



Investor Overview

September 2026

Forward-Looking Statements and Other Notices

Filana Therapeutics is in the business of new drug discovery and development. Our research and development (R&D) activities are long, complex, costly and involve a high degree of risk. Because risk is fundamental to the process of drug discovery and development, you are cautioned to not invest in our publicly traded securities unless you are prepared to sustain a total loss of the money you have invested. Only a small number of R&D programs result in regulatory approval and subsequent commercialization of a product.

Simufilam is our investigational drug product candidate. It is not approved by any regulatory authority in any jurisdiction and its safety, efficacy or other desirable attributes, if any, have not been established in patients. Data from our preclinical or prior clinical studies to date are inherently exploratory in nature, should be interpreted with caution and should not be interpreted as evidence of therapeutic safety or benefit for simufilam. Results from preclinical or earlier-stage clinical trials may not be indicative of future results from later-stage or larger-scale clinical trials and do not ensure regulatory approval. You are cautioned that subsequent results may differ materially.

This presentation, together with any accompanying oral statements, contains forward-looking statements, including, without limitation, plans to initiate a clinical study with simufilam for TSC-related epilepsy, the potential for simufilam as a treatment for TSC-related epilepsy and other potential indications, the timing of anticipated milestones, the potential to benefit from Orphan Drug Designation, and cash use in future periods. These statements may be identified by words such as “may,” “anticipate,” “believe,” “could,” “expect,” “would,” “forecast,” “intend,” “plan,” “possible,” “potential” and other words and terms of similar meaning or their negatives.

Such statements are based on our current expectations and projections about future events and performance. Such statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to, those risks relating to our ability to conduct or complete preclinical and clinical studies on expected timelines, to demonstrate the specificity, safety, efficacy or potential health benefits of our product candidates, if any, and including those described in the section entitled “Risk Factors” in our Annual Report on Form 10-K for the year ended December 31, 2025, and subsequent Quarterly Reports on Form 10-Q and Current Reports on Form 8-K filed with the SEC. The foregoing sets forth some, but not all, of the factors that could cause actual results to differ from expectations in any forward-looking statement. In light of these risks, uncertainties and assumptions, the forward-looking statements and events discussed in this presentation are inherently uncertain and may not occur, and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements. Accordingly, you should not rely upon forward-looking statements as predictions of future events. Information in this Presentation speaks only as of the date hereof, and except as required by law, we disclaim any intention or responsibility for updating or revising any forward-looking statements contained in this presentation. For further information regarding these and other risks related to our business, investors should consult our filings with the SEC, which are available on the SEC's website at www.sec.gov.

This presentation may contain statistical data and drug information based on independent sources, industry publications or other publicly available information. We have not independently verified the accuracy or completeness of such data and information. Accordingly, we make no representations as to the accuracy or completeness of such data or information. You are cautioned not to give undue weight to such data. This presentation is solely our responsibility and does not represent the views of the National Institutes of Health or any other government agency, or clinical site investigators, or other third-parties.

Holders of our common stock should carefully read our Annual Report on Form 10-K in its entirety, including the risk factors therein.

Investment Highlights



Significant unmet need in TSC-related epilepsy: Approximately 40,000–45,000 U.S. patients are affected, with more than 60% of treated patients remaining refractory to antiepileptic therapy.



Differentiated potential treatment approach: Simufilam is an oral small molecule being developed to modulate filamin A, a novel potential target in TSC-related epilepsy.



Established scientific and clinical foundation: Two preclinical mouse models support anti-seizure activity, while more than 2,000 subjects have received simufilam across prior clinical studies.



Phase 2a proof-of-concept catalyst approaching: FDA clinical hold lifted; patient screening expected to begin in Q1 2027 in a ~40-patient study across 13 U.S. sites.



Positioned to execute: Leading CRO engaged, clinical sites identified and product supply in place, supported by an experienced CNS team and a strong cash position.

Experienced Leadership Team

Management Team



Richard Barry
President & Chief Executive
Officer



Angelique Bordey, PhD
Senior Vice President,
Neuroscience



Eric Schoen
Chief Financial Officer



Christopher Cook
Chief Operating & Legal
Officer



Joseph Hulihan, MD
Chief Medical Officer

Board of Directors

Claude Nicaise, MD, *Chairman*

Richard Barry

Pierre Gravier, MS

Patrick Scannon, MD/PhD

Robert Anderson

Dawn C. Bir

Proof-of-Concept Study Design for TSC-Related Epilepsy

Randomized, double-blind, multi-center study



Screening: assess baseline seizure frequency

Randomization: two doses of simufilam, each added to standard of care

Assess safety and efficacy

Available to patients who complete treatment

Study Population:

- 13 U.S. sites
- ~40 patients ages 12–55

Primary Endpoints:

Safety, tolerability, pharmacokinetics

Secondary Endpoints:

Seizure-related measures, including seizure frequency, seizure intensity and duration, nighttime seizures, and sleep-related outcomes



TSC-Related Epilepsy

What is Tuberous Sclerosis Complex (TSC)¹?

