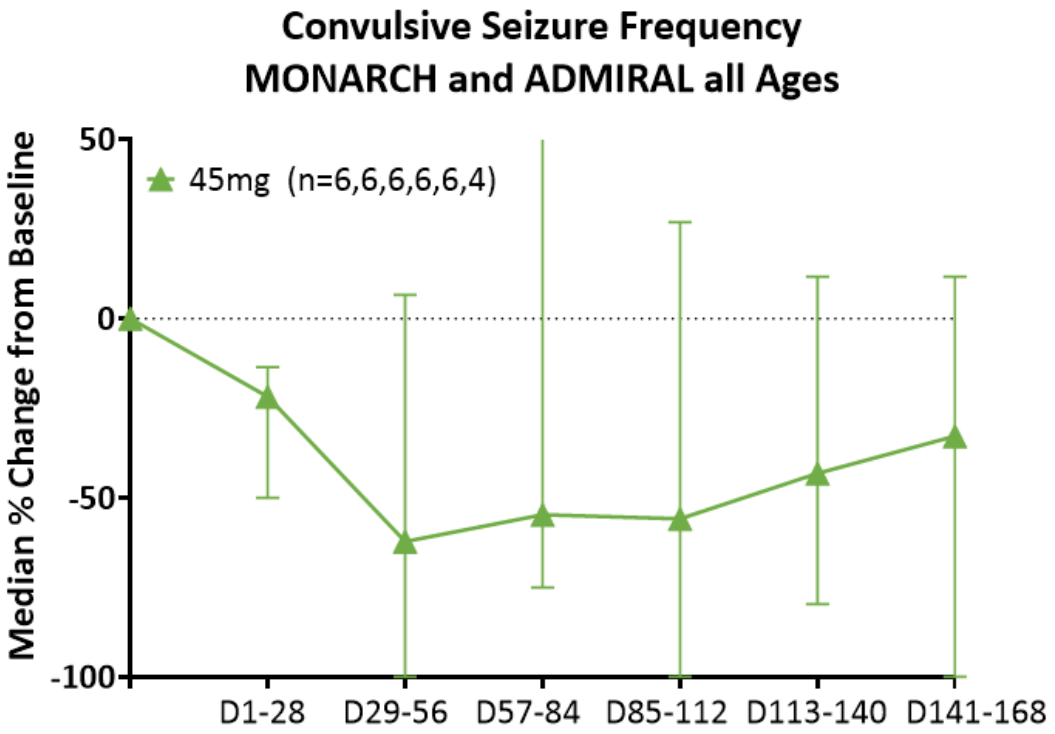




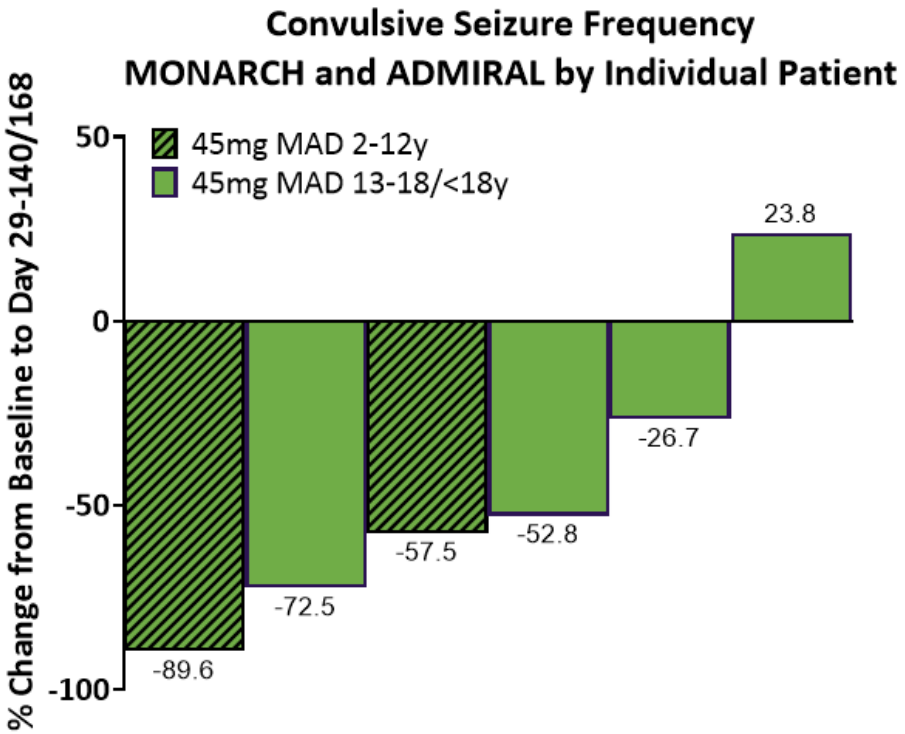
Source: Preliminary Interim Analysis of STK-001 for the Treatment of Dravet Syndrome from the Phase 1/2a MONARCH and ADMIRAL Studies, Presented on November 14, 2022.

