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): March 31, 2025

Palvella Therapeutics, Inc.

(Exact name of registrant as specified in its charter)

Nevada	**001-37471**	**30-0784346**
(State or other jurisdiction of incorporation)	**(Commission File Number)**	**(IRS Employer Identification No.)**
125 Strafford Ave, Suite 360 Wayne, Pennsylvania		**19087**
(Address of principal executive offices)		**(Zip Code)**

Registrant's telephone number, including area code: (484) 253-1461

(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	**Trading Symbol(s)**	**Name of each exchange on which registered**
Common Stock, $0.001 par value per share	PVLA	The Nasdaq Capital 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 March 31, 2025, Palvella Therapeutics, Inc. (the "Company") announced its financial results for the year ended December 31, 2024. A copy of the press release is being furnished as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated herein by reference.

The information furnished pursuant to this Item 2.02, including Exhibit 99.1 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 (the "Securities Act"), or the Exchange Act, except as expressly set forth by specific reference in such filing.

Item 7.01 Regulation FD Disclosure.

On March 31, 2025, the Company will hold its earnings call and use a slide presentation in conjunction with the earnings call. A copy of the presentation is furnished herewith as Exhibit 99.2, and incorporated herein by reference.

The information furnished pursuant to Item 7.01, including Exhibit 99.2, shall not be deemed "filed" for purposes of Section 18 of the Exchange Act or otherwise subject to the liabilities of that section, and shall not be deemed to be incorporated by reference in any filing under the Securities Act 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 of Palvella Therapeutics, Inc., dated March 31, 2025*
99.2	Earnings Call Presentation of Palvella Therapeutics, Inc., dated March 31, 2025*
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

* Furnished herewith

SIGNATURES

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.

Palvella Therapeutics, Inc.

Date: March 31, 2025

By: /s/ Matthew Korenberg
Matthew Korenberg
Chief Financial Officer

Exhibit 99.1

palvella
THERAPEUTICS

Palvella Therapeutics Reports Full Year 2024 Financial Results and Provides Corporate Update

Upon close of merger and $78.9mm concurrent private placement from a syndicate of leading healthcare-dedicated investors, completed transformation to a publicly traded rare disease biopharmaceutical company advancing a late clinical-stage pipeline and a platform for treating serious, rare genetic skin diseases

Top-line results from SELVA, a Phase 3 single-arm, baseline-controlled trial evaluating QTORIN™ 3.9% rapamycin anhydrous gel (QTORIN™ rapamycin) for the treatment of microcystic lymphatic malformations (microcystic LMs), on track for the first quarter of 2026

Top-line results from TOIVA, a Phase 2 single-arm, baseline-controlled trial evaluating QTORIN™ rapamycin for the treatment of cutaneous venous malformations (cutaneous VMs), on track for the fourth quarter of 2025

Planned QTORIN™ pipeline expansion in second half of 2025

Cash and cash equivalents of over $83 million as of December 31, 2024 expected to fund operations into the second half of 2027

Company to host conference call at 8:30 a.m. ET today

WAYNE, PA., March 31, 2025 (GLOBE NEWSWIRE) -- (Nasdaq: PVLA) Palvella Therapeutics, Inc. (Palvella or “the Company”), a clinical-stage biopharmaceutical company focused on developing and commercializing novel therapies to treat patients suffering from serious, rare genetic skin diseases for which there are no U.S. Food and Drug Administration (FDA)-approved therapies, reported financial results for the full year ending December 31, 2024 and provided a corporate update.

"2024 was marked by significant progress towards achieving our vision of becoming the leading rare disease biopharmaceutical company focused on serious, rare genetic skin diseases," said Wes Kaupinen, Founder and Chief Executive Officer of Palvella. "Upon the close of our merger and concurrent private placement in December 2024, we were able to rapidly advance QTORIN™ rapamycin, our lead product candidate from the QTORIN™ platform, into the Phase 3 SELVA study for the treatment of microcystic lymphatic malformations and the Phase 2 TOIVA study for the treatment of cutaneous venous malformations. Microcystic LMs and cutaneous VMs are both serious, rare, and chronically debilitating genetic diseases for which QTORIN™ rapamycin has the potential to be the first FDA-approved therapy and standard of care in the U.S.”

Mr. Kaupinen continued, “In addition to these two initial indications for QTORIN™ rapamycin, which we believe together could exceed $1 billion in U.S. peak annual sales,

we plan to broaden our pipeline in 2025 by advancing new and existing QTORIN™ programs for additional serious, rare genetic skin diseases with no FDA-approved therapies."