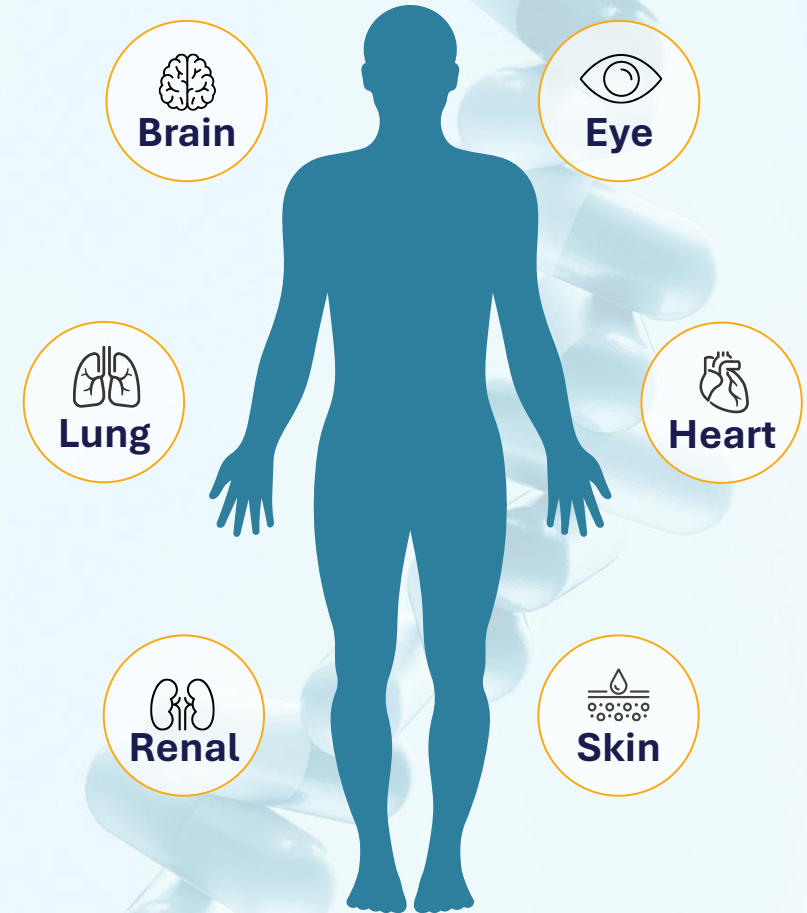
Tuberous Sclerosis Complex (TSC)

is a genetic disorder caused by mutations in the *TSC1* or *TSC2* gene and can involve multiple organs.

The disorder may present at any age and is often diagnosed based on specific clinical criteria and/or genetic testing.

Epilepsy is one of the most common neurologic manifestations associated with TSC.

