

Stoke Therapeutics First Quarter 2026 Business Update

Webcast for Investors & Analysts

May 7, 2026

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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, statements regarding: the ability of zorevunersen to treat the underlying causes of Draven syndrome and reduce seizures or show improvements in behavior or cognition at the indicated dosing levels or at all; the potential for zorevunersen to be a first-in-class, disease-modifying therapy for Draven syndrome; the clinical development of zorevunersen, STK-002 and our other product candidates, including the initiation, timing and expected progress of clinical trials (including our EMPEROR Phase 3 study and our OSPREY Phase 1 study); the timing of regulatory interactions or the outcomes thereof; our ability to achieve an NDA submission or approval on the expedited timeframe disclosed or at all; the potential for us to advance and develop our research and development programs; our expectations, plans, aspirations and goals, including those related to the potential of zorevunersen and our collaborations with Biogen and Acadia; the anticipated market, access, reimbursement and label, if any, for zorevunersen; our future operating results, financial position and cash runway and ability to fund operations into 2028 and through to a potential U.S. commercial launch. Statements including words such as "anticipate," "believe," "hope," "plan," "will," "continue," "expect," "ongoing," or "potential," and statements in the future tense are forward-looking statements. These forward-looking statements involve risks and uncertainties, as well as assumptions, which, if they prove incorrect or do not fully materialize, could cause our results to differ materially from those expressed or implied by such forward-looking statements, including, but not limited to, risks and uncertainties related to: our ability to advance, obtain regulatory approval of, and ultimately commercialize our product candidates, including zorevunersen and STK-002; the timing of data readouts and interim and final results of nonclinical and clinical studies; nonclinical and clinical data are voluminous and detailed, and regulatory authorities may interpret or weigh the importance of data differently and reach different conclusions than us or others, request additional information, have additional recommendations, or change their guidance or requirements before or after approval; receiving Breakthrough Therapy Designation may not lead to a faster development or regulatory review or approval and does not mean zorevunersen will receive marketing approval; our ability to fund development activities and achieve development goals; our ability to protect our intellectual property; the global business, political and macroeconomic conditions, including inflation, interest rate volatility, cybersecurity events, uncertainty with respect to the federal budget, instability in the global banking system and volatile market conditions, and global events, including public health crises and ongoing geopolitical conflicts, such as the conflicts in Ukraine and the Middle East; and other risks and uncertainties described under the heading "Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2025, our quarterly reports on Form 10-Q and the other documentation we file from time to time with the Securities and Exchange Commission. These forward-looking statements speak only as of the date of this presentation, and we undertake no obligation to revise or update any forward-looking statements to reflect events or circumstances after the date hereof.

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This presentation discusses product candidates, including zorevunersen and STK-002, that have not yet been approved for marketing by the U.S. Food and Drug Administration or any other regulatory agency.

