# UNITED STATES
# SECURITIES AND EXCHANGE COMMISSION
**Washington, D.C. 20549**

## FORM 8-K

**CURRENT REPORT**
**Pursuant to Section 13 or 15(d)**
**of the Securities Exchange Act of 1934**

**Date of Report (Date of earliest event reported): May 7, 2026**

# PRAXIS PRECISION MEDICINES, INC.
**(Exact name of registrant as specified in its charter)**

| Delaware | 001-39620 | 47-5195942 |
|---|---|---|
| (State or other jurisdiction of incorporation) | (Commission File Number) | (I.R.S. Employer Identification No.) |

**Praxis Precision Medicines, Inc.**
**99 High Street, 30th Floor**
**Boston, Massachusetts 02110**
**(Address of principal executive offices, including zip code)**

**(617) 300-8460**
**(Registrant's telephone number, including area code)**

**Not Applicable**
**(Former Name or Former Address, if Changed Since Last Report)**

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

| Title of each class | Trade Symbol(s) | Name of each exchange on which registered |
|---|---|---|
| **Common Stock, $0.0001 par value per share** | **PRAX** | **The Nasdaq Global Select Market** |

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

**Item 2.02. Results of Operations and Financial Condition.**

On May 7, 2026, Praxis Precision Medicines, Inc. (the "Company") announced its financial results for the quarter ended March 31, 2026. A copy of the press release containing these announcements is furnished as Exhibit 99.1 to this Current Report on Form 8-K (the "Current Report").

**Item 7.01. Regulation FD Disclosure.**

On May 7, 2026, the Company updated its corporate presentation for use in meetings with investors, analysts and others. The presentation is available in the "Investors + Media" portion of the Company's website at investors.praxismedicines.com and a copy is furnished as Exhibit 99.2 to this Current Report.

The information in this Current Report under Items 2.02 and 7.01, including Exhibit 99.1 and Exhibit 99.2 attached hereto, is intended to be furnished and shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such filing.

**Item 9.01. Financial Statements and Exhibits.**

(d) Exhibits

| Exhibit No. | Description |
|---|---|
| 99.1 | Press Release, dated May 7, 2026 |
| 99.2 | Praxis Precision Medicines, Inc. May 2026 Corporate Presentation |
| 104 | Cover Page Interactive Data File (embedded within the inline XBRL document) |

**SIGNATURE**

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

PRAXIS PRECISION MEDICINES, INC.

Date: May 7, 2026

By: /s/ Marcio Souza
Marcio Souza
Chief Executive Officer

**Praxis Precision Medicines Provides Corporate Update and Reports First Quarter 2026 Financial Results**

*FDA accepted the new drug application (NDA) for ulixacaltamide in Essential Tremor with a PDUFA target action date of January 29, 2027, and the NDA for relutrigine, with priority review, in SCN2A and SCN8A developmental and epileptic encephalopathies (DEEs) with a PDUFA target action date of September 27, 2026*

*EMBRAVE Part A study results showed elsunersen treatment led to a 77% placebo-adjusted reduction in monthly seizures and demonstrated disease-modifying improvements in patients with early-seizure onset SCN2A-DEE*

*Topline results from the POWER1 study of vormatrigine in focal onset seizures expected in Q2 2026*

*Recruiting completed for relutrigine EMERALD study in broad DEEs, with topline results expected in Q4 2026*

*Cash and investments of approximately $1.4 billion as of March 31, 2026 maintains runway into 2028*

*Conference call today, May 7, 2026 at 8:30am*

**BOSTON, May 7, 2026 —** Praxis Precision Medicines, Inc. (NASDAQ: PRAX), a fully integrated, leading central nervous system (CNS) precision neuroscience biopharmaceutical company, today provided a corporate update and reported financial results for the first quarter of 2026.

“This quarter marks yet another inflection point for Praxis, with FDA acceptance of NDAs for both ulixacaltamide and relutrigine, positioning us for two U.S. launches within the next eight months as we accelerate our commercial roadmap to ensure readiness and market access upon approval. Our clinical pipeline continues to deliver, with EMBRAVE Part A data showing a 77% placebo-adjusted reduction in monthly seizures and disease-modifying improvements for elsunersen in early-onset SCN2A-DEE. Looking ahead, we expect topline results from the POWER1 study of vormatrigine in focal epilepsy this quarter, followed by the EMERALD readout in broad DEEs in the fourth quarter. Importantly, we remain well-capitalized to execute on this catalyst-rich period and deliver these therapies to patients,” said Marcio Souza, president and chief executive officer.

**Recent Highlights and Anticipated Milestones**

***Cerebrum™ Small Molecule Platform***

**Ulixacaltamide for Essential Tremor (ET):** ET is one of the most common movement disorders, affecting approximately seven million patients in the U.S. Ulixacaltamide was the first investigational therapy to demonstrate positive results in a Phase 3 program in ET and was granted Breakthrough Therapy Designation by the FDA in December 2025.

- The FDA has accepted Praxis’ NDA for ulixacaltamide for the treatment of ET and has set a target action date under the Prescription Drug User Fee Act (PDUFA) of January 29, 2027.
- Commercial preparations and pre-launch activities continue to accelerate:
  - ESSENTIAL *to me* disease education campaign for healthcare providers was launched in April 2026 to raise awareness of Essential Tremor.
  - Commercial organization leadership hired, with build of cross-functional commercial organization and infrastructure on-track.
  - Distribution network is established, with commercial inventory build in-progress ahead of launch.
- At the recent American Academy of Neurology (AAN) Annual Meeting, Praxis shared several oral presentations and posters on ulixacaltamide. The oral plenary session on Phase 3 results from Essential3 was recognized as an Abstract of Distinction in Movement Disorders by the AAN.

**Relutrigine for DEEs:** Relutrigine is a sodium channel modulator designed to precisely target the hyperexcitable state of sodium-channels, with therapeutic potential across developmental epilepsies. Relutrigine has been granted Breakthrough Therapy Designation and Orphan Drug Designation by the FDA.

- The FDA has accepted with priority review the relutrigine NDA for the treatment of SCN2A and SCN8A DEEs, with a PDUFA target action date of September 27, 2026. If approved, relutrigine will be the first therapy for SCN2A/8A DEE and be eligible for a Pediatric Review Voucher.
- Preparations for the commercial launch of relutrigine are progressing well, including continued hiring within commercial and medical teams, building sufficient inventory, establishing a comprehensive patient support program and engaging with payers to ensure timely market access upon potential approval.
- Recruitment for the EMERALD study in broad DEEs is complete, with topline results expected in the fourth quarter of 2026. Assuming successful initial NDA approval of relutrigine, the EMERALD study, if positive, would serve as the basis for a supplemental NDA submission in 2027.

**Vormatrigine for Focal Onset Seizures (FOS) and Generalized Epilepsy:** An estimated 3.5 million people in the U.S. suffer from common epilepsies. Sodium channel therapy is the cornerstone of treatment for patients with epilepsy, yet currently approved drugs have significant safety and efficacy limitations. Vormatrigine is the most potent sodium-channel modulator ever developed for epilepsy and is designed to precisely target the hyperexcitable state of sodium-channels in adult common epilepsies.

- The POWER1 Phase 3 study for FOS is on track for topline results in the second quarter of 2026.
- POWER2, the second Phase 3 study for vormatrigine in FOS, continues to progress towards completion in the second half of 2026 with topline results anticipated in 2027.
- The POWER3 study to evaluate vormatrigine as a monotherapy remains on track to commence in the first half of 2026.

***Solidus™ Antisense Oligonucleotide (ASO) Platform***

- **Elsunersen for early-seizure-onset SCN2A DEE:** SCN2A early-onset DEE is a rare, genetic epilepsy characterized by early-onset seizures and severe impact on development.
  - Topline results from the EMBRAVE Part A Phase 1/2 study evaluating SCN2A early onset seizure patients were announced in April 2026 and presented at AAN:
    - Treatment with elsunersen led to a significant 77% placebo-adjusted seizure reduction from baseline (p=0.015).
    - 71% of patients treated with elsunersen achieved >50% seizure reduction by period 6, with results sustained during the open label extension for up to one year.
    - 57% of patients treated with elsunersen had at least a 28-day period of seizure freedom.
    - 100% of patients treated with elsunersen experienced improvements in sleep, motor function, muscle tone, attention or neuropsychomotor development compared to no observed improvements in placebo group.
    - Elsunersen was well-tolerated, with no drug-related SAEs, no discontinuations and no neuroinflammation signals at doses up to 8 mg.
    - Clinical updates from the elsunersen Emergency Use Program were also presented at AAN, highlighting durable seizure reduction and meaningful quality-of-life improvements across six patients treated globally, with more than 100 doses administered to date.
  - Enrollment is progressing in the EMBRAVE3 registrational trial, with topline results expected in 2027.