67% (4/6) Patients Experienced a Greater Than 50% Reduction in Convulsive Seizure Frequency

Reductions began after the first dose and continued with additional treatment



83% (5/6) experienced a reduction from baseline in convulsive seizure frequency after three doses (45mg)

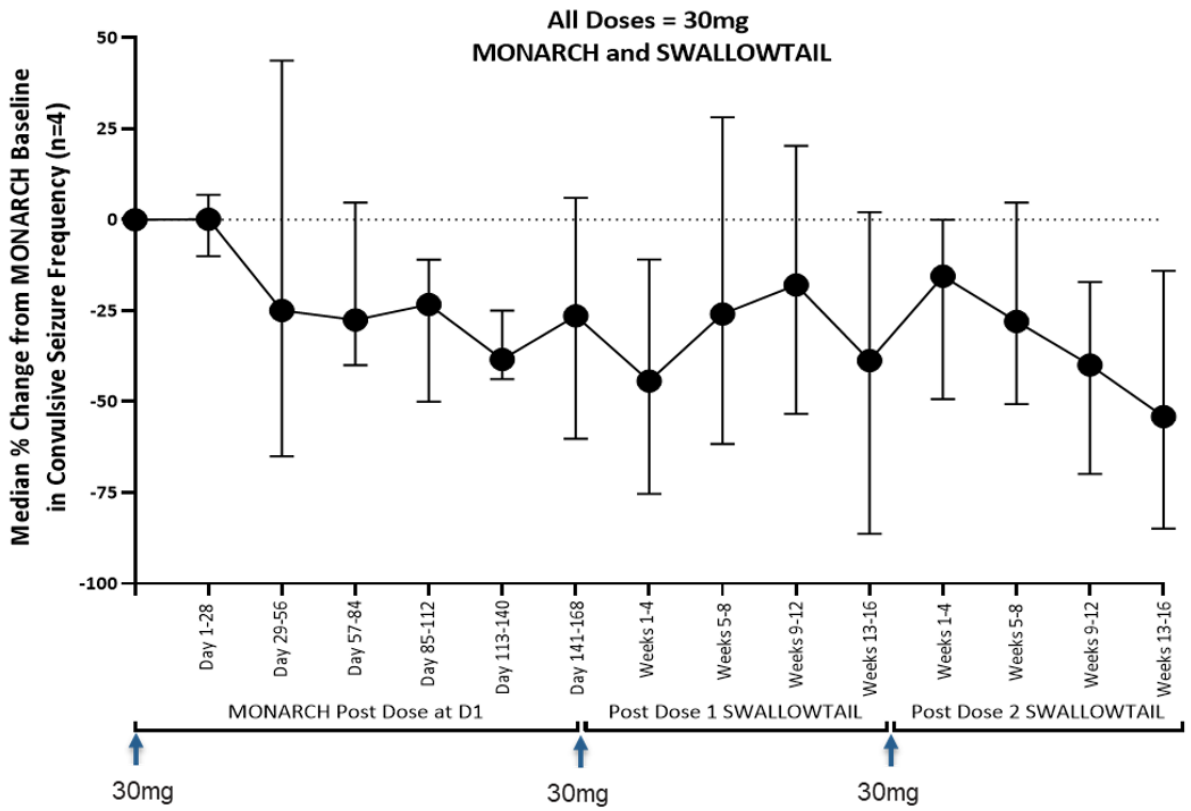


4/6 patients were taking concomitant fenfluramine

Source: MONARCH and ADMIRAL Interim Analyses: Phase 1/2a Studies Investigating Safety and Drug Exposure of STK-001, an Antisense Oligonucleotide (ASO), in Children and Adolescents with Dravet Syndrome (DS) (AES 2022)