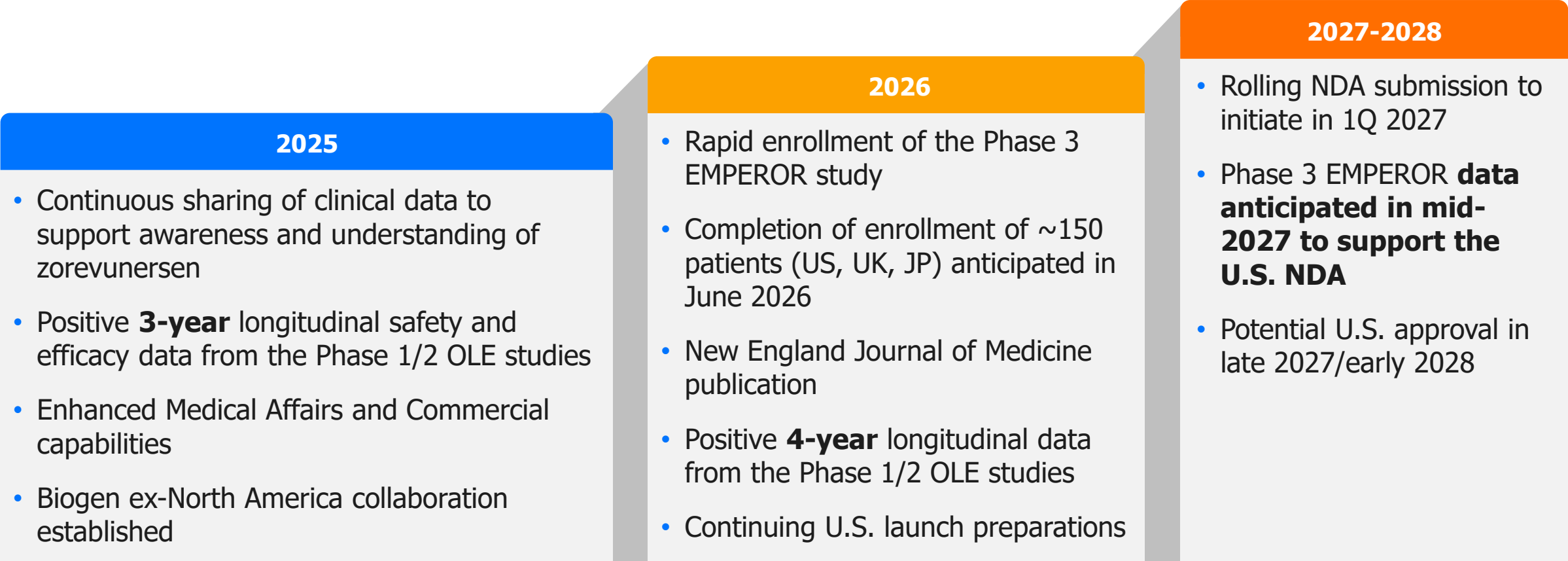
Opening Remarks

Ian F. Smith

Chief Executive Officer & Director

An Important Growth Trajectory Driven by Appreciation for zorevunersen, Phase 3 Progress

Stoke has transformed itself over the last year and is well positioned for our next phase of growth



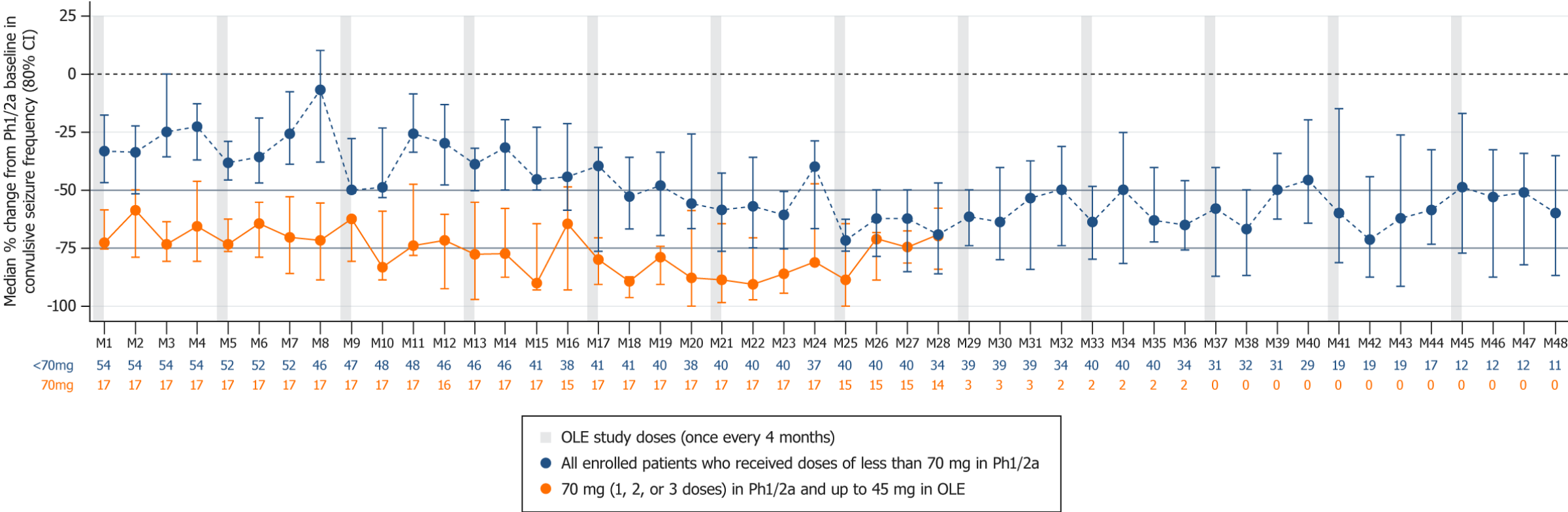
4-Year Longitudinal Data and Phase 3 Progress

Barry Ticho, M.D., Ph.D.