- Praxis remains on track to nominate a development candidate for each of its three early stage ASO therapeutic initiatives in the first half of 2026:
  - PRAX-080 is focused on targeting PCDH19 mosaic expression disorder.
  - PRAX-090 is designed to address SYNGAP1 loss-of-function (LoF) mutations, a leading cause of severe intellectual disability and epilepsy in DEEs.
  - PRAX-100 targets SCN2A LoF mutations, the predominant genetic link to de novo autism spectrum disorders.

**First Quarter 2026 Financial Results:**

As of March 31, 2026, Praxis had $1.4 billion in cash, cash equivalents and marketable securities, compared to $926.1 million in cash, cash equivalents and marketable securities as of December 31, 2025. This increase of $473.9 million was primarily attributable to net proceeds from Praxis' January 2026 follow-on public offering and interest income on

marketable securities, partially offset by cash used in operations. The Company's cash, cash equivalents and marketable securities as of March 31, 2026 are expected to fund operations into 2028.

Research and development expenses were $78.0 million for the first quarter of 2026, compared to $60.8 million for the first quarter of 2025. The increase in research and development expenses of $17.2 million was primarily attributable to $9.2 million in increased expenses related to the Company's Cerebrum™ platform, $3.8 million in increased personnel-related expenses and $3.0 million in increased expenses related to the Company's Solidus™ platform.

General and administrative expenses were $27.9 million for the first quarter of 2026, compared to $13.9 million for the first quarter of 2025. The increase in general and administrative expenses of $14.0 million was primarily attributable to $9.8 million in increased personnel-related expenses and $3.5 million in increased professional expenses.

Praxis incurred a net loss of $92.6 million for the first quarter of 2026, including $17.1 million of stock-based compensation expense, compared to $69.3 million for the first quarter of 2025, including $8.8 million of stock-based compensation expense.

As of March 31, 2026, Praxis had 27.9 million shares of common stock outstanding.

**Conference Call**
Praxis will discuss first quarter 2026 financial results and business highlights on a conference call taking place today, May 7 at 8:30 am ET, which can be accessed by visiting this registration link. The live audio webcast will also be available through the Events & Presentations page of the Investors + Media section of the Company's website.

**About Ulixacaltamide**
Ulixacaltamide is a differentiated and highly selective small molecule inhibitor of T-type calcium channels designed to block abnormal neuronal burst firing in the Cerebello-Thalamo-Cortical (CTC) circuit correlated with tremor activity. Ulixacaltamide has received Breakthrough Therapy Designation from the FDA and is the most advanced program within Praxis' Cerebrum™ small molecule platform.

**About Vormatrigine**
Vormatrigine is a next-generation, functionally selective small molecule targeting the hyperexcitable state of sodium-channels in the brain that is currently being developed as a once daily, oral treatment for adult focal onset seizures and generalized epilepsy. Preclinical data demonstrates vormatrigine is differentiated from standard of care, with the potential to be best-in-class for focal epilepsy. In vitro, vormatrigine has demonstrated superior selectivity for disease-state NaV channel hyperexcitability. In vivo studies of vormatrigine have demonstrated unprecedented potency in the maximal electroshock seizure (MES) model, a highly predictive translational model for efficacy in focal epilepsy. Data from patients in the RADIANT study demonstrated a robust seizure reduction and generally safe and well tolerated profile. To learn more about the POWER1 and POWER2 studies, please visit POWER studies.

**About Relutrigine**
Relutrigine is a first-in-class small molecule in development for the treatment of developmental and epileptic encephalopathies (DEEs) as a preferential inhibitor of persistent sodium current, shown to be a key driver of seizure symptoms in severe DEEs. Relutrigine's mechanism of precision sodium channel (NaV) modulation is consistent with superior selectivity for disease-state NaV channel hyperexcitability. In vivo studies of relutrigine have demonstrated dose-dependent inhibition of seizures up to complete control of seizure activity in SCN2A, SCN8A and other DEE mouse models. Relutrigine has been generally well-tolerated in three Phase 1 studies and has demonstrated biomarker changes indicative of NaV channel modulation. Data from cohort 1 of the Phase 2 EMBOLD study demonstrated a well-tolerated, robust, short- and long-term improvement in motor seizures in a heavily pre-treated population, alongside maintained seizure freedom in some patients with SCN2A- and SCN8A-DEE. Relutrigine has received Orphan Drug Designation (ODD) and Rare Pediatric Disease Designation from the FDA for the treatment of SCN2A-DEE, SCN8A-DEE and Dravet syndrome; as well as Breakthrough Therapy Designation (BTD), and ODD from the European Medicines Agency for the treatment of SCN2A-DEE and SCN8A-DEE. To learn more about the EMERALD study, please visit Emerald | Resilience Studies.

**About Elsunersen**
Elsunersen is an antisense oligonucleotide (ASO) designed to selectively decrease SCN2A gene expression, directly targeting the underlying cause of early-seizure-onset SCN2A-DEE to treat seizures and other symptoms in patients with

gain-of-function SCN2A mutations. In vitro studies of elsunersen have demonstrated reduction in both SCN2A gene expression and protein levels. In vivo, elsunersen has demonstrated significant, dose-dependent reduction in seizures, improvement in behavioral and locomotor activity and increased survival in SCN2A mouse models, with potential to be the first disease-modifying treatment for SCN2A-DEE. Elsunersen has received ODD and RPDD from the FDA, and ODD and PRIME designations from the European Medicines Agency for the treatment of SCN2A-DEE. The elsunersen program is ongoing under a collaboration with Ionis Pharmaceuticals, Inc., and RogCon, Inc. To learn more about the EMBRAVE3 study, please visit Embrave | Resilience Studies.

**About Praxis**
Praxis Precision Medicines is a fully integrated, leading central nervous system (CNS) precision neuroscience biopharmaceutical company, translating insights from genetic epilepsies into the development of therapies for CNS disorders characterized by neuronal excitation-inhibition imbalance. Praxis is applying genetic insights to the discovery and development of therapies for rare and more prevalent neurological disorders through our proprietary small molecule platform, Cerebrum™, and antisense oligonucleotide (ASO) platform, Solidus™, using our understanding of shared biological targets and circuits in the brain. Praxis has established a diversified, multimodal CNS portfolio including multiple programs across movement disorders and epilepsy, with four late-stage product candidates. For more information, please visit www.praxismedicines.com and follow us on Facebook, LinkedIn and X/Twitter.

**Forward-Looking Statements**
This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995 and other federal securities laws, including express or implied statements regarding Praxis' future expectations, plans and prospects, including, without limitation, statements regarding the anticipated timing of clinical trials, the development of Praxis' product candidates and plans to initiate new clinical programs, the anticipated timing of regulatory submissions and interactions, potential market opportunity and commercial potential of Praxis' product candidates and our projected cash runway, as well as other statements containing the words "anticipate," "believe," "continue," "could," "endeavor," "estimate," "expect," "anticipate," "intend," "may," "might," "plan," "potential," "predict," "project," "seek," "should," "target," "will" or "would" and similar expressions that constitute forward-looking statements under the Private Securities Litigation Reform Act of 1995.

The express or implied forward-looking statements included in this press release are only predictions and are subject to a number of risks, uncertainties and assumptions, including, without limitation: uncertainties inherent in clinical trials; the expected timing of clinical trials, data readouts and the results thereof, and submissions for regulatory approval or review by governmental authorities; regulatory approvals to conduct trials; and other risks concerning Praxis' programs and operations as described in its Annual Report on Form 10-K for the year ended December 31, 2025 and other filings made with the Securities and Exchange Commission. Although Praxis' forward-looking statements reflect the good faith judgment of its management, these statements are based only on information and factors currently known by Praxis. As a result, you are cautioned not to rely on these forward-looking statements. Any forward-looking statement made in this press release speaks only as of the date on which it is made. Praxis undertakes no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future developments or otherwise.