Reductions in Seizure Frequency Were Maintained with Ongoing Treatment

CONVULSIVE SEIZURE FREQUENCY

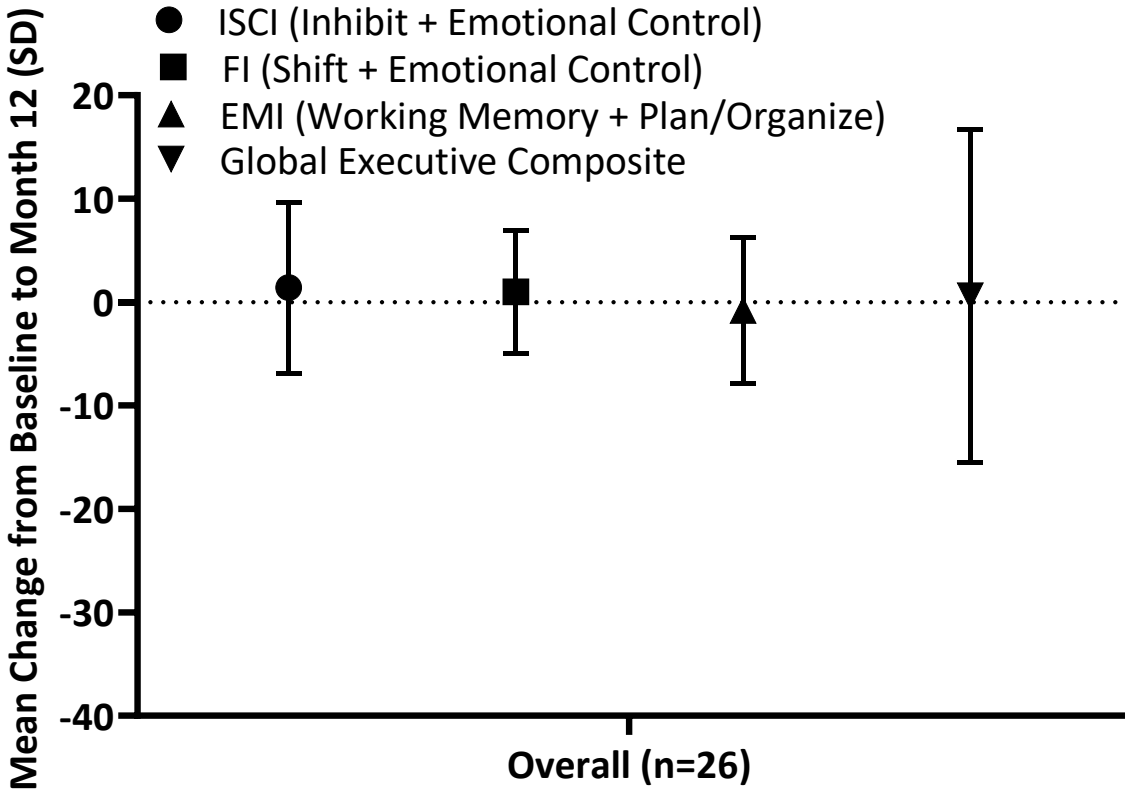


No exclusions for AED modification

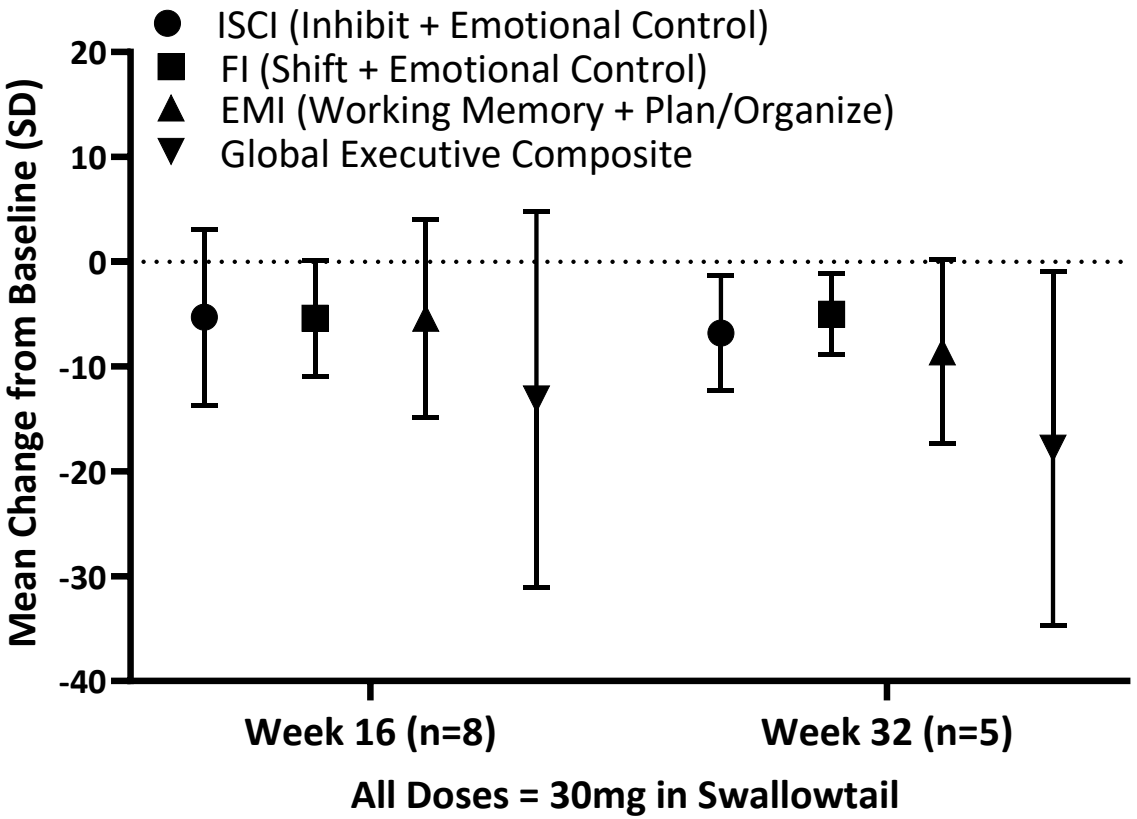
Source: SWALLOWTAIL: An Open-Label Extension (OLE) Study for Children and Adolescents with Dravet Syndrome (DS) who Previously Participated in a Study of Antisense Oligonucleotide (ASO) STK-001 (AES 2022)

Early Indication of Improvements in Non-Seizure Comorbidities as Measured by BRIEF-P*

BUTTERFLY
An observational study of Dravet Syndrome patients



swallowtail
A DRAVET SYNDROME EXTENSION STUDY



All Doses = 30mg in Swallowtail

*Behavior Rating Inventory of Executive Function–Preschool Version, an assessment of pediatric executive function

Source: Preliminary Interim Analysis of STK-001 for the Treatment of Dravet Syndrome from the Phase 1/2a MONARCH and ADMIRAL Studies, Presented on November 14, 2022.