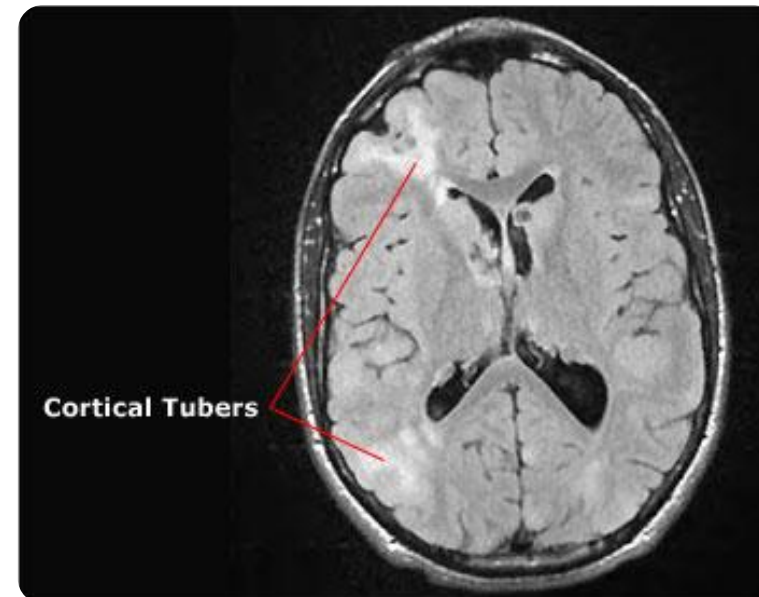
How TSC presents



Epilepsy in People with TSC

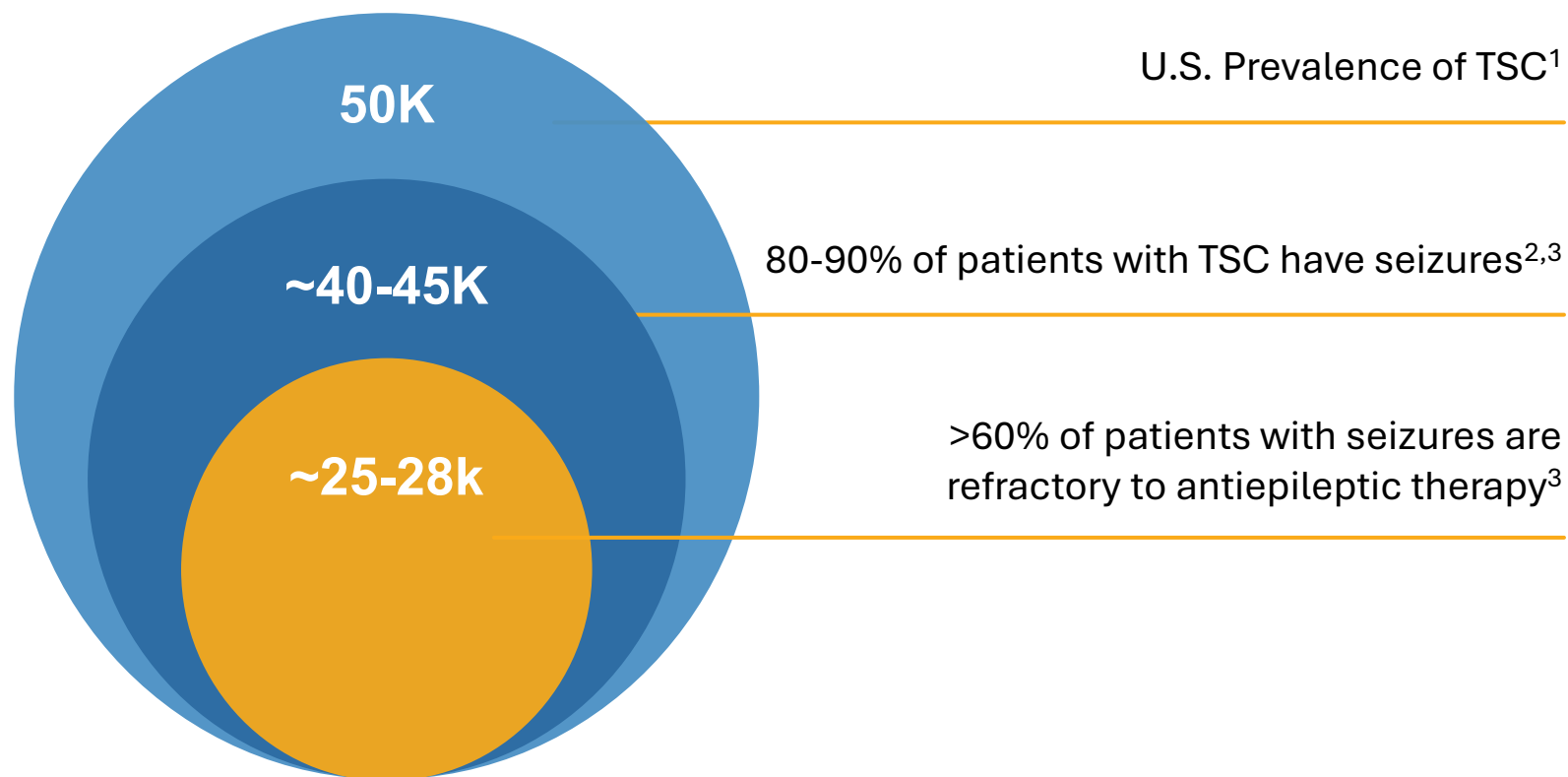
Seizure onset typically occurs in the first year of life¹.

80% to 90% of TSC patients experience seizures at some point in their life².



Brain malformations, otherwise known as cortical tubers, are a hallmark of disease, found in the brain of more than 80% of TSC patients³.

TSC-Related Epilepsy U.S. Market Opportunity



Current treatments include antiepileptic agents⁴.

Two therapies have been approved by FDA specifically for TSC-related epilepsy: everolimus⁵ and cannabidiol⁶.

Use of multiple agents and surgical intervention underscore the unmet need for improved therapy in people with TSC-related epilepsy⁷.

Sources: 1. <https://www.tscalliance.org/understanding-tsc/what-is-tsc/>, 2. Longbo Zhang et al., Filamin A inhibition reduces seizure activity in a mouse model of focal cortical malformations. *Sci. Transl. Med.* 12, eaay0289(2020). DOI:10.1126/scitranslmed.aay0289, 3. Chu-Shore CJ, Major P, Camposano S, Muzykewicz D, Thiele EA. The natural history of epilepsy in tuberous sclerosis complex. *Epilepsia*. 2010 Jul;51(7):1236-41. doi: 10.1111/j.1528-1167.2009.02474.x. Epub 2009 Dec 22. PMID: 20041940; PMCID: PMC3065368, 4. <https://www.tscalliance.org/understanding-tsc/clinical-manifestations/brain-neurological-function/epilepsy-seizure-disorders/epilepsy-treatment-options-for-individuals-with-tsc/>, 5. Everolimus/AFINITOR is distributed by Novartis Pharmaceuticals. 6. Cannabidiol/Epidiolox is distributed by Jazz Pharmaceutical, 8. Betts, K. A., Stockl, K. M., Yin, L., Hollenack, K. A., & Wang, M. (2020). 7. Economic Burden Associated with Tuberous Sclerosis Complex in Patients with Epilepsy. *Value in Health*, 23, S262-S262.

Simufilam, A Filamin A Modulator

**Simufilam is a
Novel Filamin A
Modulator**

Filamin A regulates diverse aspects of neuronal cell development¹.
Simufilam represents a potential first-in-class oral small molecule that potentially modulates filamin A function².
Multiple commercial-scale GMP batches of simufilam have been reproducibly manufactured on behalf of Filana Therapeutics.