**Investor Contact:**
Praxis Precision Medicines
investors@praxismedicines.com
857-702-9452

**Media Contact:**
Dan Ferry
LifeSci Advisors
Daniel@lifesciadvisors.com
617-430-7576

**PRAXIS PRECISION MEDICINES, INC.**
**CONDENSED CONSOLIDATED BALANCE SHEETS**
**(Amounts in thousands)**
**(Unaudited)**

| | March 31, 2026 | December 31, 2025 |
|---|---|---|
| **Assets** | | |
| Cash and cash equivalents | $ 536,333 | $ 357,329 |
| Marketable securities | 911,470 | 568,759 |
| Prepaid expenses and other current assets | 10,909 | 11,580 |
| Property and equipment, net | 170 | 147 |
| Operating lease right-of-use assets | 1,320 | 92 |
| **Total assets** | $ 1,460,202 | $ 937,907 |
| **Liabilities and stockholders' equity** | | |
| Accounts payable | $ 31,158 | $ 24,628 |
| Accrued expenses | 17,648 | 35,033 |
| Operating lease liabilities | 1,435 | 110 |
| Common stock | 15 | 15 |
| Additional paid-in capital | 2,644,109 | 2,017,566 |
| Accumulated deficit | (1,232,569) | (1,140,008) |
| Accumulated other comprehensive (loss) gain | (1,594) | 563 |
| **Total liabilities and stockholders' equity** | $ 1,460,202 | $ 937,907 |

**PRAXIS PRECISION MEDICINES, INC.**
**CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS**
**(Amounts in thousands, except share and per share amounts)**
**(Unaudited)**

| | Three Months Ended March 31, | |
|---|---|---|
| | **2026** | **2025** |
| Operating expenses: | | |
| Research and development | $ 77,987 | $ 60,806 |
| General and administrative | 27,873 | 13,922 |
| Total operating expenses | 105,860 | 74,728 |
| Loss from operations | (105,860) | (74,728) |
| Other income: | | |
| Other income, net | 13,299 | 5,432 |
| Total other income | 13,299 | 5,432 |
| Net loss | $ (92,561) | $ (69,296) |
| Net loss per share attributable to common stockholders, basic and diluted | $ (3.20) | $ (3.29) |
| Weighted average common shares outstanding, basic and diluted | 28,883,596 | 21,055,834 |


PRAXIS
DARE FOR MORE®
Corporate Overview
May 2026

# Forward Looking Statements

This presentation may contain "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to our business, operations, and financial conditions, including but not limited to express or implied statements regarding the current beliefs, expectations and assumptions regarding the future of our business, future plans and strategies, including statements regarding the estimated market for our product candidates, if approved, our development plans, our preclinical and clinical results and other future conditions, including our cash runway, and the safety, efficacy, and regulatory and clinical design or progress, potential regulatory submissions, approvals and timing thereof of any of our product candidates. Any forward-looking statements in this presentation are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this presentation, including, without limitation, risks relating to: (i) the success and timing of our ongoing clinical trials, (ii) the success and timing of our product development activities and initiating clinical trials, (iii) the success and timing of our collaboration partners' product development activities, (iv) the timing of and our ability to obtain and maintain regulatory approval of any of our product candidates, (v) our plans to research, discover and develop additional product candidates, (vi) our ability to enter into collaborations for the development of new product candidates, (vii) our ability to establish manufacturing capabilities, and our collaboration partners' abilities to manufacture our product candidates and scale production, (viii) our ability to meet any specific milestones set forth herein, and (ix) the potential addressable market sizes for product candidates. New risks and uncertainties may emerge from time to time, and it is not possible to predict all risks and uncertainties. Except as required by applicable law, we do not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise. Although we believe the expectations reflected in such forward-looking statements are reasonable, we can give no assurance that such expectations will prove to be correct. Accordingly, readers are cautioned not to place undue reliance on these forward-looking statements.

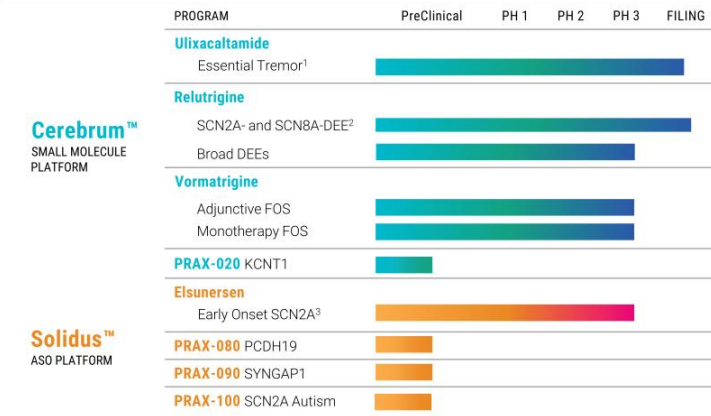
For further information regarding the risks, uncertainties and other factors that may cause differences between our expectations and actual results, you should review the "Risk Factors" section of our Annual Report on Form 10-K for the year ended December 31, 2025 and other filings made with the Securities and Exchange Commission .

Certain information contained in this presentation relates to or is based on studies, publications, surveys and other data obtained from third-party sources and our own internal estimates and research. While we believe these third-party sources to be reliable as of the date of this presentation, we have not independently verified, and make no representation as to the adequacy, fairness, accuracy or completeness of, any information obtained from third-party sources. In addition, all of the market data included in this presentation involves a number of assumptions and limitations, and there can be no guarantee as to the accuracy or reliability of such assumptions. Finally, while we believe our own internal research is reliable, such research has not been verified by any independent source.


PRAXIS' MISSION
The needs of patients with CNS disorders are devastatingly urgent. Our mission is to help patients by delivering life-altering treatments faster and more effectively than has ever been done before — and to do it again and again.
PRAXIS
3

# Praxis: Broad and Deep CNS Pipeline with Near-term Catalysts

## PIPELINE SNAPSHOT




## UPCOMING MILESTONES

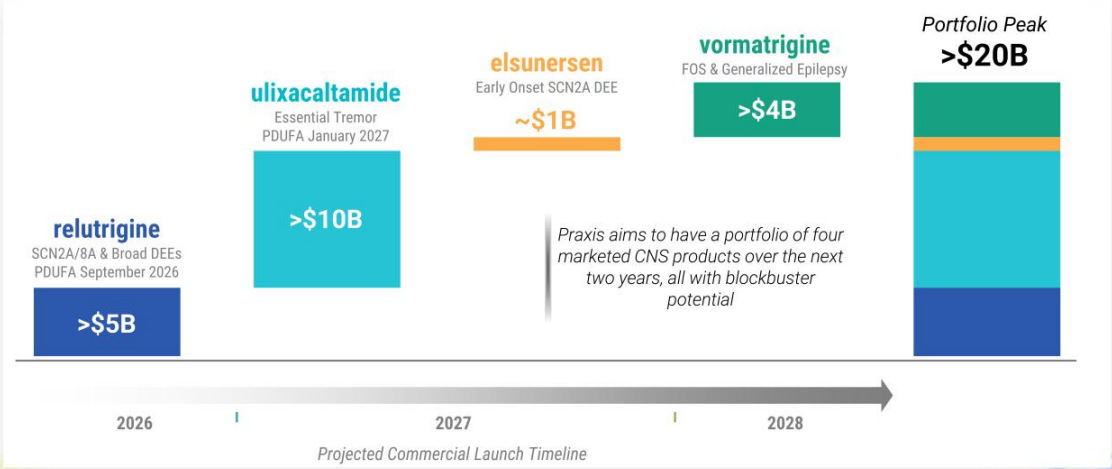
- PDUFA target action date of January 29, 2027
- PDUFA target action date of September 27, 2026, under Priority Review
- Topline EMERALD data in 4Q 2026
- Topline POWER1 data 2Q 2026
- Initiate POWER3 in 1H 2026
- EMBRAVE3 completion anticipated in 2027
- Declare clinical candidate in 1H 2026
- Declare clinical candidate in 1H 2026
- Declare clinical candidate in 1H 2026