Chief Medical Officer

Substantial, Durable Reductions in Seizure on Top of SOC Observed Through Four Years of Treatment with Zorevunersen

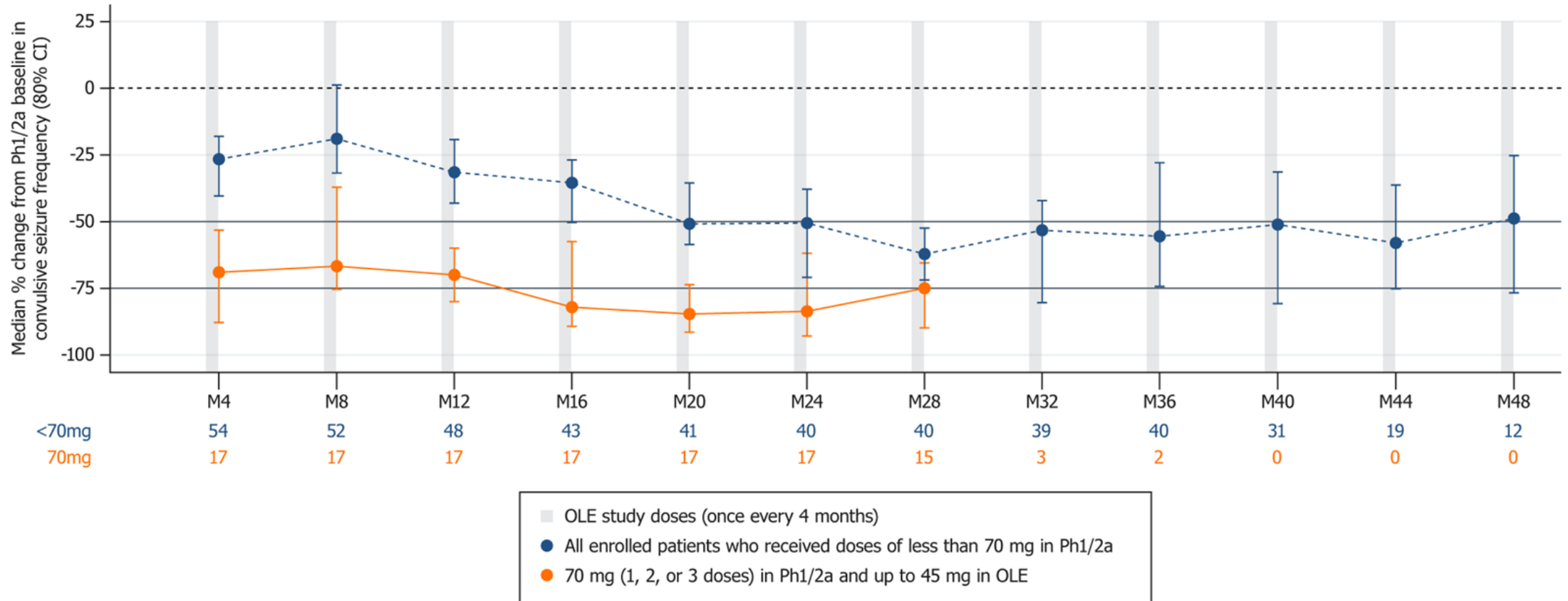
Data for all patients who continued treatment in the OLEs separated by dose received in the Ph1/2a studies



Data cut date: 19 Feb 2026; 1 Month – 4 Weeks; One patient who received an incorrect dose of zorevunersen in Phase 1/2a, 3 patients who experienced less than the minimum number of convulsive seizures during Phase 1/2a baseline, and 1 patient who transferred into OLE with a delay of approximately 10 months were excluded. Patients were not included in 6M after last Ph1/2a dose time point if they didn't enter OLE. No exclusions were made for ASM modification. Intervals with <50% diary data were excluded for individual patients. This accounts for the difference in M48 n's as measured in 28-day intervals (n=11) vs 16-week intervals (n=12).

