Stoke Therapeutics Second Quarter 2026 Business Update

Webcast for Investors & Analysts

August 3, 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 Dravet syndrome and reduce seizures or show improvements in behavior or cognition at the indicated dosing levels or at all; the potential benefits, safety and efficacy of zorevunersen; 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 ability of STK-002 to treat the underlying causes of Autosomal Dominant Optic Atrophy (ADOA) and maintain or improve vision; the potential for us to advance and develop our research and development programs, including our ability to enhance our platform expansion under our new CSO ; 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 through to a potential U.S. launch of zorevunersen in early 2028. Statements including words such as "anticipate," "believe," "expect," "hope," "plan," "will," "continue," "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 being 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; 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 form 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

Advancing Through a Period of Execution and Expansion

Zorevunersen Phase 3 Progress

- **Enrollment** of 162 patients **completed** in June 2026
- **No treatment discontinuations** to date
- **Data readout expected Q3 2027**

U.S. NDA Preparation Underway

- **Pre-NDA meeting** with FDA scheduled for H2 2026
- **Rolling submission** planned to **initiate in Q1 2027** and **complete in 2H 2027**
- Long-term 4-year OLE data support ongoing educational efforts

Pipeline Beyond Zorevunersen

- Dose escalation continuing following completion of sentinel cohort in the Phase 1 study of **STK-002 in patients with ADOA**
- Lead optimization underway to identify a clinical candidate for **SYNGAP1-related disorders**
- Appointment of CSO Thomas McCauley, PhD

$420M pro-forma cash position supports investment in the business, provides runway through to a potential U.S. launch of zorevunersen in early 2028

STOKE THERAPEUTICS

Phase 3 Progress and 4-Year OLE Data

Barry Ticho, M.D., Ph.D.
Chief Medical Officer

Phase 3 Enrollment of 162 Patients Completed in June 2026 Supports a Data Readout in Q3 2027

Patient Progression Through Key Study Milestones

n=162

~145 through Week 8
completion of 2x70mg loading doses or sham

~80 through Week 24
completion of 3 doses or sham

~60 through Week 28
primary endpoint

First patients approaching **Week 52**

No treatment discontinuations

- 162 patients enrolled (U.S., UK, Japan) in **10 months**
- Final patient randomized in **June 2026**
- **52 Week** treatment period after which all data will be analyzed
- **Data readout** anticipated in **Q3 2027**