STK-001 Has Potential to Address the Genetic Cause of Dravet Syndrome (DS)

Summary of Key Ph1/2a Interim Data

- Single and multiple doses of STK-001 up to 45mg were well-tolerated ✓
- 55% median reduction in convulsive seizure frequency observed in patients treated with three doses of STK-001 (45mg) ✓
- Reductions in Seizure Frequency Were Maintained with Ongoing Treatment ✓
- Early indication of improvements in non-seizure comorbidities as measured by BRIEF-P** ✓

Additional data anticipated in 2023 from the 45mg and 70mg multiple dose cohorts

Source: Preliminary Interim Analysis of STK-001 for the Treatment of Dravet Syndrome from the Phase 1/2a MONARCH and ADMIRAL Studies, Presented on November 14, 2022.
*Multiple dose cohorts (20mg, 30mg, 45mg)
**Behavior Rating Inventory of Executive Function–Preschool Version, an assessment of pediatric executive function

Autosomal Dominant Optic Atrophy (ADOA): A Severe, Progressive Optic Nerve Disorder

65-90%

of cases caused by mutations in one allele of the *OPA1* gene, most of which lead to a **HAPLOINSUFFICIENCY**

RESULTING in

50%

OPA1 protein expression and disease manifestation

1 out of 30,000

people are affected globally with a higher incidence of ~1 out of 10,000 in Denmark due to a founder effect

>400

Different *OPA1* mutations reported in ADOA patients

Up to 46%

of patients are registered legally blind

80%

of patients are symptomatic by age 10

~18,000

people affected in the U.S., Canada, Japan, Germany, France and the UK

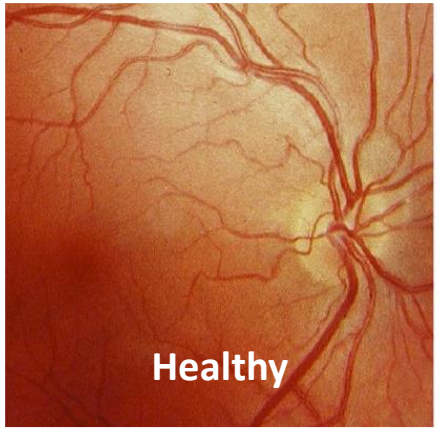
Sources: Yu-Wai-Man P et al. *Ophthalmology*, 2010; Yu-Wai-Man P, Chinnery PF. *Ophthalmology*, 2013; P. Amati-Bonneau P et al. *The International Journal of Biochemistry & Cell Biology*, 2009; Lenaers G, Hamel C, Delettre C, et al. *Orphanet J Rare Dis*, 2012; Chun BY and Rizzo JF III. *Curr Opin Ophthalmol,* 2016; Le Roux B, Lenaers G, Zanlonghi X et al. *Orphanet J Rare Dis*, 2019; "What is ADOA?" Autosomal Dominant Optic Atrophy Association. Accessed May 6, 2020, from https://www.adoaa.org/what-is-adoa;

No Approved Disease-Modifying Therapies for ADOA

Healthy Vision

Simulation of Optic Neuropathy

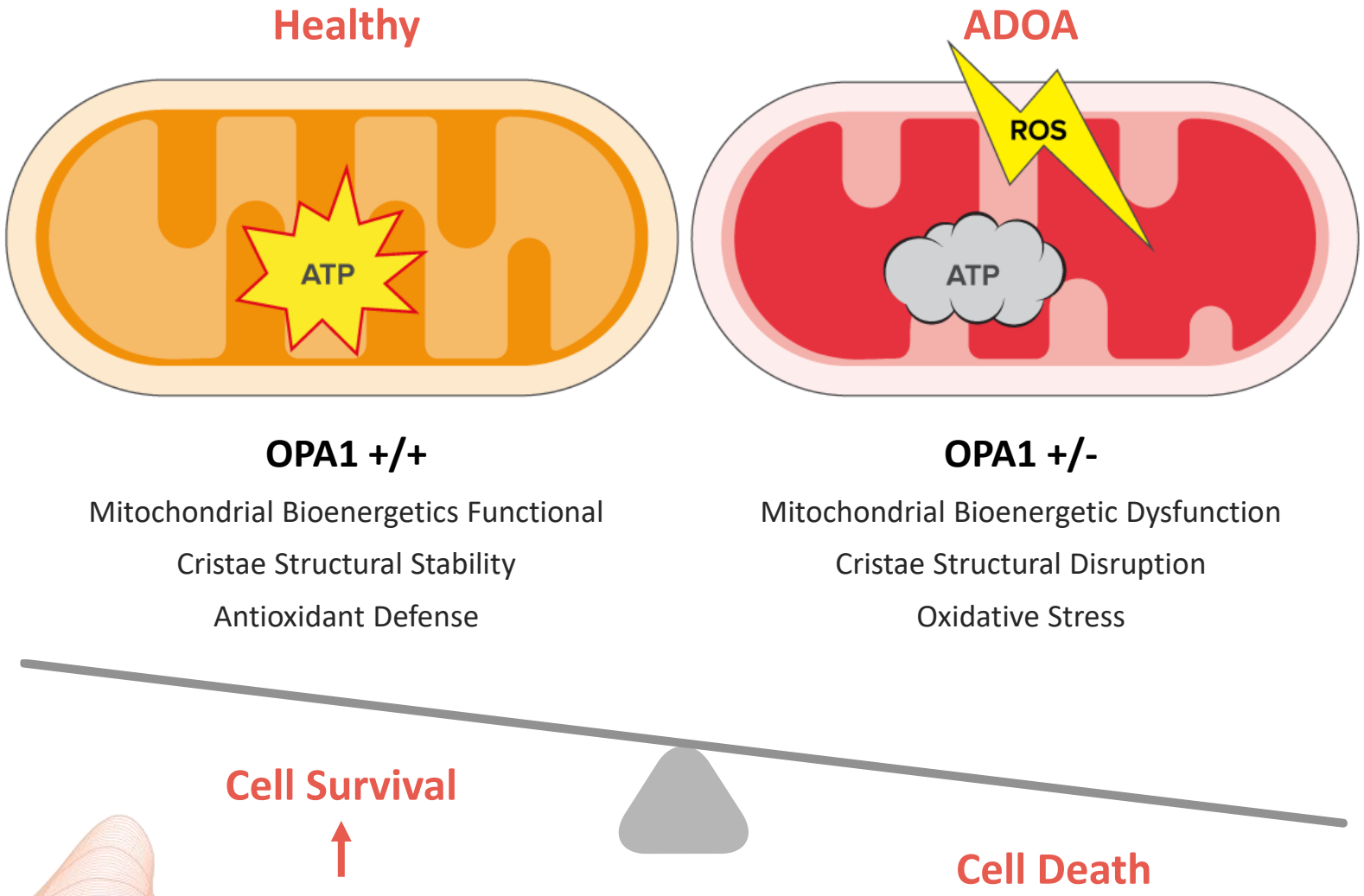
- Most common inherited optic nerve disorder
- Leads to central field defects and reduced color vision in both eyes
- Severity can vary; rate of vision loss difficult to predict
- Supportive services and low-vision aids are offered for patients