Median Percent Change from Ph1/2a Baseline Through Year 4 Shown in 16-Week Intervals

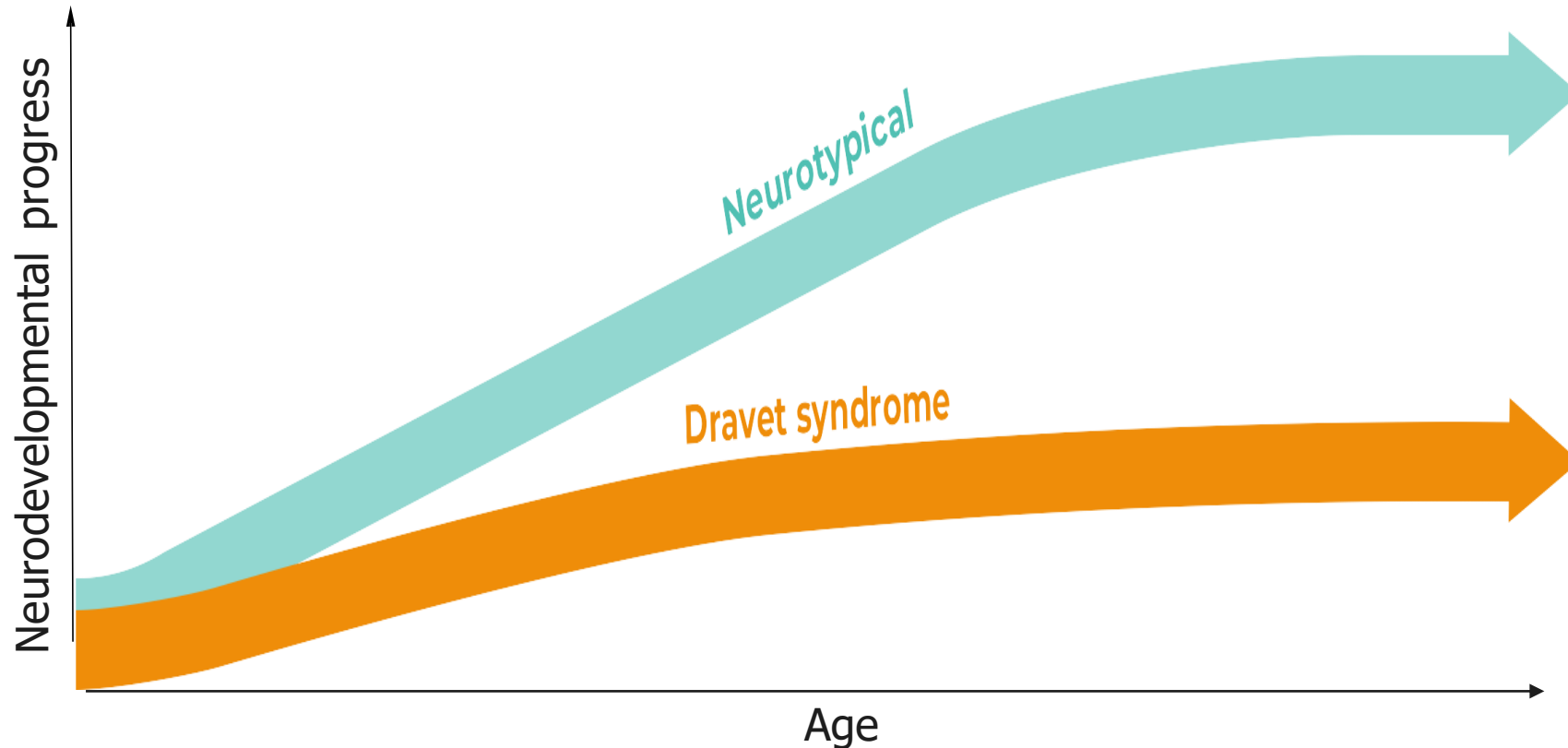
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Development in Patients with Dravet Syndrome Differs Markedly from that of Neurotypical Children





Neurodevelopment plateaus at ~2 years of age for children living with Dravet syndrome¹



Comparison of developmental trajectory between neurotypical children and patients with Dravet syndrome

The Vineland-3 Assessment Tool is Commonly Used to Evaluate Non-Seizure Outcomes in Dravet Syndrome