Recent Research and Development Highlights

QTORIN™ rapamycin for the treatment of microcystic LMs

- •Microcystic LMs are a rare, chronically debilitating genetic disease caused by dysregulation of the phosphatidylinositol 3-kinase (PI3K)/mammalian target of rapamycin (mTOR) pathway. The disease is characterized by malformed lymphatic vessels that protrude through the skin and persistently leak lymph fluid (lymphorrhea) and bleed, often leading to recurrent serious infections and cellulitis that can cause hospitalization.
- •There are no FDA-approved treatments for the estimated more than 30,000 individuals in the U.S. with microcystic LMs.
- •In October 2024, the Company was awarded an Orphan Products Clinical Trials Program grant of up to $2.6 million from the FDA Office of Orphan Products Development to support the Phase 3 SELVA study, a 24-week, Phase 3, single-arm, baseline-controlled clinical trial of QTORIN™ rapamycin for the treatment of microcystic LMs. Out of 51 grant applications received by the FDA Orphan Products Grants Program in fiscal year 2024, Palvella's Phase 3 clinical trial was one of seven new clinical trials that was awarded a grant.
- •Two posters, including data supporting QTORIN™ rapamycin as a potential targeted therapy for the treatment of microcystic LMs and a review of the Phase 3 SELVA study, were presented at the 12th Pediatric Dermatology Research Alliance (PeDRA) Annual Conference in October 2024.
- •In November 2024, Palvella dosed the first patient in the Phase 3 SELVA study.
- •Breakthrough Therapy Designation, Orphan Drug Designation, and Fast Track Designation from the U.S. FDA have been granted to QTORIN™ rapamycin for the treatment of microcystic LMs. Orphan Drug Designation has also been granted by the European Medicines Agency.
- •Top-line results from SELVA are anticipated in the first quarter of 2026.

QTORIN™ rapamycin for the treatment of cutaneous VMs

- •Cutaneous VMs are a rare genetic disease caused by mutations in genes that cause overactivation of the PI3K/mTOR signaling pathway, leading to dysfunctional veins within the skin. These malformations can cause substantial morbidity and functional impairment, significantly impact quality of life, and are associated with severe bleeding, ulceration, and other potential complications.

- There are no FDA-approved treatments for the estimated more than 75,000 individuals in the U.S. with cutaneous VMs.
- Published case studies and real-world evidence from over 20 publications have provided preliminary evidence of clinical benefit from the off-label use of systemic rapamycin in the treatment of patients with venous malformations who have TIE2 and PIK3CA mutations while highlighting the need for topical agents which could potentially reduce the toxicities associated with systemic therapy.
- In January 2025, Palvella announced the dosing of the first patients in TOIVA, a Phase 2 single-arm, open-label, baseline-controlled clinical trial of QTORIN™ rapamycin for the treatment of cutaneous VMs.
- Fast Track Designation from the FDA has been granted to QTORIN™ rapamycin for the treatment of venous malformations.
- Top-line results from TOIVA are anticipated in the fourth quarter of 2025.

QTORIN™ rapamycin and QTORIN™ platform expansion

- The next target clinical indication for QTORIN™ rapamycin is anticipated in the second half of 2025. The expansion of QTORIN™ rapamycin into additional indications is supported by comprehensive publications by leading researchers, including Andrew Swarbrick et al (2021) and Dr. Joyce Teng and colleagues (Fogel et al, 2015) which highlight the broad potential of rapamycin in several difficult to treat, mTOR-driven skin diseases while advocating for targeted, topical approaches suited to improve tolerability and safety.

- The second product candidate from the QTORIN™ platform is anticipated in the second half of 2025. Similar to QTORIN™ rapamycin, we believe this product candidate will have the potential to be developed for several serious, rare genetic skin diseases.

Recent Corporate Highlights

- In July 2024, Palvella announced a definitive merger agreement with Pieris Pharmaceuticals. In December 2024, the Company closed the merger and a concurrent private placement of $78.9 million, co-led by BVF Partners L.P. and Frazier Life Sciences. Additional new investors included Blue Owl Healthcare Opportunities, Nantahala Capital, DAFNA Capital Management, ADAR1 Capital Management, and a healthcare dedicated fund. Existing investors Samsara BioCapital, Petrichor, CAM Capital, Ligand Pharmaceuticals (Nasdaq: LGND), Integrated Finance Group (an AscellaHealth partner company), BioAdvance, and Gore Range Capital also committed to participate in the financing.