1. Ulixacaltamide has received Breakthrough Therapy Designation (BTD)
2. Relutrigine has received BTD, Orphan Drug Designation (ODD) and Rare Pediatric Disease (RPD) designation from the FDA, and ODD from the European Medicines Agency (EMA) for the treatment of SCN2A and SCN8A-DEE and RPD designation for Dravet Syndrome
3. Elsunersen has received ODD and RPD designation from the FDA, and ODD and Priority Medicines (PRIME) designations from the EMA for the treatment of early SCN2A DEE

DEE: developmental & epileptic encephalopathy, FOS: focal onset seizures

# CNS Portfolio with >$20B in Peak Sales Potential

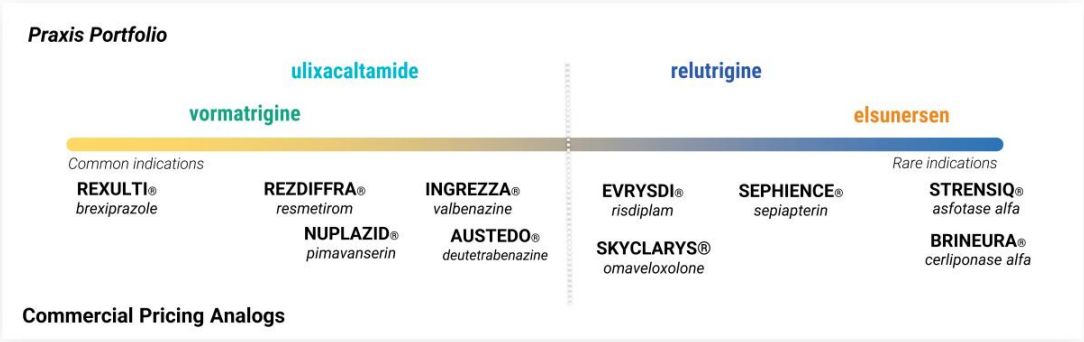
***Four late-stage assets. Two pending PDUFA dates. Cash runway into 2028***

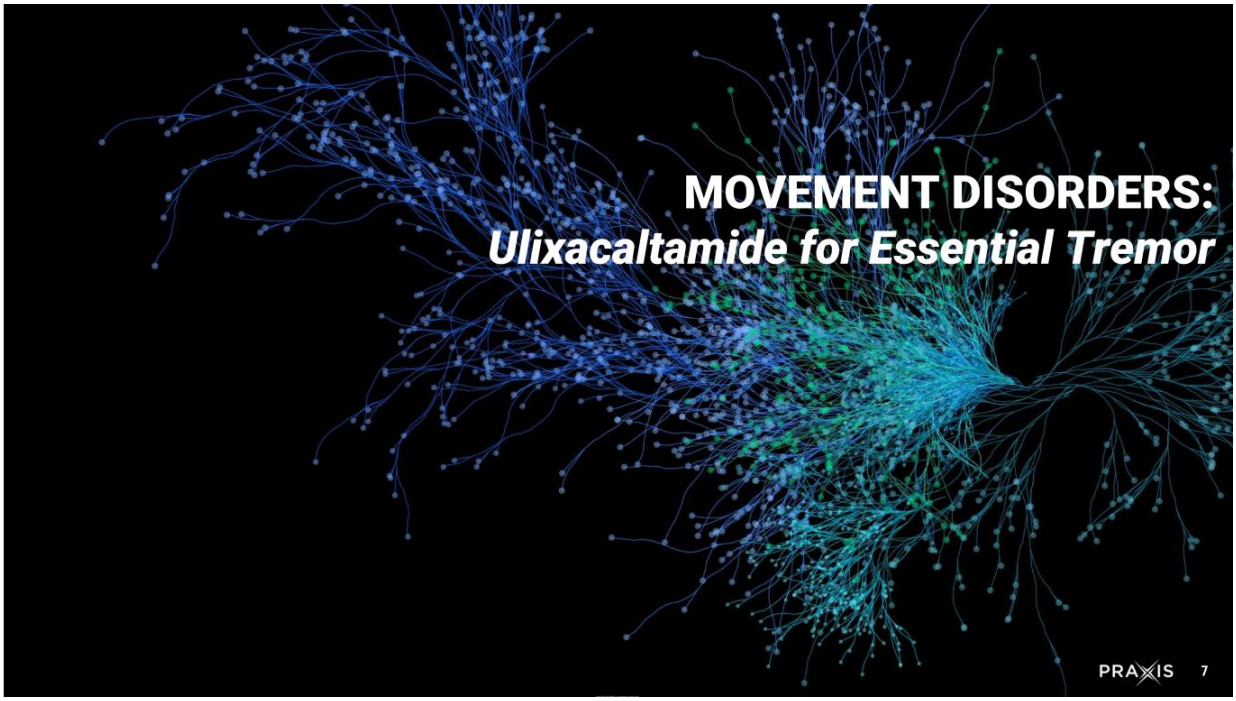


# Pricing for Recent Approvals Reflects Significant Value to Patients

## *Praxis portfolio poised for similar impact*

- High unmet-need indications with few, if any, effective treatment modalities
- Significant price potential within current market analogs



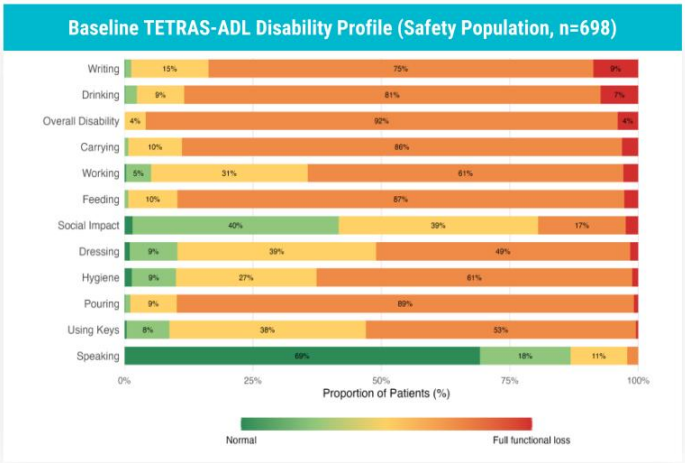

MOVEMENT DISORDERS:
Ulixacaltamide for Essential Tremor
PRAXIS
7

# Essential Tremor: A Major Unmet Need

**No ET-specific FDA-approved therapies**

- An estimated **7 million people** in the U.S. live with ET
- Major functional impacts affecting writing, eating, drinking and social activities
- High psychosocial burden (frustration, anxiety, embarrassment, isolation)
- Significant proportion receive no or inadequate treatment

# Patients Participating in ESSENTIAL3 had Significant Impact to Daily Activities at Baseline



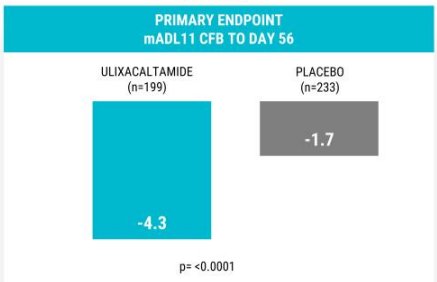

- ESSENTIAL3 included a broad population with high functional disability - average baseline TETRAS-ADL score of ~31
- ESSENTIAL3 patients averaged 30 years since ET diagnosis, with 94% reporting worsening symptoms over the past three years

Farmer et al. AAN 2026 Plenary Presentation
TETRAS-ADL: The Essential Tremor Rating Assessment Scale – Activities of Daily Living subscale

# ESSENTIAL3: Two Positive Phase 3 Studies Supporting Breakthrough Therapy Designation, PDUFA in January 2027

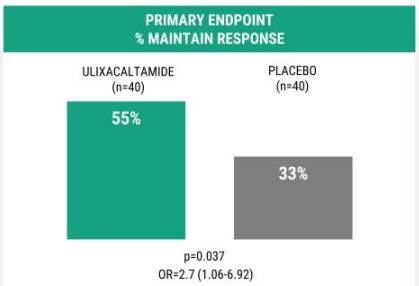
***The first successful Phase 3 program for a drug in Essential Tremor***