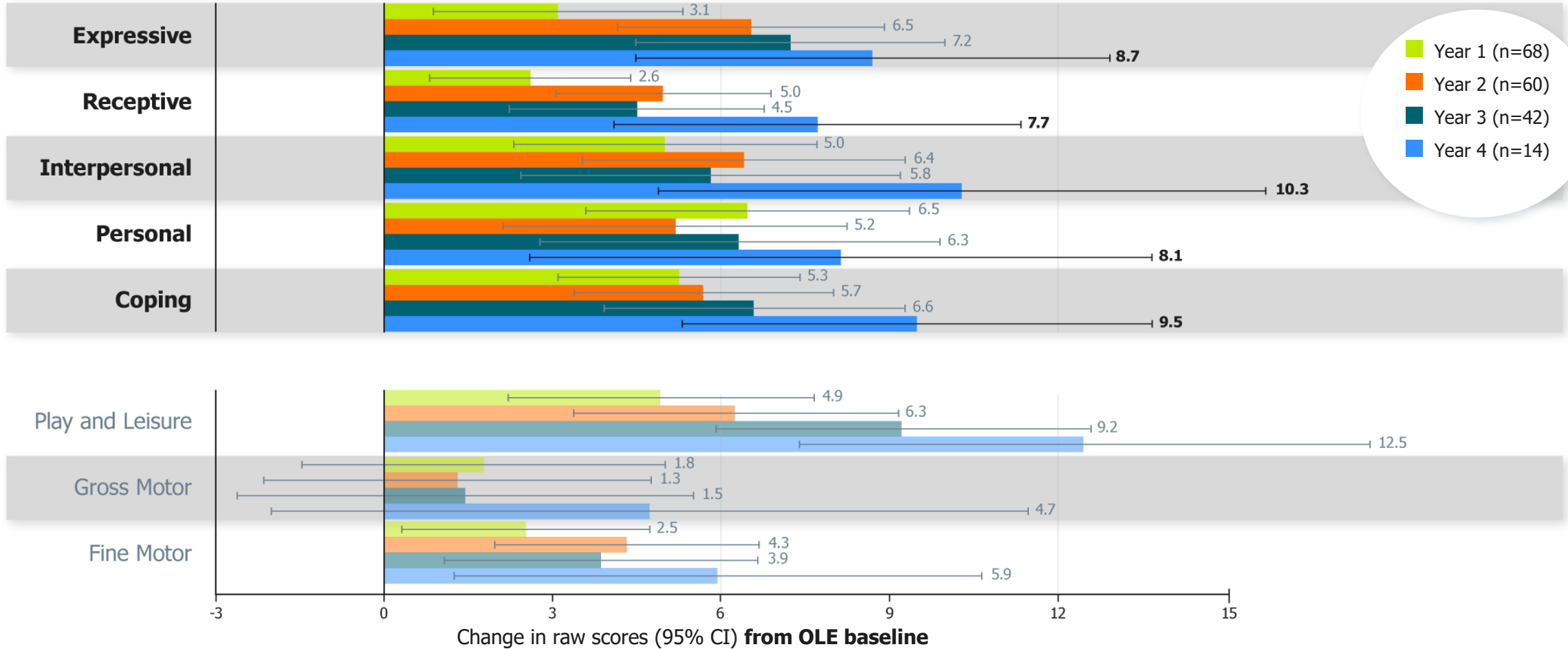
Vineland-3 Adaptive Behavior Scales*

Domains	 <p>COMMUNICATION</p>	 <p>SOCIALIZATION</p>	 <p>MOTOR SKILLS</p>	 <p>DAILY LIVING SKILLS</p>
	Subdomains	<p>Receptive: Responds upon hearing name called</p> <p>Expressive: Says "Dada", "Mama", or caregiver name</p> <p>Written: Writes alphabet letters using correct orientation</p>	<p>Interpersonal Relationships: Responds upon hearing name called</p> <p>Play and Leisure: Responds when parent or caregiver is playful</p> <p>Coping Skills: Transitions easily from one activity to another</p>	<p>Gross Motor: Moves, scoots, or crawls across the floor</p> <p>Fine Motor: Picks up small objects with thumb and fingers</p>

4-Year Phase 1/2 OLE Data Showed Continuing Improvements in Cognition and Behavior

<0.01 statistical significance achieved in 5 key subdomains for each year compared to OLE baseline

Phase 3 EMPEROR Subdomains



Datacut date: 19 Feb 2026; no patient exclusion; Data included through Week 192. 1 year = 48 weeks. "n" represents the number of completed patient visits at each timepoint shown; these values may not correspond exactly to the number of patients contributing to the model-based estimates for each subdomain. Mixed-effects model for repeated measures constructed using available data from enrolled patients in OLE studies.