•Matthew Korenberg was appointed as Chief Financial Officer in October 2024. Mr. Korenberg is a seasoned executive with significant operational and financial leadership experience, including senior roles at Ligand Pharmaceuticals (NASDAQ: LGND) and in healthcare investment banking at Goldman Sachs.

Full Year 2024 Financial Results

•Cash and cash equivalents as of December 31, 2024, were $83.6 million. Palvella expects such resources will be sufficient to fund its operations into the second half of 2027, and sufficient to accomplish its current strategic agenda.

•Research and development expenses were $8.2 million for the twelve months ended December 31, 2024, compared to $8.8 million for the twelve months ended December 31, 2023.

•General and administrative expenses were $5.9 million for the twelve months ended December 31, 2024, compared to $3.1 million for the twelve months ended December 31, 2023. The increase in G&A expenses was primarily driven by increases in expenses related to becoming a public company.

•Net loss was $17.4 million or $7.83 per basic and diluted share for the twelve months ended December 31, 2024, compared to net income of $17.9 million or $2.19 and $2.17 per basic and diluted share, respectively, for the twelve months ended December 31, 2023.

•Shares outstanding were 13,687,830 as of December 31, 2024, including 11,012,105 shares of common stock and 2,675,725 common share equivalents assuming conversion of our outstanding preferred shares and prefunded warrants.

Conference Call Details

Palvella will host a conference call and live audiovisual webcast to discuss the Company's full year 2024 financial results and provide a corporate update at 8:30 a.m. ET today. To access the live webcast of the call with slides please click here or visit the "Events & Presentations" section of Palvella's website. To access the call by phone, please use this registration link, and you will be provided with dial in details. A replay of the webcast will be available approximately 2 hours after the conclusion of the call and archived for 90 days under the "Events & Presentations" section of the Company's website at www.palvellatx.com.

About Microcystic Lymphatic Malformations

Microcystic LMs are a rare, chronically debilitating genetic disease caused by dysregulation of the phosphatidylinositol 3-kinase (PI3K)/mammalian target of rapamycin (mTOR) pathway. The disease is characterized by malformed lymphatic vessels that protrude through the skin and persistently leak lymph fluid (lymphorrhea) and bleed, often

leading to recurrent serious infections and cellulitis that can cause hospitalization. The natural history of microcystic LMs is persistent and progressive without spontaneous resolution, with symptoms generally worsening during life, including increases in the number and size of malformed vessels that lead to complications and lifetime morbidity. There are currently no FDA-approved treatments for the estimated more than 30,000 diagnosed patients with microcystic LMs in the United States.

About Cutaneous Venous Malformations

Cutaneous VMs are a rare genetic disease caused by mutations in genes that cause overactivation of the PI3K/mTOR signaling pathway, leading to dysfunctional veins within the skin. These malformations can cause substantial morbidity and functional impairment, significantly impact quality of life, and are associated with severe bleeding, ulceration, and other potential complications. An urgent need exists for an FDA-approved, targeted, localized therapy to treat cutaneous VMs. While published case studies and real-world evidence have provided preliminary evidence of clinical benefit from the off-label use of systemic mTOR inhibitors for venous malformations, there are currently no FDA-approved therapies for the estimated more than 75,000 diagnosed patients with cutaneous VMs in the U.S.

About Palvella Therapeutics

Founded and led by rare disease drug development veterans, Palvella Therapeutics, Inc. (Nasdaq: PVLA) is a clinical-stage biopharmaceutical company focused on developing and commercializing novel therapies to treat patients suffering from serious, rare genetic skin diseases for which there are no FDA-approved therapies. Palvella is developing a broad pipeline of product candidates based on its patented QTORIN™ platform, with an initial focus on serious, rare genetic skin diseases, many of which are lifelong in nature. Palvella's lead product candidate, QTORIN 3.9% rapamycin anhydrous gel (QTORIN™ rapamycin), is currently being evaluated in the Phase 3 SELVA clinical trial in microcystic lymphatic malformations and the Phase 2 TOIVA clinical trial in cutaneous venous malformations. For more information, please visit www.palvellatx.com or follow Palvella on LinkedIn or X (formerly known as Twitter).

QTORIN™ rapamycin is for investigational use only and has not been approved or cleared by the FDA or by any other regulatory agency for any indication.