**Simufilam's
Value Proposition
in TSC-Related
Epilepsy**

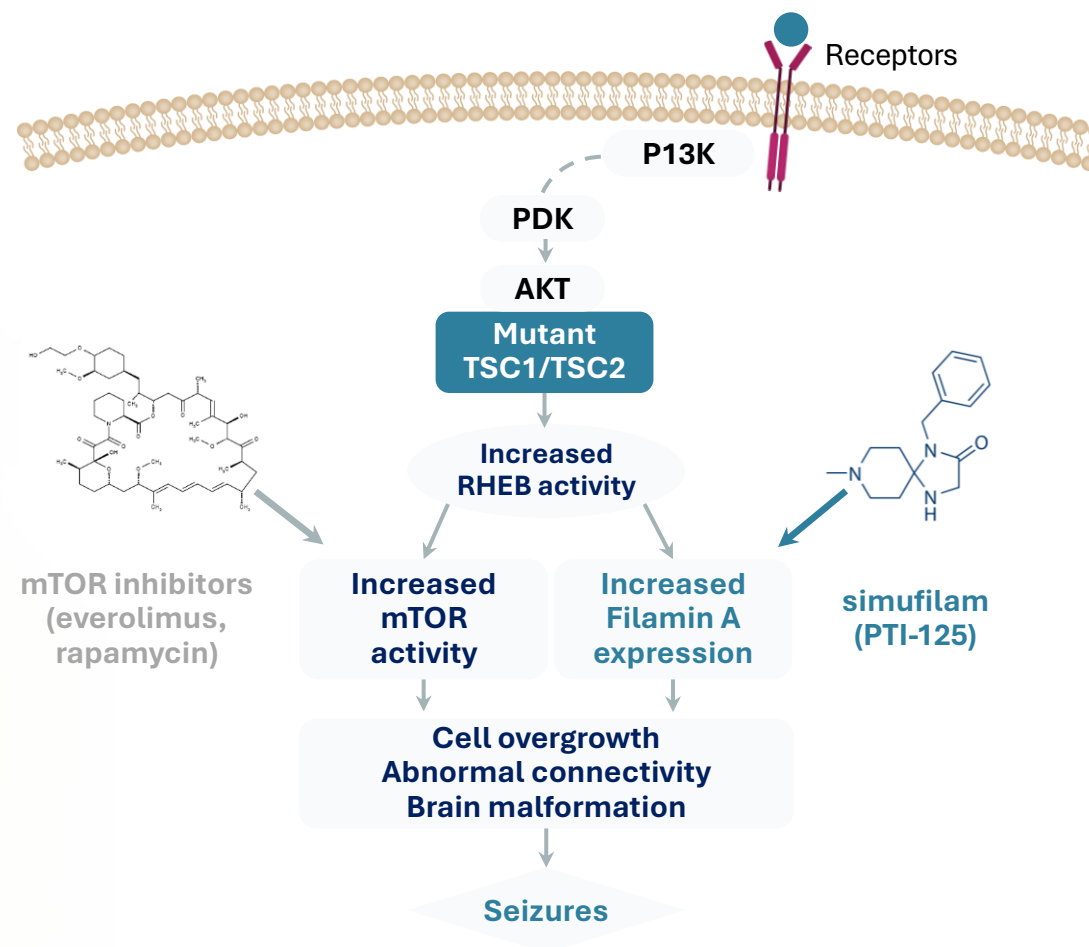
Potential to reduce the frequency of seizures.
Generally well-tolerated safety profile, based on data from >2,000 subjects treated across prior clinical trials.

Simufilam Represents a Novel Potential Approach to the Treatment of TSC-Related Epilepsy

The combination of mTOR activation and filamin A overexpression causes cell overgrowth, abnormal connectivity, and brain malformations, leading to seizures¹.

Filamin A represents a novel target, independent of mTOR.

Simufilam is a potential first-in-class filamin A-modulating agent with the potential to treat TSC-related epilepsy².





Preclinical Research

Encouraging Preclinical Data Support Development of Simufilam as a Potential Therapy for TSC-Related Epilepsy



Studies conducted in the lab of Dr. Angélique Bordey (the Bordey Lab) at Yale identified filamin A overexpression in patients with TSC and FCDII, suggesting that filamin A could be a **new therapeutic target in TSC-related epilepsy**^{1,2,3}.



The Bordey Lab showed that simufilam reduced seizure frequency in the Yale mouse model. Simufilam also reduced neuron overgrowth and brain malformations in these mice^{1,3}.



Attenuation of the increase in seizure activity was later shown in another mouse model of TSC-related seizures provided by the TSC Alliance (the “TSCA mouse model”).



Simufilam appears to modulate filamin A function³, **a new potential mechanism of action in TSC-related epilepsy.** Filamin A plays a role in brain development, including cell growth and neuronal connectivity².



Planning a clinical proof of concept study to evaluate simufilam in patients with TSC-related epilepsy, collaborating closely with the TSC Alliance and key opinion leaders.

Two Mouse Models of TSC-Related Epilepsy¹ Enable Preclinical Studies

Mouse Model		Characteristics Connecting Brain Abnormalities and Seizures
Yale Mouse Model²	<i>In utero</i> expression of a constitutively active RHEB, a key regulator of mTOR signaling.	 Cortical tuber-like malformations lead to spontaneous seizures.
TSCA Mouse Model²	Transgenic mice with embryonic, conditional <i>Tsc1</i> deletion (cKO, human <i>GFAP</i> promoter).	 Widespread forebrain abnormalities lead to spontaneous seizures.

Filamin A Modulators Impact Neuron Morphology and Seizure Activity in the Yale Mouse Model¹



Seizure activity worsens with time, consistent with increasing cell overgrowth and dendritic abnormalities.



Evaluate multiple age cohorts and treatment and control regimens.