Zorevunersen Generally Well-Tolerated with Long-Term Dosing

Phase 1/2a studies

(n=81)

- **30%** of patients experienced a study drug–related TEAE
- Most common: CSF protein elevations (14%) and procedural vomiting (5%)
- **22%** of patients experienced a TESAE
- All were unrelated to the study drug except for one patient with SUSARs
- 1 patient died due to SUDEP, **unrelated to zorevunersen**

OLE studies

(n=75)

- **CSF protein elevation*** occurred in **94%** of patients and was **classified as a TEAE in 59%**
 - No serious or severe clinical manifestations associated with CSF protein elevation were observed
 - One patient discontinued treatment due to elevated CSF protein
- 1 patient died due to SUDEP and 1 due to malnutrition; both deaths were **unrelated to zorevunersen**

>850 doses

administered to date in the Phase 1/2 and OLE studies

Patients have received treatment for **more than 5 years**

Phase 1/2a data cut: December 12, 2023 (after End of Study); OLE data cut: Feb 19, 2026.

** ≥ 1 CSF protein value > 50 mg/dL.*

72/75 patients in the OLEs had at least one assessment after baseline. 68/72 (94%) had elevated values and 40/68 (59%) were classified as a TEAE.

CSF, cerebrospinal fluid; SUSAR, suspected unexpected serious adverse reaction; TEAE, treatment-emergent adverse event; TESAE, treatment-emergent serious adverse event.

Enrollment of ~150 Patients Expected to Complete in June 2026 to Support a Data Readout in Mid-2027



Rolling U.S. NDA submission planned to initiate in Q1 2027

~150

Patients in the U.S., UK, Japan



- New patient entry into **screening now closed**
- Final patient expected to be randomized in **June 2026**
- **Data readout** anticipated in **mid-2027**

≥ 20



Patients in Europe

- 15/16 sites active in DE, FR, IT, SP
- Completion of enrollment expected in Q3 2026



Blinded 52-week treatment period

- Primary endpoint of change in seizure frequency measured at Week 28
- Key secondary endpoints measuring cognition and behavior (incl. 5 key Vineland subdomains) assessed at Week 52

Phase 3 enrollment in <1 year supports the need and support for zorevunersen, a potential disease-modifying treatment for Dravet syndrome

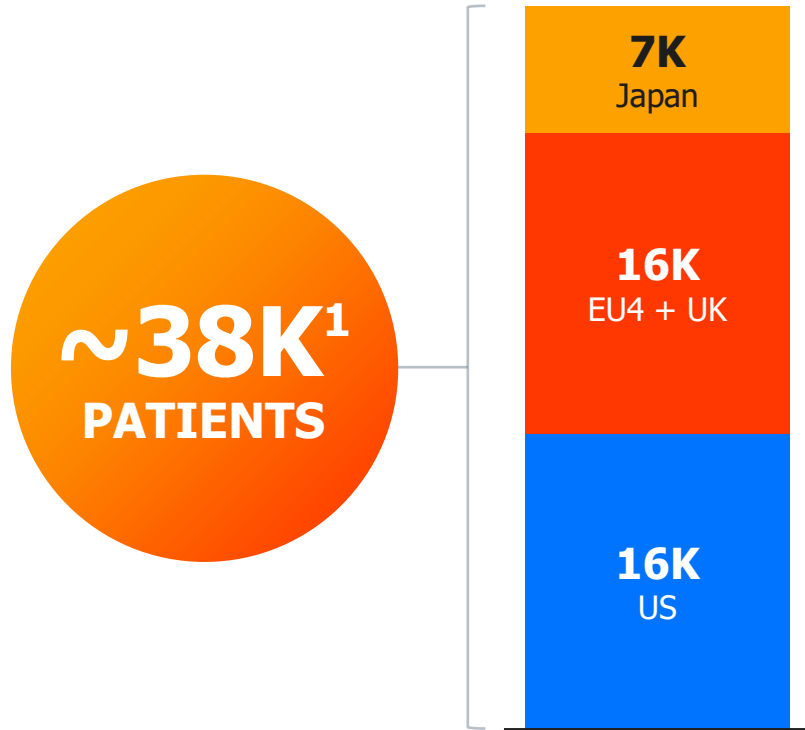
Preparing for a Potential U.S. Launch

Jason Hoitt
Chief Patient Officer

Significant Market Opportunity

~38K patients with Dravet syndrome across 7 major geographic markets

PREVALENCE OF DRAVET SYNDROME*



SIGNIFICANT NEED

DESPITE AVAILABILITY OF ANTI-SEIZURE MEDICINES (ASMs)