**Study 1: 12-week Parallel-group Design (n=432)**




- Effect as early as 2 weeks, maintained for 12-weeks
- Benefit for patients on background propranolol and other ET medications, with or without intention tremor and with or without family history of ET

**Study 2: Blinded Stable Responder, Randomized Withdrawal Design (n=80)**



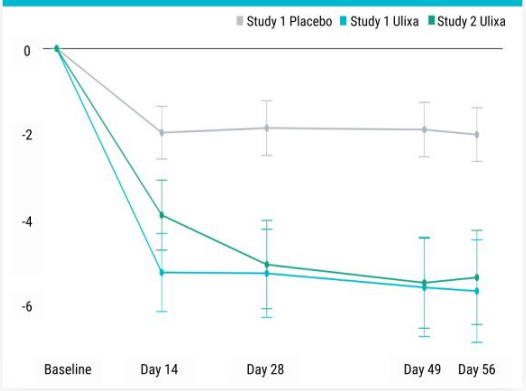

- Demonstrates maintenance of benefit
- Validates durability and robustness of response
- Reinforces functional benefit observed in Study 1

- Once-daily ulixacaltamide was generally well tolerated across studies, with no drug-related SAEs
- Most TEAEs occurred during titration, were mild to moderate and resolved
- CNS AEs occurred early in treatment and resolved quickly

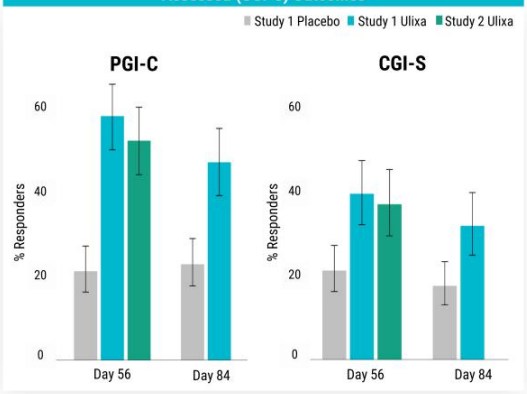
Modified intent-to-treat population, defined as all randomized participants who received ≥1 dose of study drug and had ≥1 post-baseline efficacy assessment
CFB: change from baseline, mADL11: modified ADL 11-item score, OR: odds ratio, SAE: serious adverse event, TEAE: treatment emergent adverse event
Shtilbans et al. AAN 2026 Poster Presentation; Farmer et al. AAN 2026 Plenary Presentation

# Rapid, Durable, and Consistent Effects Observed in Both Study 1 and Study 2 Across Multiple Endpoints

**TETRAS-ADL Change from Baseline***

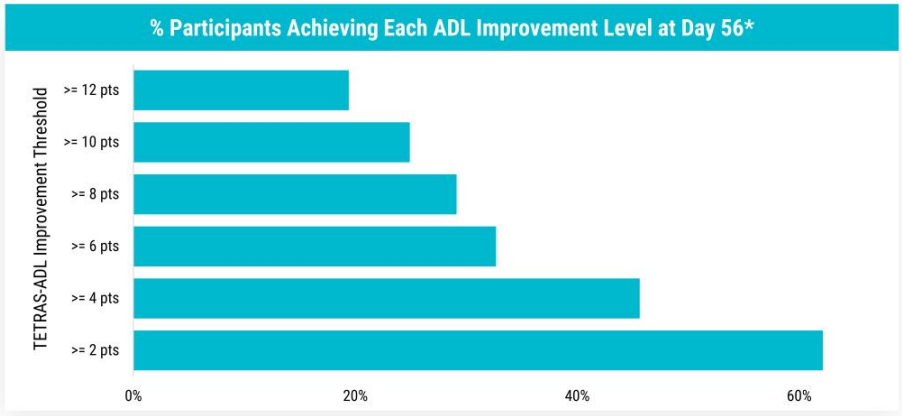



**% Responders across Patient-Reported (PGI-C) and Clinician-Assessed (CGI-S) Outcomes**




Intention-to-treat population
*LS Means; (+/- 95% CI)
Shtilbans et al. AAN 2026 Poster Presentation
PGI-C: Patient Global Impression of Change; CGI-S: Clinical Global Impression of Severity

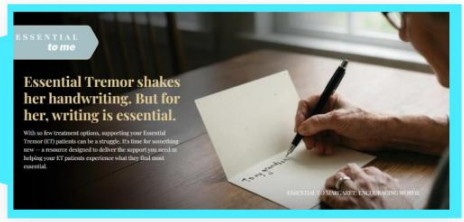
# Benefit Maintained Across Increasingly Stringent Response Thresholds in Studies 1 and 2




*Treatment difference: p-value < 0.01 at all responder thresholds (logistic regression)
Intention-to-treat population for the combined Study 1 and 2 populations for ulixacaltamide patients
Shtilbans et al. AAN 2026 Poster Presentation

# Building Commercial Capabilities for Unprecedented Ulixacaltamide Launch in Early 2027

- Building the team: Commercial leadership in place, Field force build-out on-track for launch
- Engaging ET physicians with "ESSENTIAL *to me*" disease awareness campaign launched at the American Academy of Neurology (ANN) conference
- Building medical community awareness about the profile of ulixacaltamide as a potential therapy for their ET patients
- Establishing a distribution network, engaging in market access preparation and building inventory



# Physicians Validate Ulixacaltamide's Profile Across Key Dimensions

***Findings from HCP Observational Study Conducted in March 2026***

*surveyed* **>2,300** US Physicians *representing* **>43,000** ET Patients*

### Meaningfulness of endpoint

- Physicians view ADL improvement as highly meaningful in ET, supporting mADL11 as a clinically relevant primary endpoint

### Compelling efficacy in ESSENTIAL3

- mADL11 results land strongly with physicians as a clear functional benefit
- Rapid onset and sustained response are seen as highly impactful

### Breadth of benefit

- Secondary endpoints show consistency across clinician and patient reported measures
- Consistent efficacy across patient subgroups is viewed as supportive of broad, profile-agnostic use in ET

### Favorable tolerability

- Tolerability and safety profile seen as favorable

**Results reinforce peak potential of >$10B in the US for ulixacaltamide**

Source: HCP observational quant study, March 2026. Findings reflect physician perceptions of investigational data; not promotional
* Confirmed with active claims, linked to MD NPI: Medical Doctor National Provider Identifier

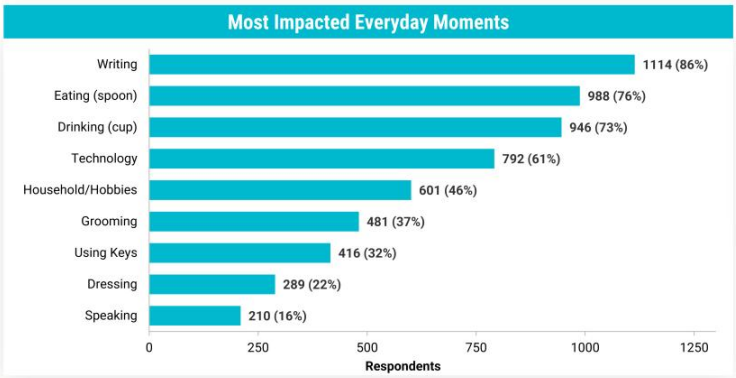
# Matching the Needs of Patients with Ulixacaltamide