Forward-Looking Statements

This press release contains forward-looking statements (including within the meaning of Section 21E of the Securities Exchange Act of 1934, as amended, and Section 27A of the Securities Act of 1933, as amended (Securities Act)). These statements may discuss goals, intentions, and expectations as to future plans, trends, events, results of operations or financial condition, or otherwise, based on current beliefs of the management of Palvella, as well as assumptions made by, and information currently available to, the

management of Palvella. Forward-looking statements generally include statements that are predictive in nature and depend upon or refer to future events or conditions, and include words such as “may,” “will,” “should,” “would,” “expect,” “anticipate,” “plan,” “likely,” “believe,” “estimate,” “project,” “intend,” and other similar expressions or the negative or plural of these words, or other similar expressions that are predictions or indicate future events or prospects, although not all forward-looking statements contain these words. Statements that are not historical facts are forward-looking statements. Forward-looking statements include, but are not limited to, statements regarding the expected timing of the presentation of data from ongoing clinical trials, Palvella’s clinical development plans and related anticipated development milestones, Palvella’s cash and financial resources and expected cash runway, and the potential of, and expectations regarding, Palvella’s programs, including QTORIN™ rapamycin, and its research-stage opportunities, including its expected therapeutic potential and market opportunity. Forward-looking statements are based on current beliefs and assumptions that are subject to risks and uncertainties and are not guarantees of future performance. Actual results could differ materially from those contained in any forward-looking statement as a result of various factors, including, without limitation: the ability to raise additional capital to finance operations; the ability to advance product candidates through preclinical and clinical development; the ability to obtain regulatory approval for, and ultimately commercialize, Palvella’s product candidates, including QTORIN™ rapamycin; the outcome of early clinical trials for Palvella’s product candidates, including the ability of those trials to satisfy relevant governmental or regulatory requirements; the fact that data and results from clinical studies may not necessarily be indicative of future results; Palvella’s limited experience in designing clinical trials and lack of experience in conducting clinical trials; the ability to identify and pivot to other programs, product candidates, or indications that may be more profitable or successful than Palvella’s current product candidates; the substantial competition Palvella faces in discovering, developing, or commercializing products; the negative impacts of global events on operations, including ongoing and planned clinical trials and ongoing and planned preclinical studies; the ability to attract, hire, and retain skilled executive officers and employees; the ability of Palvella to protect its intellectual property and proprietary technologies; reliance on third parties, contract manufacturers, and contract research organizations; and the risks and uncertainties described in the filings made by Palvella with the Securities and Exchange Commission (SEC), including the annual report on Form 10-K, quarterly reports on Form 10-Q and current reports on Form 8-K, filed with or furnished to the SEC and available at www.sec.gov. The events and circumstances reflected in our forward-looking statements may not be achieved or occur, and actual results could differ materially from those projected in the forward-looking statements. New risk factors and uncertainties may emerge from time to time, and it is not possible for management to predict all risk factors and uncertainties that Palvella may face. Except as required by applicable law, Palvella does 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.

This press release contains hyperlinks to information that is not deemed to be incorporated by reference into this press release.

Contact Information

Investors
Wesley H. Kaupinen
Founder and CEO, Palvella Therapeutics
wes.kaupinen@palvellatx.com

Media
Marcy Nanus
Managing Partner, Trilon Advisors LLC
mnanus@trilonadvisors.com

PALVELLA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(in thousands, except share and per share amounts)

	Year Ended December 31,	
	2024	2023
Operating expenses:		
Research and development	$ 8,151	$ 8,793
General and administrative	5,944	3,076
Total operating expenses	14,095	11,869
Loss from operations	(14,095)	(11,869)
Total other income (expense), net	(3,339)	30,560
Net loss	$ (17,434)	$ 18,691
Less: Cumulative Series D preferred dividends	—	(776)
Net (loss) income attributable to common stockholders	$ (17,434)	$ 17,915
Net (loss) income per share:		
— Basic	$ (7.83)	$ 2.19
— Diluted	$ (7.83)	$ 2.17
Weighted-average number of common shares used in computing net (loss) income per share:		
— Basic	2,225,934	1,770,167
— Diluted	2,225,934	1,793,980

PALVELLA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED BALANCE SHEET INFORMATION
(in thousands)

	December 31, 2024	December 31, 2023
Assets		
Cash and cash equivalents	$ 83,602	$ 7,350
Other current assets	4,632	198
Total current assets	88,234	7,548
Total assets	$ 88,234	$ 7,548
Liabilities, Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Current liabilities	$ 12,038	$ 2,360
Non-current liabilities	13,589	9,068
Total liabilities	25,627	11,428
Total convertible preferred stock	—	70,603
Total stockholders' equity (deficit)	62,607	(74,483)
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	$ 88,234	$ 7,548

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First-in-disease therapies for patients with rare genetic skin diseases