There are currently **no disease modifying medicines** approved for the treatment of Dravet syndrome

2-year natural history study² demonstrated **frequent seizures and significant cognitive and behavioral impairments** despite treatment with standard ASMs, including:

- A **plateauing in neurodevelopment at ~2 years of age** leading to a widening gap between children with Dravet syndrome and their neurotypical peers
- On average, 14.3 seizures/28 days at baseline and a **10.6 percent increase in major motor seizure frequency over two years**

*Numbers may not add up due to rounding. EU4: Germany, France, Italy and Spain; ASMs: anti-seizure medications.

¹Based on preliminary management estimates, which scaled annual incidence to prevalence using country-specific live birth rates over the past 85 years and adjusted for Dravet-specific mortality. The estimate is based on incidence rates published by Wu et al., *Pediatrics*, 2015. Lagae et al., *Developmental Medicine & Child Neurology*, 2017; 2018 Health Advances Report; Dravet Syndrome Foundation Voice of the Patient Report.;

²Sullivan J, Wirrell E, Knupp K. et al. Natural history of children and adolescents with Dravet syndrome: a 24-month follow-up. *Neurology*. 2025;105:e214388

Concentrated U.S. Market Enables Targeted Engagement



HCPs

~70% of Dravet patients seen by ~1,200 HCPs



Vast majority of high-volume treaters are neurologists



2022 treatment guidelines* support early genetic testing and use of a DMT as early as possible



Sites of Care

~70% of Dravet patients covered by 124 Sites of Care



Dravet Centers of Excellence identified from the Dravet Syndrome Foundation



Patients

16k Prevalent

6k Addressable at launch

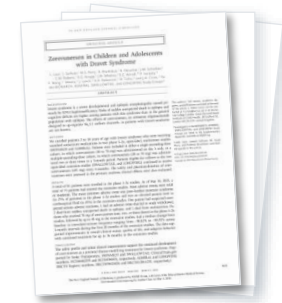
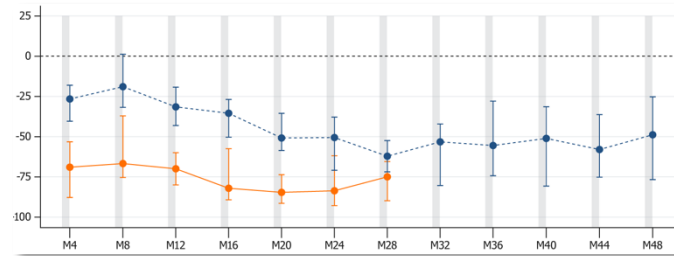


ICD10 code established in 2020 will continue to increase patient identification

SCREEN1A

“Unseen” disease awareness campaign to promote genetic testing and diagnosis

Confidence in the Value of zorevunersen as a Potential Disease-Modifying Treatment for Dravet Syndrome



Phase 3 Progress

- Dosing regimen, endpoint selection and powering assumptions **informed by robust Phase 1/2 and 2-year OLE dataset**
- **<1 year enrollment** driven by high awareness and need
- Data readout anticipated in mid-2027 to complete **planned U.S. rolling NDA submission**

Longitudinal Data

- **5 years of clinical data** from the Phase 1/2 and OLEs provide confidence in the disease-modifying potential of zorevunersen
- By our **mid-2027 Phase 3 readout**, an additional year of OLE data are expected
- HCPs and payers indicate these **OLE data may be the most compelling data** at the time of a potential approval¹

Visibility & Credibility

- Publication of **zorevunersen data in NEJM²**, the world's leading medical journal is enhancing awareness and understanding
- Publication supports education and access efforts

Financial Summary

Thomas Leggett

Chief Financial Officer

First Quarter 2026 Financial Summary

\$411.0M

Cash, Cash Equivalents, & Marketable Securities
as of 3/31/2026

(Includes \$80.7M of proceeds from ATM sales of 2.6M shares of common stock)

Current financial position anticipated to fund operations into 2028 and supports Phase 3 execution through to a potential U.S. launch

Closing Remarks

Ian F. Smith

Chief Executive Officer & Director

Q&A