## Patient Survey Overview – *Everyday Moments Most Impacted by Essential Tremor*

**1,294**
Total Patient Responses

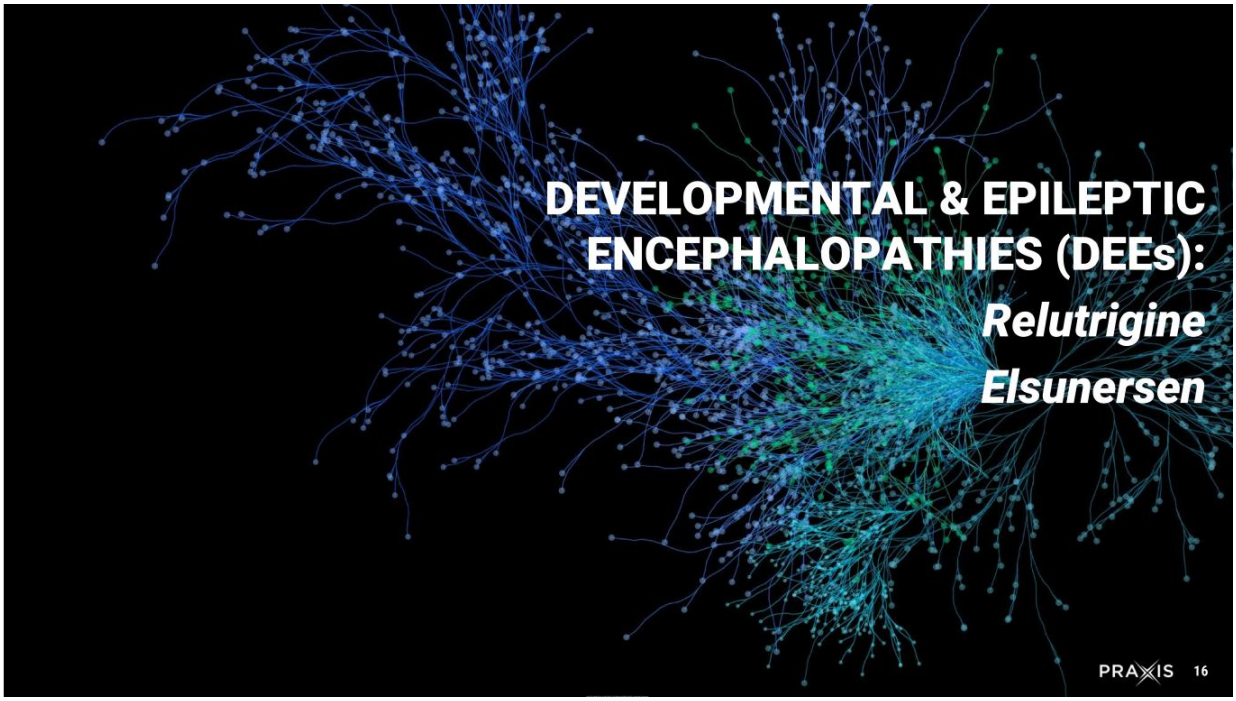
Mean Daily Functional Impact: **5**
1 (No impact) — 7 (Extreme impact)

**65%**
Had seen a Physician in the last 12 Months




Impacted activities match benefits observed in the ESSENTIAL3 program

Source: Praxis data on file, ET patient observational study, March/April 2026


DEVELOPMENTAL & EPILEPTIC ENCEPHALOPATHIES (DEEs):
Relutrigine
Elsunersen
PRAXIS
16

# Relutrigine: Potential for Class Leading Efficacy and Tolerability

**Relutrigine**

**Small molecule functional state modulator**

**No titration required**

**Once daily dosing**

**Liquid formulation - oral or G/J tube administration**

**Precision Mechanism:**

Superior selectivity for hyperactive $Na_V$ channels, a known driver of seizure activity across DEEs

**Clinical Profile:**

- In EMBOLD study, demonstrated robust seizure reduction and unprecedented seizure-free periods over 28-day intervals
- Generally well-tolerated with mostly mild to moderate AEs, no drug-related SAEs and no dose reductions required

**Regulatory Designations:**

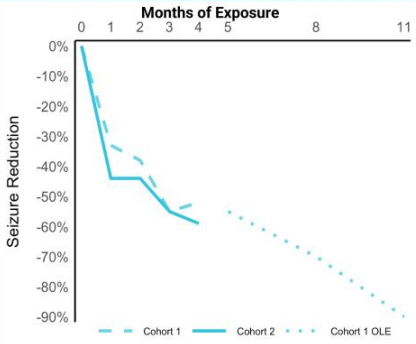
- FDA: Orphan Drug, Rare Pediatric Disease Designations for SCN2A DEE, SCN8A DEE, and Dravet syndrome, plus Breakthrough Therapy
- EMA: Orphan Drug Designations for SCN2A DEE and SCN8A DEE
- NDA accepted, with September 27, 2026 PDUFA target action date with priority review

AE: adverse event, DEE: developmental & epileptic encephalopathy, $Na_V$: voltage-gated sodium channel, SAE: serious adverse event

# EMBOLD Study: Disease-Modifying Results in SCN2A/8A DEEs

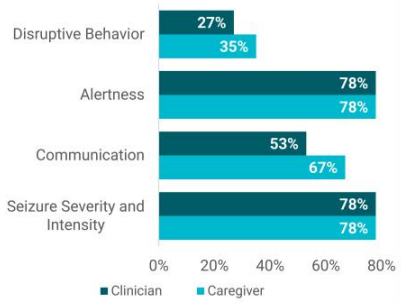
***Study stopped early at interim analysis; NDA accepted with PDUFA September 27, 2026***

**SEIZURE REDUCTION OVER TIME ON RELUTRIGINE COHORTS 1 and 2, COHORT 1 OLE**




**MARKED IMPROVEMENT IN DISEASE MODIFYING DOMAINS**

**PROPORTION OF PATIENTS IMPROVING BY DOMAIN**




- >80% of patients were on stable doses of sodium channel blockers at baseline
- AEs were mostly mild to moderate
- No drug-related SAEs
- No dose reduction of relutrigine required

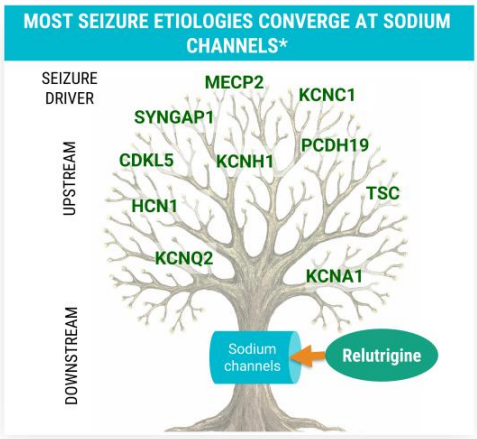
EMBOLD

OLE: open label extension
Kamireddy et al AES 2025

# Relutrigine Sodium Channel MOA Targets Phenotypic DEEs with Applicability Beyond SCN2A/8A EMBOLD Population

***Current US DEE market is over 200,000 patients and growing as population ages***

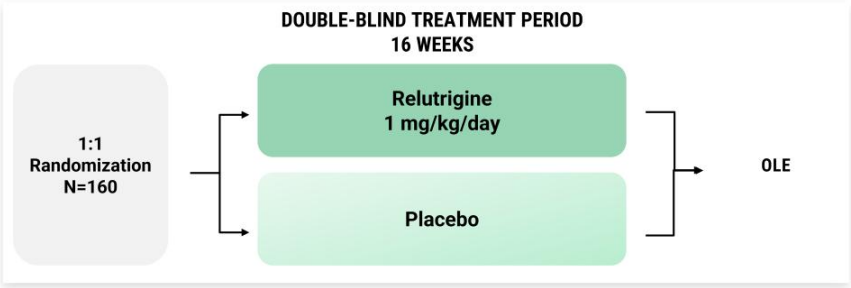
- Seizure-activity in DEEs, independent of etiology, requires participation of sodium channels
- Relutrigine's mechanism of action targets hyperactive $Na_V$ channels addressing the neuronal hyperexcitability driving seizures
- By targeting a common pathway implicated in DEE symptomology, relutrigine has the potential to be applicable across a broad range of DEEs




DEE: developmental & epileptic encephalopathy, TSC: tuberous sclerosis complex
*Illustrative etiologies, not limited by examples shown

# EMERALD Study Targets Phenotypic DEEs, Regardless of Etiology

***Topline readout expected Q4 2026***




**Primary Endpoint:**

Change from baseline in monthly motor seizure frequency

**Key Inclusion Criteria**

- Ages ≥2 and ≤65 years
- Has a documented diagnosis of a developmental and epileptic encephalopathy in childhood
- Has 4 or more countable motor seizures during the 28-day observation period
- Taking no more than 2 sodium channel blockers; no restriction on # of other antiseizure medications for inclusion criteria