Sources: Yu-Wai-Man P et al. *Ophthalmology*, 2010; Yu-Wai-Man P, Chinnery PF. *Ophthalmology*, 2013;
Lenaers G, Hamel C, Delettre C, et al. *Orphanet J Rare Dis*, 2012; Chun BY and Rizzo JF III. *Curr Opin Ophthalmol,* 2016
Image of child sourced from ICR, Ophthalmology Center Barcelona. Accessed Jan. 8, 2021 from https://icrcat.com/en/eye-conditions/leber-hereditary-optic-neuropathy/ Credit: Lhon Eye Society Sweden. Image shown depicts Leber Hereditary Optic Neuropathy, which presents visual effects similar to ADOA.

OPA1 is Critical for Normal Mitochondrial Function and Cellular Metabolism

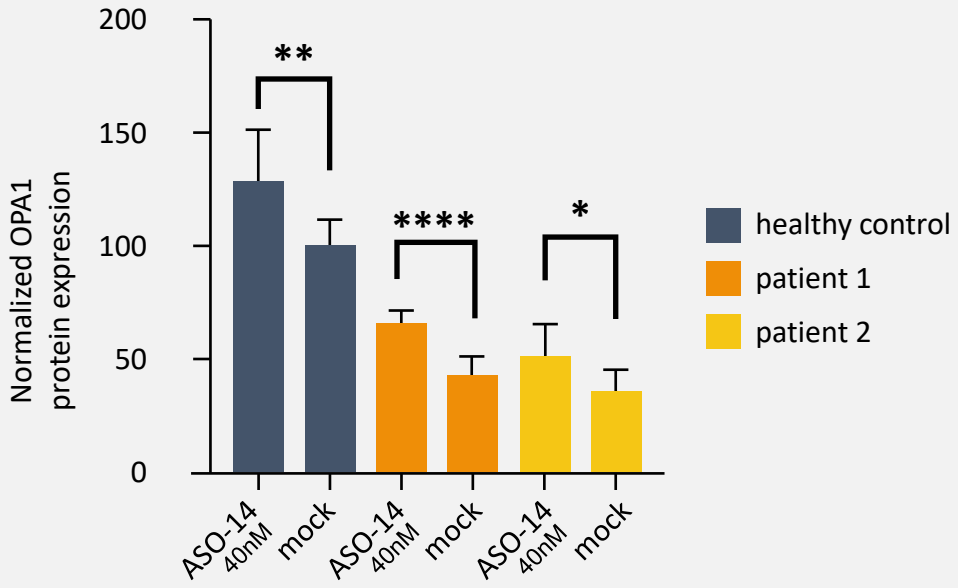
- Retinal ganglion cells have very high energy (ATP) requirements
- Impaired mitochondrial function leads to cell death
- OPA1 is critical for mitochondrial function and energy production