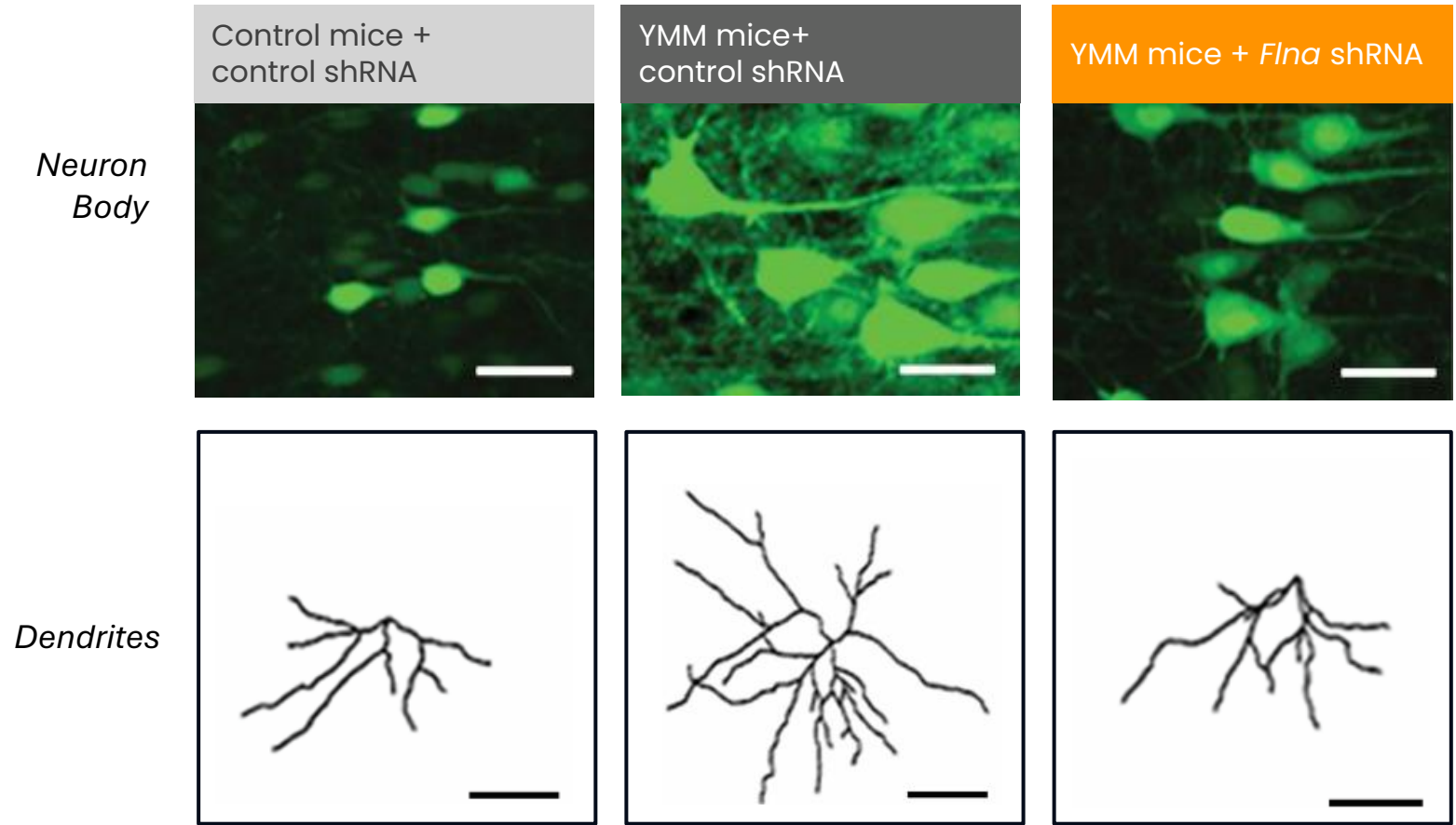
EEG seizure measurements for a range of 5-40 days.

Main Endpoints:

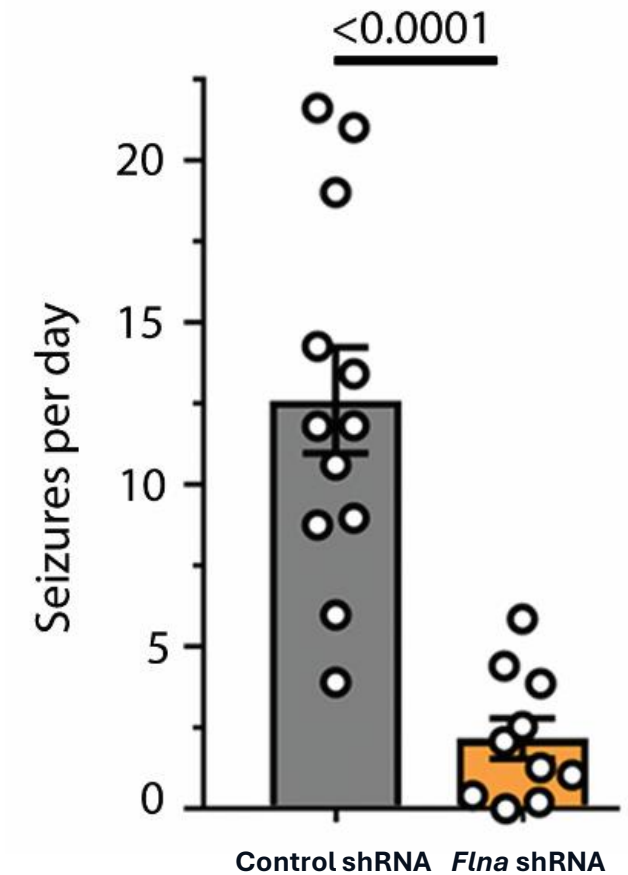
neuron cell body overgrowth, dendrite morphology and seizure activity

Normalizing Filamin A Expression via shRNA Recovered Neuron Morphology and Reduced Seizure Activity in Yale Mouse Model