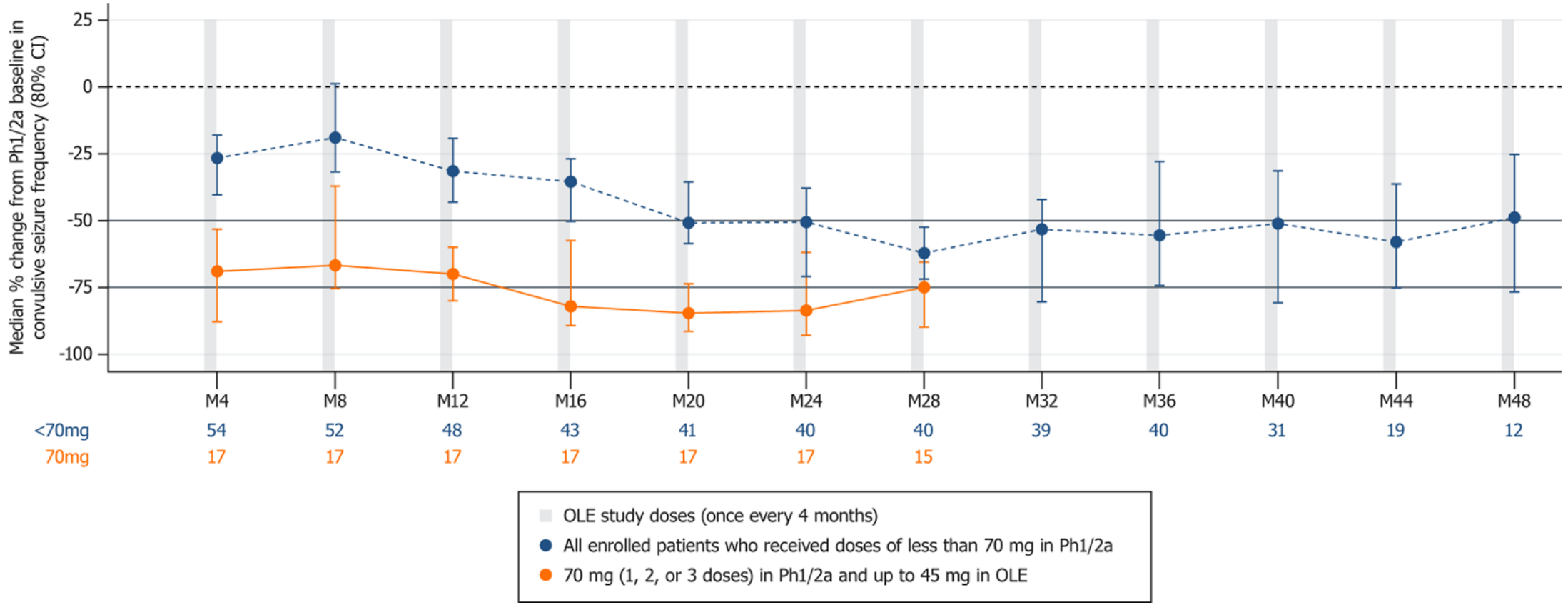
~30
Additional patients in Europe

- Completion of enrollment expected in August
- Data not planned for inclusion in U.S. NDA submission

Rolling U.S. NDA submission planned to initiate in Q1 2027 and complete in H2 2027

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

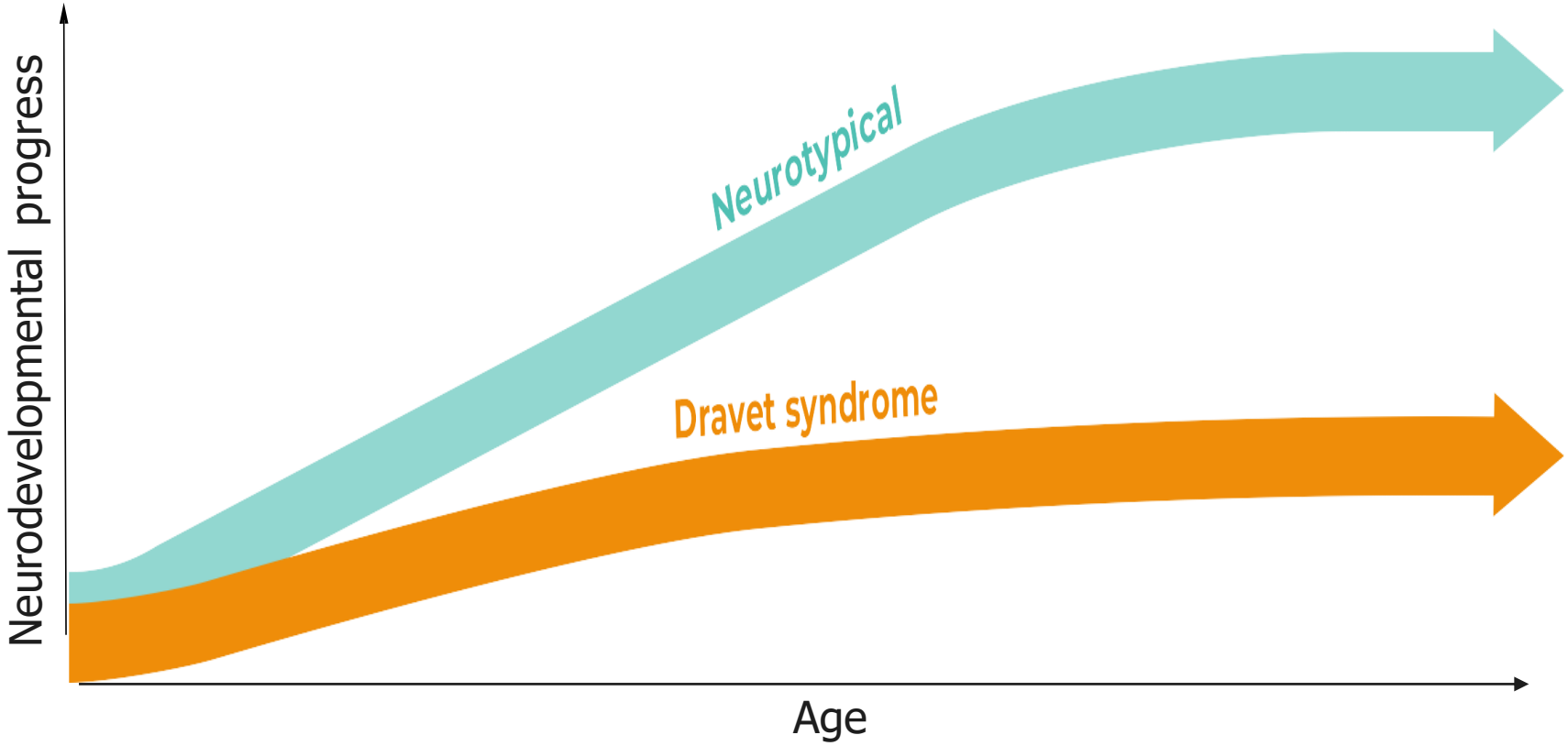
Data for all patients who continued treatment in the OLEs separated by dose received in the Ph1/2a studies