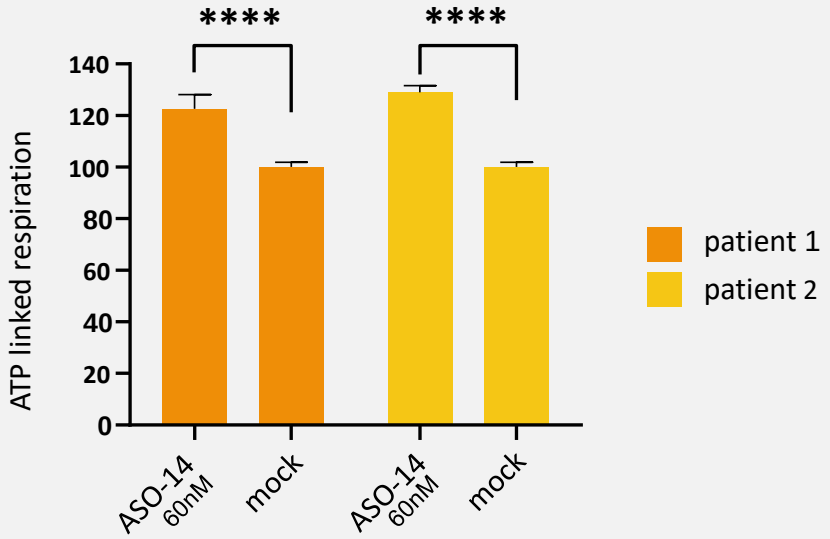
* ROS = Reactive Oxygen Species

TANGO ASO Increases OPA1 Protein and ATP Linked Mitochondrial Respiration in ADOA Patient Cells

ASO treatment increased OPA1 protein levels in OPA1 deficient ADOA patient cells



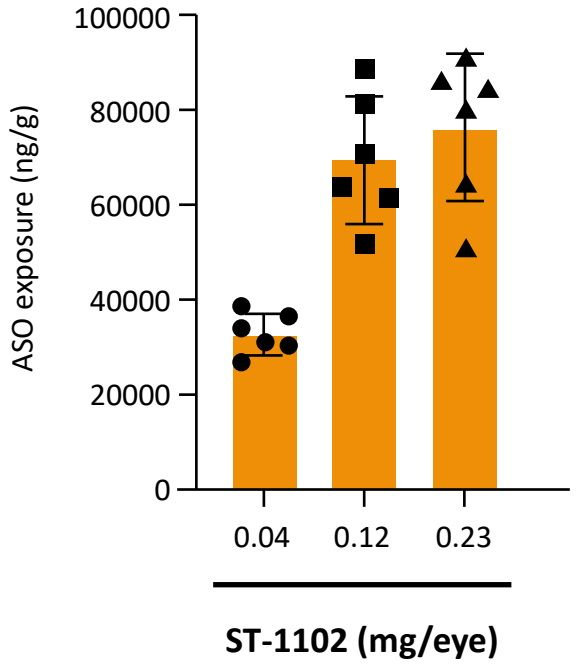
ASO treatment increased ATP linked respiration in OPA1 deficient ADOA patient cells



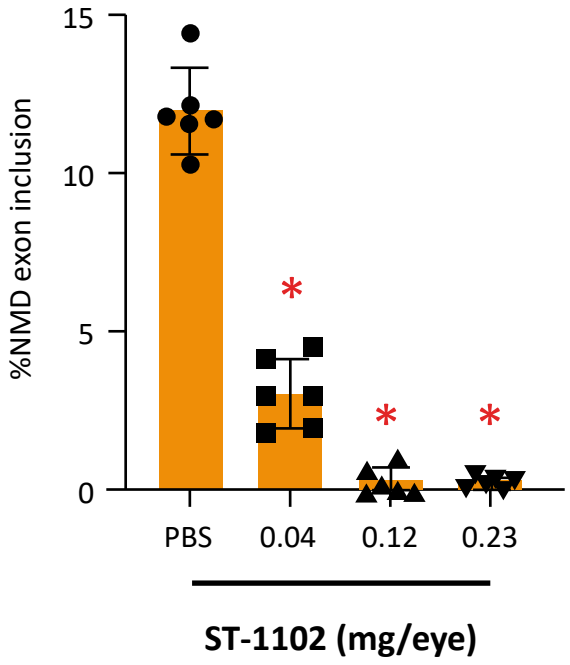
Source (left graph): Stoke data
Source (right graph): Venkatesh A, et al. Antisense oligonucleotide mediated increase in OPA1 improves mitochondrial function in fibroblasts derived from patients with autosomal dominant optic atrophy (ADOA). Presented at The Association for Research in Vision and Ophthalmology; May 1-7, 2021.

