Filana Therapeutics Announces FDA Lift of Clinical Hold on Simufilam, Enabling Phase 2a Study in TSC-Related Epilepsy

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- *Patient screening for the Phase 2a study is expected to begin by the first quarter of 2027*
- *Filana has engaged a leading clinical research organization, has identified clinical trial sites, and is recruiting potential investigators*
- *Ongoing collaboration with the TSC Alliance and the broader TSC community is intended to support patient engagement and study execution*

AUSTIN, Texas, Sept. 22, 2026 (GLOBE NEWSWIRE) -- Filana Therapeutics, Inc. (NASDAQ: FLNA, “Filana Therapeutics”, the “Company”), a biotechnology company currently focused on developing simufilam for the treatment of Tuberous Sclerosis Complex (TSC)-related epilepsy, today announced that the U.S. Food and Drug Administration (FDA) has lifted the clinical hold on the Company's investigational new drug (IND) application, enabling Filana to initiate a planned Phase 2a proof-of-concept study in patients aged 12 to 55 with TSC-related epilepsy. Patient screening is expected to begin by the first quarter of 2027.

“With the clinical hold lifted, we can now execute our development plan for simufilam in TSC-related epilepsy,” said **Rick Barry, President and Chief Executive Officer of Filana**. “While on hold, we completed key trial-readiness activities, including engaging a leading clinical research organization, identifying clinical trial sites, and recruiting potential investigators. With investigational drug supply in place, implementation of our Phase 2a study of simufilam is now able to progress expeditiously toward site initiation and patient enrollment.”

“Drug-resistant epilepsy remains a challenge for a majority of individuals affected by TSC,” said **Kari Luther Rosbeck, President and Chief Executive Officer of the TSC Alliance**. “Research investigating different molecular pathways involved in TSC offers hope for potential new options for those living with the disease. The TSC Alliance applauds Filana for their innovative work in exploring a new target mechanism and their plans to launch a clinical trial to test the effectiveness of that potential new treatment to help address a major unmet need for the TSC community.”

Planned Phase 2a Trial Design

Filana’s Phase 2a study is a 16-week multicenter, randomized, double-blind clinical trial to evaluate two doses of simufilam in patients with refractory TSC-related seizures. The study is planned to be conducted at 13 sites in the U.S. with a target enrollment of 40 subjects aged 12 to 55. All subjects who complete the double-blind treatment phase are eligible to participate in a 48-week extension study.

The study is designed to assess safety, tolerability, pharmacokinetics, and seizure-related measures, including seizure frequency, seizure intensity and duration, nighttime seizures, and sleep-related outcomes. Data from the study are expected to help inform the development path for simufilam in TSC-related epilepsy, including the design of future studies in younger patient populations.

Scientific Rationale

Simufilam is an oral small molecule intended to modulate the filamin A protein. The clinical evaluation of simufilam in TSC-related epilepsy is supported by findings from two preclinical mouse models. Initial studies were conducted in the laboratory of Angélique Bordey, PhD, Rothberg Professor of Neurosurgery at Yale School of Medicine and Senior Vice President, Neuroscience at Filana. Those studies showed that simufilam reduced seizure frequency in a mouse model of focal onset seizures involving TSC-related pathology[1]. In a separate study conducted with the TSC Alliance Preclinical Consortium using a well-accepted TSC-knockout mouse model, simufilam attenuated the progression of seizure activity in a dose-dependent manner[2].

Filana holds an exclusive worldwide license from Yale University to intellectual property supporting the use of simufilam in TSC-related epilepsy.

About TSC and TSC-Related Epilepsy

TSC is a rare genetic disorder resulting from a mutation in the *TSC1* or *TSC2* gene. These mutations affect the mechanistic target of rapamycin (mTOR) pathway and can cause tumors to grow in multiple organs[3,4]. Epilepsy is the most common health issue affecting the TSC community, with 80% to 90% of TSC patients experiencing seizures[5]. TSC-related epilepsy affects approximately 45,000 people in the U.S.[1,3]. Most patients start having seizures within their first year of life[1]. Even with multiple approved treatments, more than 60% of TSC patients remain refractory to antiepileptic therapy[6].

About Filana Therapeutics, Inc.

Filana Therapeutics, Inc. (NASDAQ: FLNA), is a biotechnology company focused on developing novel, investigational therapies to modulate the filamin A protein for the treatment of central nervous system disorders, such as tuberous sclerosis complex (TSC)-related epilepsy, and other diseases associated with dysregulation or overexpression of filamin A.

For more information, please visit: https://www.FilanaTx.com

References:

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Cautionary Note Regarding Forward-Looking Statements:

This news release contains forward-looking statements that may include but are not limited to statements regarding: the timing and plans to initiate and conduct clinical studies with simufilam in TSC-related epilepsy; the design, structure, duration, objectives, endpoints, patient population, conduct, enrollment, completion, and potential results of our planned Phase 2a clinical trial; our ability to work with clinical research organizations, clinical sites, patient advocacy organizations, and the TSC community to advance the trial efficiently; the potential for simufilam as a treatment for TSC-related epilepsy and other potential indications; and the timing of anticipated milestones, including announcing study sites and screening patients. These statements may be identified by words such as "anticipate", "before", "believe", "could", "expect", "forecast", "intend", "may", "pending", "plan", "possible", "potential", "prepares for", "will", and other words and terms of similar meaning.

Such statements are based on our current expectations and projections about future events. Such statements speak only as of the date of this news release and are subject to a number of risks, uncertainties and assumptions, including, but not limited to, those risks relating to our ability to initiate, enroll, conduct, and complete the planned Phase 2a study of simufilam in TSC-related epilepsy; our ability to engage clinical trial sites and participants; our ability to work effectively with clinical research organizations, vendors, investigators, patient advocacy organizations, and the TSC community; potential changes to the clinical trial protocol, trial design, endpoints, timing, patient population, or development plans; our ability to generate clinical data that support further development of simufilam; risks inherent in drug discovery and development; and other risks specific to Filana Therapeutics, Inc., as described in the section entitled "Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2025 and subsequent reports to be filed with the SEC. The foregoing sets forth many, 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 news release 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. Except as required by law, we disclaim any intention or responsibility for updating or revising any forward-looking statements. 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.

All of our pharmaceutical assets under development are investigational product candidates. These have not been approved for use in any medical indication by any regulatory authority in any jurisdiction and their safety, efficacy or other desirable attributes, if any, have not been established in any patient population. Consequently, none of our product candidates is approved or available for sale anywhere in the world.

Our clinical results from earlier-stage clinical trials or preclinical studies may not be indicative of future results from later-stage or larger scale clinical trials and do not ensure regulatory approval. You should not place undue reliance on these statements or any scientific data we present or publish.

We are in the business of new drug discovery and development. Our research and development activities are long, complex, costly and involve a high degree of risk. Holders of our common stock should carefully read our Annual Report on Form 10-K and subsequent Quarterly Reports on Form 10-Q in their entirety, including the risk factors therein. 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.


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