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.

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

Neurodevelopment plateaus at ~2 years of age for most children living with Dravet syndrome[1]



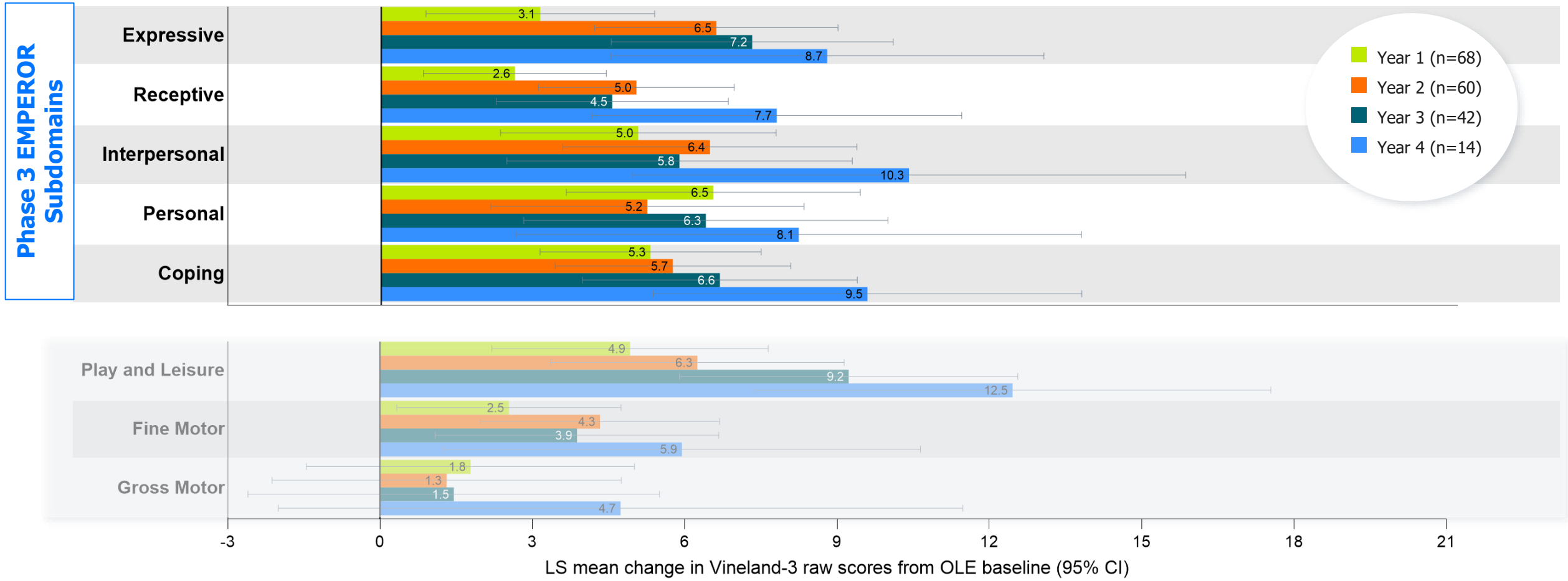
Comparison of developmental trajectory between neurotypical children and patients with Dravet syndrome

Graph provided for illustrative purposes only; progress not drawn to scale.