**Treatment**

- Relutrigine or matching placebo 1mg/kg/day. At day 35, the dose may be escalated to 1.5 mg/kg/day

ClinicalTrials.gov Identifier: NCT07010471
Kamireddy et al IEC 2025

# Elsunersen: First-in-Class ASO for Early Onset SCN2A DEE

**ELSUNERSEN**

**Antisense oligonucleotide (ASO)**

**Intrathecal administration**

**Once every 4 weeks**

**Designed for selective SCN2A mRNA reduction**

**Mechanistic Precision:**

- Selective targeting of SCN2A gain-of-function mutations, a key driver of early onset, severe seizure activity
- ASO-mediated degradation of SCN2A mRNA reduces $Na_V1.2$ hyperactivity, normalizing neuronal excitability

**Clinical Profile**:

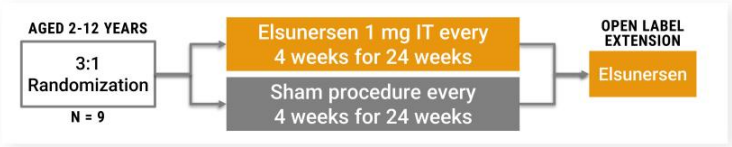
- Significant reduction in seizures achieved in patients with early onset SCN2A
- No adverse events were considered treatment-emergent or serious

**Regulatory Designations**:

- FDA: ODD and Rare Pediatric Disease designation
- EMA: ODD and PRIME designation

DEE=developmental & epileptic encephalopathy,, PRIME= Priority Medicines

# EMBRAVE Part A Topline Results Show Marked Seizure Reduction, with Disease-Modifying Benefit




Starting dose of 1 mg with optional dose escalation up to 8 mg based on individual tolerability at each dose

**77%** PRIMARY ENDPOINT
Placebo-adjusted seizure reduction from baseline
p=0.015

**71%**
Achieved >50% seizure reduction by period 6, with sustained benefit in OLE up to 1 year

**57%**
Had at least one 28-day period of seizure freedom

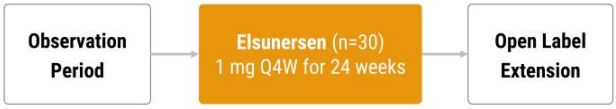
**100%**
All elsunersen-treated patients showed broad functional improvements including sleep, motor function, muscle tone and attention

- Safety findings consistent with EMBRAVE Part 1
- No drug-related SAEs
- No discontinuations
- No neuroinflammation signals at doses up to 8 mg
- Most TEAEs mild to moderate

ClinicalTrials.gov Identifier: NCT05737784
Praxis data on file

# The Pivotal EMBRAVE3 Trial is Currently Enrolling

**EMBRAVE3 - STUDY DESIGN**




**Key Inclusion Criteria**

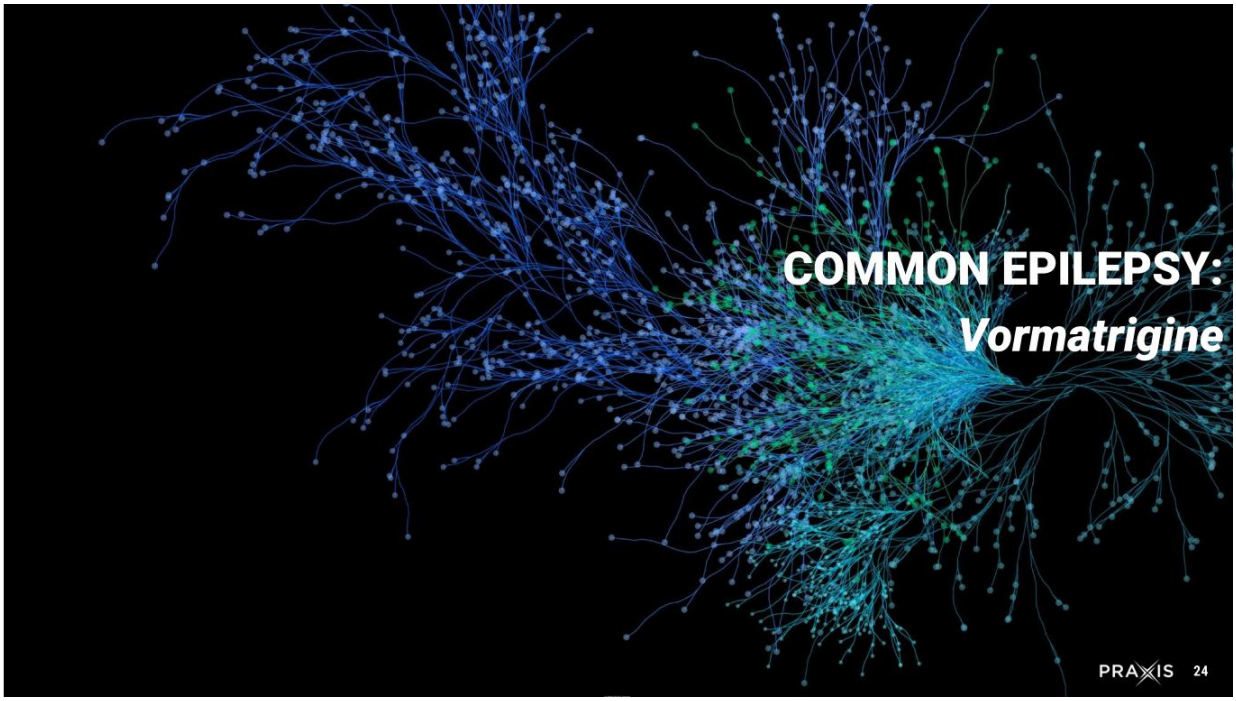
- Documented early onset SCN2A variant with seizures prior to 3 months of age
- Between the ages of 0 to ≤18 years at Screening (ages 2-18 go to Cohort 1, 1-2 to Cohort 2, 0-1 to Cohort 3)
- Seizure frequency of 4 or more countable motor seizures per 28-day during baseline

**Primary Endpoint**

- Median percent change in monthly motor seizure frequency from baseline

Expected to serve as registrational trial for NDA filing

ClinicalTrials.gov Identifier: NCT07019922


COMMON EPILEPSY:
Vormatrigine
PRAXIS
24

# Vormatrigine: Best-in-Disease Sodium Channel Modulator

***Epilepsy*** is a chronic neurological disorder that affects all age groups, causing life-threatening seizures

- An estimated **3 million** patients live with epilepsy
- ~35% of patients change medications annually
- 63% require two or more medications[1]
- Treatments are needed which are:
  - Fast acting
  - Durable
  - Better tolerability
  - Compatible with complex regimens

## Vormatrigine poised to rapidly transform the epilepsy landscape

### Superior Efficacy
- Best-in-disease efficacy in the RADIANT study
- Broad applicability across FOS and generalized epilepsy
- Sustained long-term effect

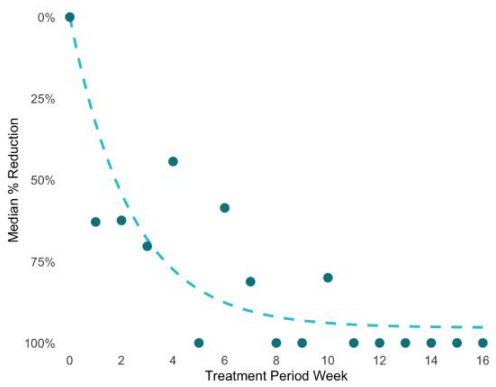
### Ease of Administration
- Once daily dose, fast acting
- No need to be taken with food or require dietary changes

### Ideal Tolerability and Limited DDIs
- Favorable safety profile
- Minimal drug-drug interaction risk with common ASMs

Kahlig et al AAN 2023; Patel et al AAN 2024; Hansen et al IEC 2025; Hansen et al AES 2025
[1]Praxis Claims Analysis on File 2024. FOS patient cohort (n = 440k); Gazdag et al AES 2026
ASM: Anti-seizure medication