TANGO ASO Demonstrates Dose-Dependent OPA1 Protein Increases in Rabbit Retina

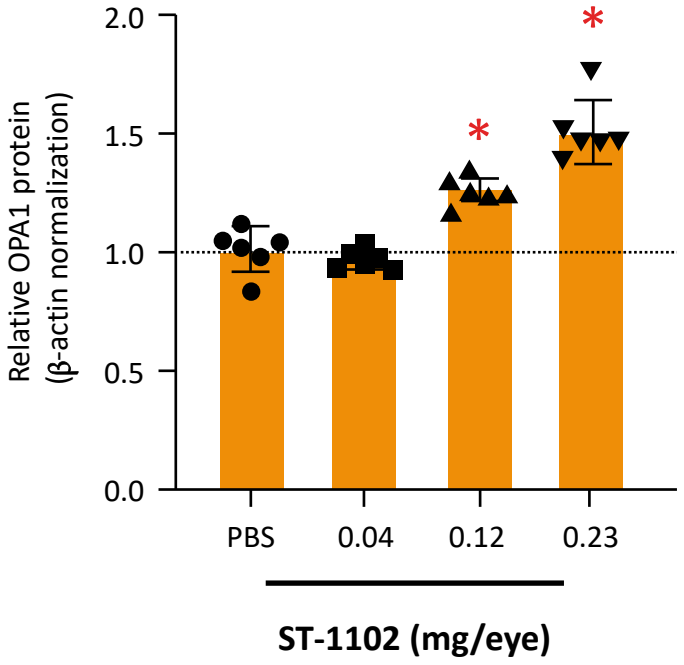
ASO exposure in retina Day 29



Target engagement Day 29



OPA1 protein Day 29

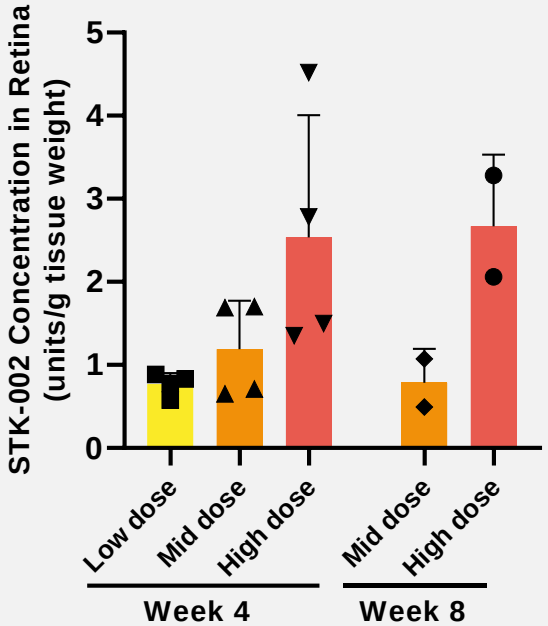


***P*<0.0005 by one-way ANOVA compared to PBS group**

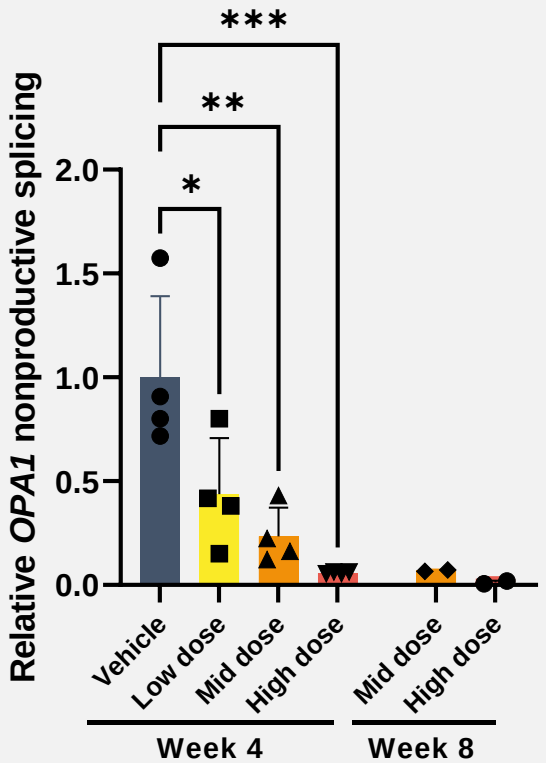
Source: Steven Gross, et al. A Prospective Natural History Study of Patients with Autosomal Dominant Optic Atrophy (ADOA) – FALCON. Presented at The North American Neuro-Ophthalmology Society; February 12-17, 2022; Austin, TX.

Dose-Related Target Engagement and OPA1 Protein Upregulation in Retinal Tissue of NHPs following IVT Administration of STK-002

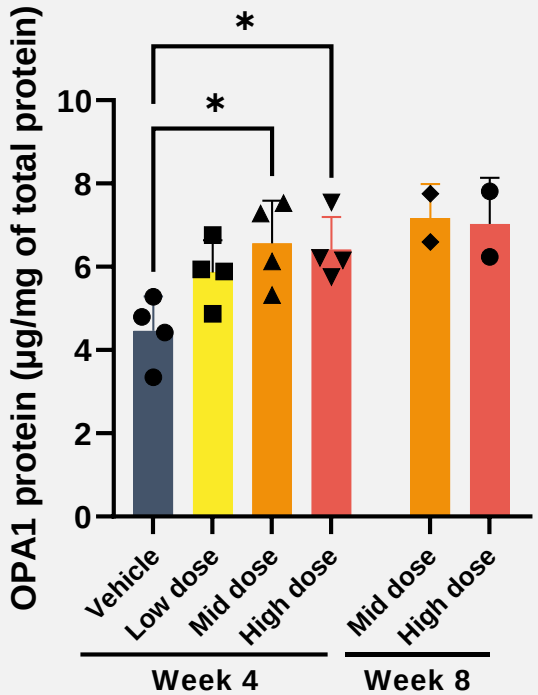
STK-002 exposure



Target engagement



OPA1 protein



NHP: Non-human primates
IVT: Intravitreal
Source: Venkatesh A, et al. STK-002, an Antisense Oligonucleotide (ASO) for the Treatment of Autosomal Dominant Optic Atrophy (ADOA), is Taken Up by Retinal Ganglion Cells (RGC) and Upregulates OPA-1 Protein Expression After Intravitreal Administration to Non-human Primates (NHPs). ASGCT; May 16-19, 2022.