1 Sullivan 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

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



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. Statistical analyses are post-hoc and exploratory.

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**

>930 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.

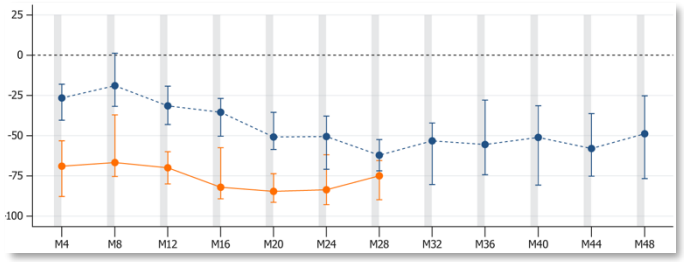
**≥1 CSF protein value >50 mg/dL.*

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.

STOKE THERAPEUTICS

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 Q3 2027 to complete **planned U.S. rolling NDA submission in H2 2027**

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 **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 approval[1]

Visibility & Credibility

- Publication of **zorevunersen data in NEJM**[2], the world's leading medical journal is enhancing awareness and understanding
- Publication supports education and access efforts
- Educational efforts to continue through 2026 and into 2027 with existing and new analyses of data from the Ph1/2 OLEs

Source [1]Stoke blinded HCP market research, Feb 2026, n=30; Stoke blinded payer research, Jan/Feb 2026, n=8. [2] Engl J Med 2026;394:969-982

Expanding the Pipeline

Barry Ticho, M.D., Ph.D.
Chief Medical Officer

Autosomal Dominant Optic Atrophy (ADOA): A Progressive Vision Disorder Caused by *OPA1* Haploinsufficiency

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

~13,000

people affected in the U.S., UK, EU-4, and Denmark

Sources: Kjer. Acta Ophthalmologica Scandinavica. 1996; Yu-Wai-Man P et al. Ophthalmology, 2010; Yu-Wai-Man P, Chinnery PF. Ophthalmology, 2013; Physician and Payer Interviews; 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 August 11, 2025, from https://www.adoaa.org/what-is-adoa

Phase 1 OSPREY Study of STK-002 in Patients with ADOA

Dosing complete in sentinel cohort
Dose escalation continuing and initial data anticipated in H1 2027

Phase 1 Study Design (UK and Europe)

1. Intravitreal injection in one eye
2. 48-week duration
3. Patients ages ≥18 to <55y (n=21)
4. Single ascending doses (0.1, 0.3, 0.5, & 0.7 mg/eye)

Primary Endpoint:
Safety, tolerability, and exposure of single ascending doses of STK-002

Secondary Endpoints:
Changes in visual function (LCVA), ocular structures, quality of life, and electroretinographic measures (e.g., ERG, PhNR) after single doses of STK-002

Exploratory Endpoints:
Mitochondrial function as measured by OcuMet Beacon FPF

Notes: LCVA = low contrast visual acuity, ERG= Electroretinogram, PhNR = ¥Photopic negative response, FPF=Flavoprotein fluorescence