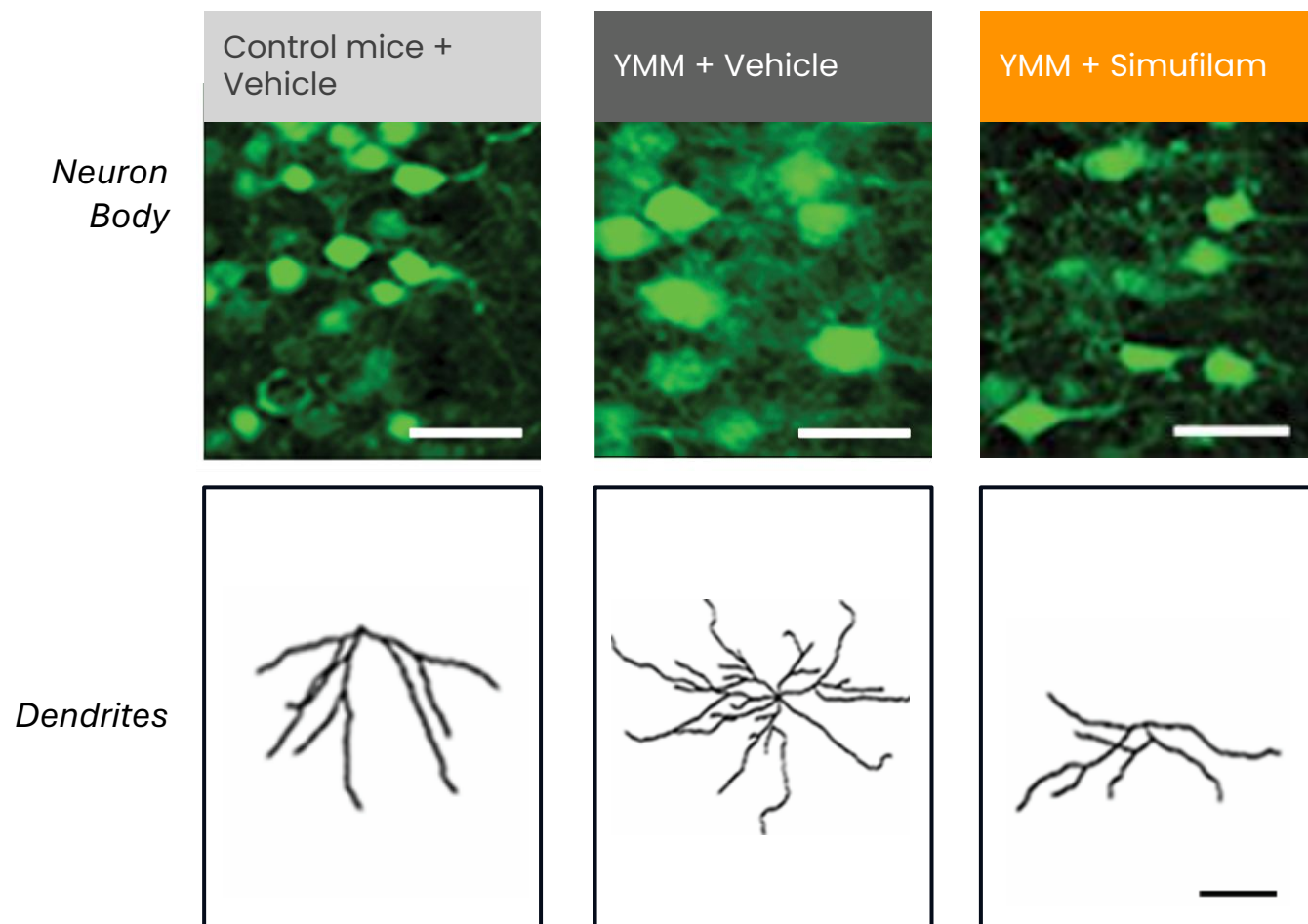
Partial Reversal of Cell Overgrowth and Dendritic Hypertrophy^{1,2}