TANGO ASOs Have the Potential to Address the Genetic Cause of ADOA

Summary of Key Preclinical Data

- Increase OPA1 protein and ATP linked respiration in ADOA patient cells ✓
- Result in dose-dependent increases in OPA1 protein expression in rabbit retina ✓
- Were well tolerated for up to 29 days after intravitreal injection in rabbit ✓
- Dose-related increase in OPA1 protein expression was observed in NHP RGCs ✓

Preclinical toxicology studies ongoing in 2022 to support future clinical trials for STK-002

Sources: Venkatesh A, et al. STK-002, an Antisense Oligonucleotide (ASO) for the Treatment of Autosomal Dominant Optic Atrophy (ADOA), is Taken Up by Retinal Ganglion Cells (RGC) and Upregulates OPA-1 Protein Expression After Intravitreal Administration to Non-human Primates (NHPs). ASGCT; May 16-19, 2022.

Collaboration with Acadia Pharmaceuticals to Pursue RNA-Based Treatments for Severe & Rare Genetic Neurodevelopmental Diseases

Collaboration leverages Stoke's proprietary TANGO research platform and Acadia's expertise in neurology drug development and commercialization

3 targets focused on severe and rare genetic neurodevelopmental diseases of the central nervous system

- Acadia receives exclusive worldwide licenses for:
  - Rett syndrome (*MECP2*)
  - Undisclosed neurodevelopmental target
- 50:50 co-development co-commercialization of SYNGAP1

Stoke receives a $60M upfront payment and potential milestones up to $907M as well as royalties on future sales

- Acadia fully funds the research and preclinical development activities for Rett syndrome (*MECP2*) and undisclosed neurodevelopmental program
- Share 50/50 in all world-wide costs and future profits for SYNGAP1 program

Investing In Our Pipeline

PROGRAM	TARGET	DISCOVERY & PRECLINICAL	PHASE 1/2	PHASE 3	PARTNER
Central Nervous System					
Dravet Syndrome	*SCN1A*		STK-001		100% Stoke Global
SYNGAP1 Syndrome	*SYNGAP1*				Stoke : Acadia 50:50
Rett Syndrome	*MECP2*				Acadia Worldwide License
Undisclosed	*Undisclosed*				Acadia Worldwide License
Ophthalmology					
ADOA	*OPA1*	STK-002			100% Stoke Global

Rett Syndrome: A Severe, Debilitating Neurological Disorder

~33%

of cases caused by hypomorphic mutations of the *MECP2* gene[1]

RESULTING in

Partial loss of function of the MeCP2 protein

1 out of **10,000** to **15,000** females are born with Rett syndrome[2]

Period of rapid decline typically begins between

6 to **18**

months[4]

Symptoms include[3]:

- **Loss of purposeful hand use**
- **Involuntary hand movements such as handwringing**
- **Loss of speech**
- **Loss of mobility or gait disturbances**

60-80% of patients have **epilepsy**[4]

Note: All seizure types have been reported in Rett syndrome. Complex partial and generalized tonic-clonic are the most common
Sources: [1] RettBase (http://mecp2.chw.edu.au/); GnomAD (https://gnomad.broadinstitute.org); NOMAD; [2] National Institutes of Health – National Institute of Neurological Disorders and Stroke; [3] International Rett Syndrome Foundation; [4] Operta et al., Brain Behav 2019

SYNGAP1 Syndrome: A Severe Intellectual Disability / Developmental and Epileptic Encephalopathy (ID/DEE)

>80%

of cases caused by a **HAPLOINSUFFICIENCY** of the *SYNGAP1* gene[1]

RESULTING in

50%

SynGAP protein expression

1-2 **out of** **100,000** children are born with SYNGAP1-ID/DEE

1-2%

of all **intellectual disability** cases[2]

84%

of patients have **generalized epilepsy**[3]

100%

of patients have **developmental delay** or **intellectual disability**[3]

~50%

of patients have **autism and other behavioral abnormalities**[3]

Sources: [1] Parker et al., *American Journal of Medical Genetics*, 2015; Jimenez-Gomez et al., Journal of Neurodevelopmental Disorders, 2019; [2] SYNGAP1 Resource Guide, Second Edition; An Overview of SYNGAP1 Basic Biology and Clinical Description. Bridge the Gap SYNGAP (now SYNGAP1 Foundation); SynGAP Research Fund; [3] SYNGAP1-Related Intellectual Disability: https://www.ncbi.nlm.nih.gov/books/NBK537721/#_syngap1-id_Clinical_Characteristics_

Our Strategy For 2022

Advance our wholly owned CNS and ophthalmology programs and expand the scope of our drug discovery efforts

Advance STK-001 for Dravet Syndrome

- Additional clinical data on STK-001 (30mg MAD) anticipated in 2H22
- Initiate dosing >30mg in MONARCH and ADMIRAL

Advance STK-002 for ADOA

- Conduct preclinical toxicology studies to support future clinical trials for STK-002
- Begin enrollment in prospective ADOA natural history study
- Present additional preclinical data for STK-002 at scientific forum

Develop & Expand Pipeline

- Continue discovery efforts to identify new targets
- Execute on collaboration with Acadia

Current Liquidity Anticipated to Fund Operations into 2025

$252.2M

Cash, Cash Equivalents, Marketable Securities, and Restricted Cash

as of 9/30/2022

39.4M

Common Shares Outstanding

as of 9/30/2022

STOKE THERAPEUTICS