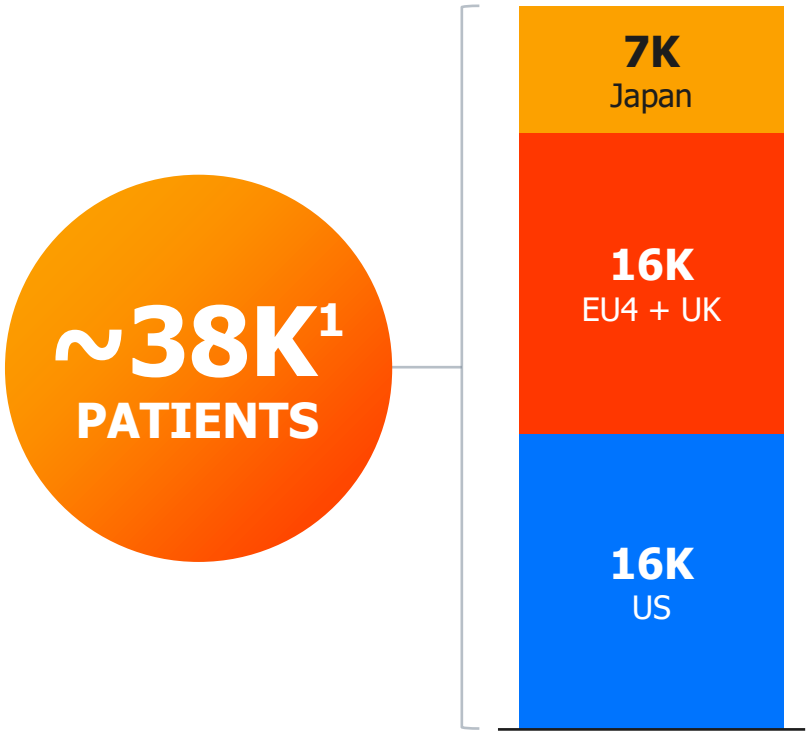
Preparing for a Potential U.S. Launch

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 study[2] 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.*

[1] Based 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.;

[2]Sullivan 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

STOKE THERAPEUTICS

Concentrated U.S. Market and Established Infrastructure Provide Foundation for Early Adoption

Treating Sites

~50% of identified U.S. patients are cared for by the top 50 sites

Existing Infrastructure

100% of top 50 sites have established intrathecal administration infrastructure

Zorevunersen Experience

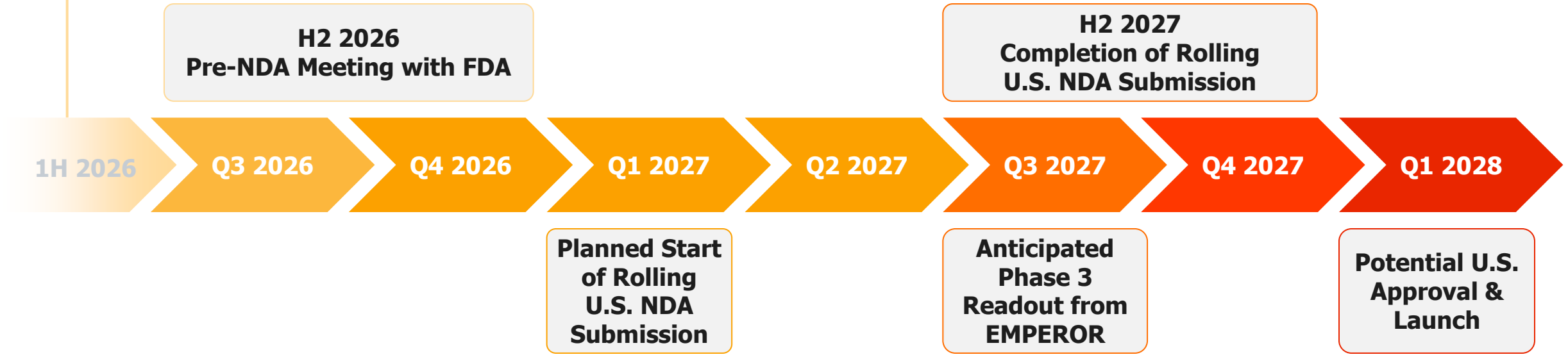
~50% of top 50 sites have participated in zorevunersen clinical studies

*"We've used Spinraza for years and **we've already established the infrastructure of recurrent intrathecal injections** for these patients. I can't imagine that I'm going to need a completely different infrastructure."*

Sources: Stoke primary research, July 2026; Komodo claims-based site model, Dec 2025; Field Medical insights. Quote from qualitative HCP interviews

Path to Potential U.S. Approval and Launch

- March: Phase 1/2 and 3-Year OLE Data Published in *The New England Journal of Medicine*
- May: 4-Year Phase 1/2 OLE Data Announced
- June: Completion of enrollment of 162 patients into EMPEROR (U.S., UK, JP)



Note: Timeline is not comprehensive and reflects the estimated timing of activities which are subject to change.

Financial Summary

Thomas Leggett
Chief Financial Officer

Second Quarter 2026 Financial Summary

$420 Million

Pro-Forma Cash, Cash Equivalents, & Marketable Securities

based on $354.3M as of June 30, 2026 and $65.7M from a subsequent ATM sale

Current financial position anticipated to fund operations through to potential U.S. commercialization of zorevunersen in early 2028

STOKE THERAPEUTICS

Q&A