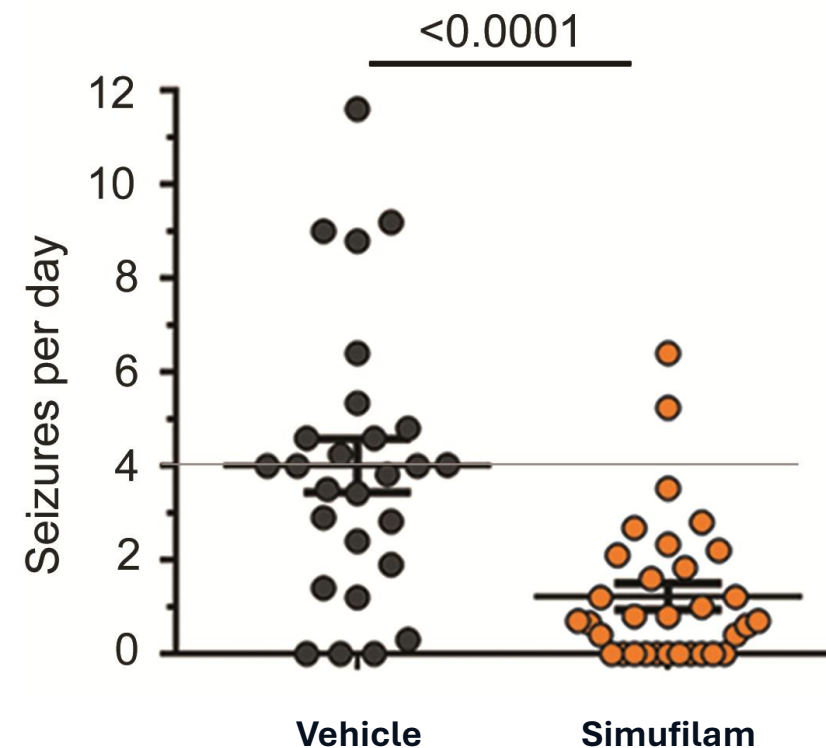
Reduction in seizure activity³



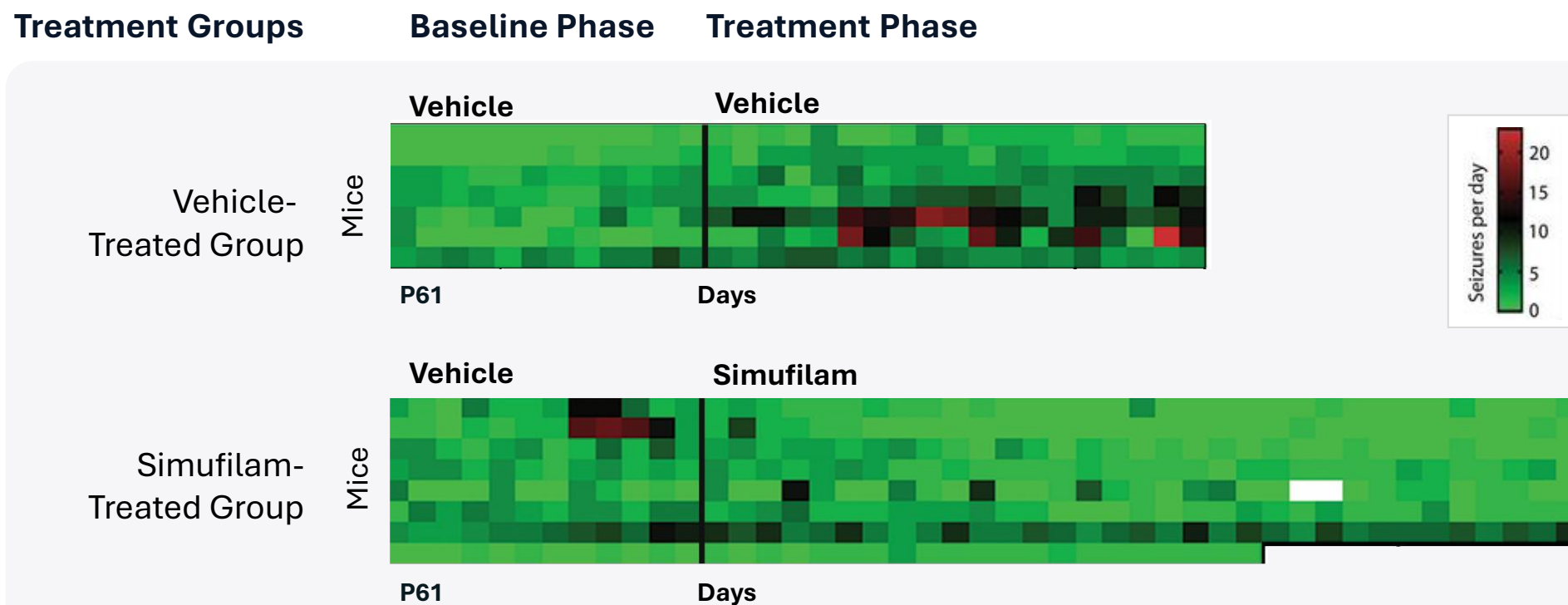
Simufilam Recovered Cell Morphology, and Reduced Seizures, Consistent with FLNA shRNA Treatment in the Yale Mouse Model