# RADIANT Phase 2 Study Showed Disease-leading Efficacy

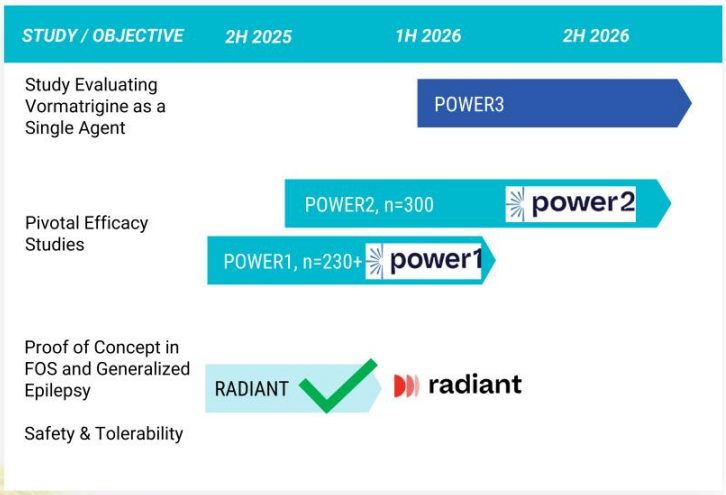
**MEDIAN % REDUCTION IN FOS (Weeks 1-16)**

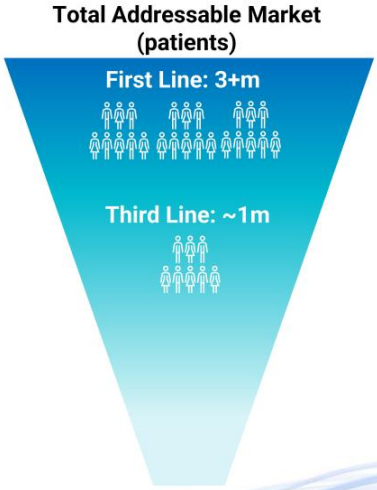



| DISEASE IMPACTING CRITERIA | RADIANT RESULTS |
|---|---|
| **Speed and durability of response** | • Rapid response after only 1 week of dosing<br>• Median reduction maintained at 100% after 10 weeks<br>• Generalized epilepsy patients had similar benefit of FOS patients |
| **Efficacy with other ASMs** | • Patients were on an average of 2.1 ASMs<br>• >30% of patients on best approved drug (cenobamate) |
| **Seizure freedom** | • 11% of patients were seizure free within the treatment period<br>• Over 30% were seizure free for any 28-day period |
| **Safety & tolerability** | • Lowest rate of TEAEs and CNS AEs with modern ASMs<br>• Most AEs were mild to moderate and transient |

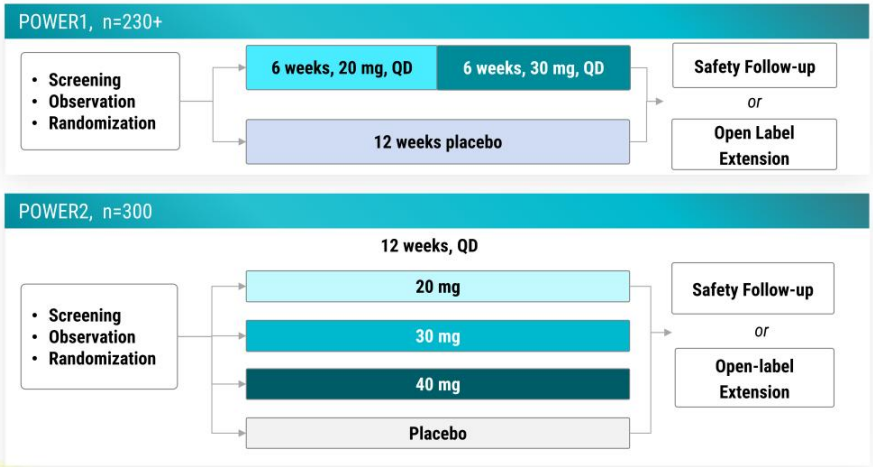
Hansen et al AES 2025

# Vormatrigine ENERGY Program: Developing for Broad, Foundational Use



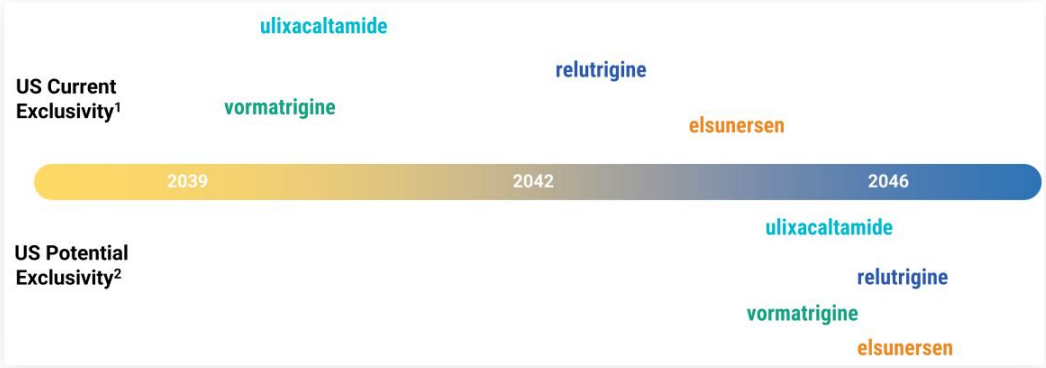
# Pivotal POWER1 Study Topline Results Q2 2026, POWER2 Topline Results in 2027




- Both studies expected to support NDA submission
- Range of doses in POWER2 based off PK/PD analysis to optimize efficacy opportunity

power1 power2

# Long, Multi-layered and Strong IP Position Across the Clinical Portfolio




1. Based on US Patent Nos. 11,649,207; 11,427,540; 12,077,502; 12,528,772; 11,014,931; 12,325,711; 12,582,652; 12,552,797; 11,866,439; 11,731,976; 11,731,978; 12,227,746; and 12,618,072
2. Based on issuing of US App Nos. 17/975,457; 18/834,466; 19/312,146; and others
3. Does not reflect any potential patent term extension

# Two Platforms Enabling Repeatable CNS Innovation

## Cerebrum™

SMALL MOLECULE PLATFORM

Cerebrum™ utilizes deep understanding of neuronal excitability and neuronal networks and applies a series of computational and experimental tools to develop orally available precision therapies

| MOLECULE | INDICATION | MECHANISM |
|---|---|---|
| ***ulixacaltamide*** | Essential Tremor[1] | Selective T-type calcium channel modulator |
| ***vormatrigine*** | FOS & Generalized Epilepsy | Sodium channel functional state modulator for broad use |
| ***relutrigine***[2] | Broad DEEs | Sodium channel functional state modulator for phenotypic DEEs |
| ***PRAX-020*** | KCNT1 | KCNT1 specific inhibitor |
| ***PRAX-050*** | Movement Disorders | Not disclosed |

## Solidus™

ANTISENSE OLIGONUCLEOTIDE (ASO) PLATFORM

Solidus™ is an efficient, targeted precision medicine discovery and development engine for ASOs anchored on proprietary, computational methodology

| MOLECULE | INDICATION | MECHANISM |
|---|---|---|
| ***elsunersen***[3] | Early onset SCN2A | Gapmer ASO |
| ***PRAX-080*** | PCDH19 | Gapmer ASO |
| ***PRAX-090*** | SYNGAP1 | Splice switching ASO |
| ***PRAX-100*** | SCN2A Autism | Undisclosed mechanism ASO |

1 Ulixacaltamide has received Breakthrough Therapy Designation (BTD)
2 Relutrigine has received BTD, Orphan Drug Designation (ODD) and Rare Pediatric Disease (RPD) designation from the FDA, and ODD from the European Medicines Agency (EMA) for the treatment of SCN2A and SCN8A-DEE and RPD designation for Dravet Syndrome
3 Elsunersen has received ODD and RPD designation from the FDA, and ODD and Priority Medicines (PRIME) designations from the EMA for the treatment of early SCN2A DEE
DEE: developmental & epileptic encephalopathy, FOS: focal onset seizures


PRAXIS
DARE FOR MORE®