60% reduction in seizure frequency¹

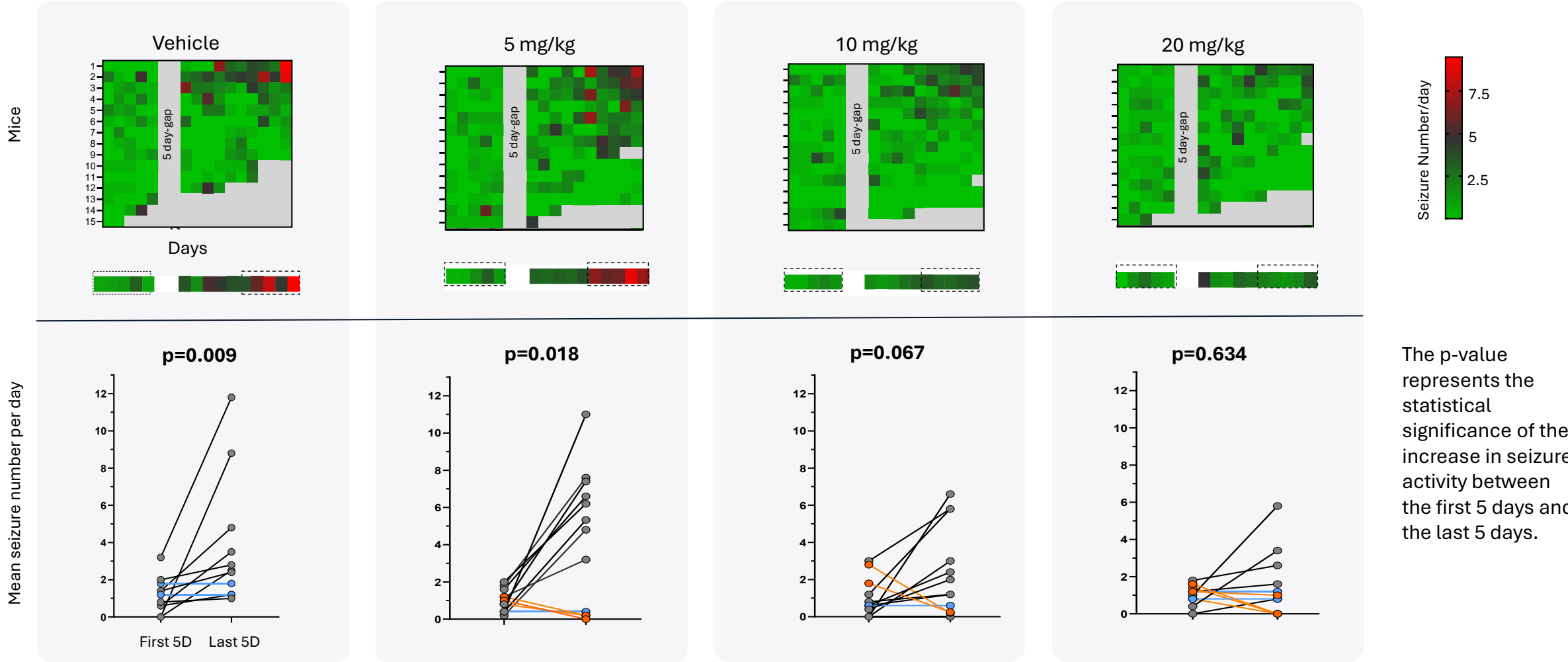


Simufilam Reduced Seizure Activity In Yale Mouse Model

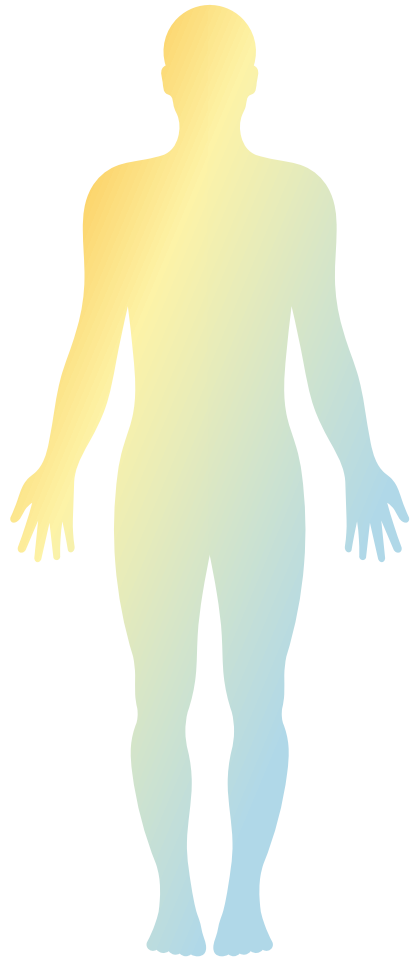


Seizure activity in untreated adult mice worsened with time,
consistent with an increase in cell overgrowth and network abnormalities

Simufilam Attenuated Increased Seizure Activity In TSCA Mouse Model, in a Dose Dependent Manner



Simufilam Showed a Good Overall Safety Profile in Prior Human Studies¹



>2,000

individuals treated with simufilam in prior clinical studies, with some patients receiving treatment for as long as 24 months.

Dataset includes several Phase 1, Phase 2 and Phase 3 studies in Alzheimer's disease.

Good overall safety profile¹

Adverse event profile comparable to placebo

No drug-related Serious Adverse Events



Intellectual Property and Development Path

Simufilam Intellectual Property Portfolio



Filana Therapeutics TSC-Related Epilepsy Development Path

Timing	Focus	Development Step
Q4 2025	Clinical	IND Filing and Clinical Hold Following the IND submission, the FDA issued a clinical hold requesting additional information, including preclinical data and protocol modifications ¹ .
Q2 2026	Preclinical	Preclinical Data Presented Preclinical data published in Epilepsia ² and presented at the Eilat XVIII antiepileptic drug conference ³ , reinforcing simufilam's biological rationale in TSC-related epilepsy.
September 2026	Clinical	Regulatory clearance Phase 2a preparations to advance with IRB submission and site contracting following FDA lift of clinical hold ⁴ .
Q4 2026	Clinical	Site engagement Company to begin recruiting and contracting study sites.
Q1 2027	Clinical	Screening initiation Initiate screening in Phase 2a study.



Building on Simufilam Data to Pursue Development in Tuberous Sclerosis Complex (TSC)- Related Epilepsy

- **Cash, cash equivalents** as of June 30, 2026, were \$82.7M; no debt¹.
- **Net cash used in operations for H2 2026** expected to be \$11 to \$15M¹, plus payment into escrow of a \$31.25M estimated loss contingency related to potential settlement of certain securities litigation, recorded in 2025. This escrow will remain restricted subject to final court approval.
- **Estimated unrestricted cash at December 31, 2026** expected to be \$36-40M¹.
- **Shares outstanding** of 48.3M as of July 27, 2026¹

Investment Highlights



Meaningful unmet need in TSC-related epilepsy, affecting ~40,000–45,000 U.S. patients



Differentiated potential treatment approach with simufilam, based on data from two preclinical models



Safety profile de-risked by prior studies in >2,000 subjects



Phase 2a proof-of-concept study advancing toward Q1 2027 screening



CRO, sites and product supply in place to support execution

Driven by a Bold Mission

We are driven by our mission to develop transformative medicines to improve the lives of patients with CNS disorders such as TSC-related epilepsy, and other diseases associated with dysregulation or overexpression of filamin A.

Thank you

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