Full Year 2024 Financial Results & Corporate Update
March 31, 2025

Forward Looking Statements

This presentation contains forward-looking statements of Palvella Therapeutics, Inc. (the Company") within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements include all statements that are not historical facts, and in some cases, can be identified by terms such as "may," "might," "will," "could," "would," "should," "expect," "intend," "plan," "objective," "anticipate," "believe," "estimate," "predict," "potential," "continue," "ongoing," or the negative of these terms, or other comparable terminology intended to identify statements about the future. Forward-looking statements contained in this presentation include, but are not limited to, statements regarding the Company's future financial or business performance, conditions, plans, prospects, trends or strategies and other financial and business matters the Company's current and prospective product candidates, the Company's planned research and development activities, the Company's planned clinical trials, including timing of receipt of data from the same, the planned regulatory framework for the Company's product candidates, the strength of the Company's intellectual property portfolio, and projections of the Company's future financial results and other metrics. Such forward-looking statements are subject to risks, uncertainties, and other factors which could cause actual results to differ materially from those expressed or implied by such forward looking statements.

These forward-looking statements are based upon current estimates and assumptions of the Company and its management 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. Factors that may cause actual results to differ materially from current expectations include, but are not limited to: competition, the ability of the company to grow and manage growth, maintain relationships with suppliers and retain its management and key employees; the success, cost and timing of the Company's product development activities, studies and clinical trials; changes in applicable laws or regulations; the possibility that the Company may be adversely affected by other economic, business or competitive factors; the Company's estimates of expenses and profitability; the evolution of the markets in which the Company competes; the ability of the Company to implement its strategic initiatives and continue to innovate its existing products; and the ability of the Company to defend its intellectual property.

Nothing in this Presentation should be regarded as a representation by any person that the forward-looking statements set forth herein will be achieved or that any of the contemplated results of such forward-looking statements will be achieved. You should not place undue reliance on forward-looking statements, which speak only as of the date they are made. The Company undertakes no duty to update these forward-looking statements.

Industry and Market Data

The Company may from time to time provide estimates, projections and other information concerning its industry, the general business environment, and the markets for certain conditions, including estimates regarding the potential size of those markets and the estimated incidence and prevalence of certain medical conditions. Information that is based on estimates, forecasts, projections, market research or similar methodologies is inherently subject to uncertainties, and actual events, circumstances or numbers, including actual disease prevalence rates and market size, may differ materially from the information reflected in this presentation. Unless otherwise expressly stated, we obtained this industry, business information, market data, prevalence information and other data from reports, research surveys, studies and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data, and similar sources, in some cases applying our own assumptions and analysis that may, in the future, prove not to have been accurate.

Trademarks

This Presentation may contain trademarks, service marks, trade names and copyrights of other companies, which are the property of their respective owners. Solely for convenience, some of the trademarks, service marks, trade names and copyrights referred to in this Presentation may be listed without the TM, SM © or ® symbols, but the Company will assert, to the fullest extent under applicable law, the rights of the applicable owners, if any, to these trademarks, service marks, trade names and copyrights.

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Vision: Build the leading therapeutics company focused on rare genetic skin diseases

Palvella debuted as a publicly listed company (NASDAQ:PVLA) in December 2024

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What Sets Palvella Apart

First-in-Disease Therapies

- Exclusively focused on developing transformational therapies for rare diseases with no FDA approved treatments

Rare Disease Expertise

- Team with expertise in rare disease drug development, including regulatory and patient interactions
- Proven track record building successful rare disease companies, including Insmed

Capital Efficiency

- Disciplined approach to operating business with our investors' capital top of mind

Late-stage Pipeline and Platform

- Lead product candidate, QTORIN™ rapamycin, in two ongoing studies: Phase 3 (microcystic LMs) and Phase 2 (cutaneous VMs)
- Versatile QTORIN™ platform with potential across rare diseases

Our Mission is to Serve Patients with Rare Diseases

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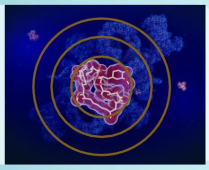
Our Breakthrough Innovation: QTORIN™ 3.9% Rapamycin Anhydrous Gel

OPTIMIZED CONCENTRATION

QTORIN synergistic solubility results in *3.9%* concentration

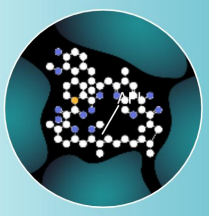


DERMAL ENGAGEMENT

rapamycin concentration in dermis exceeds IC90 for mTOR inhibition[1]

TOLERABILITY

no traditional penetration enhancers; limited systemic absorption[2]



Stable at room temperature for > 2 years

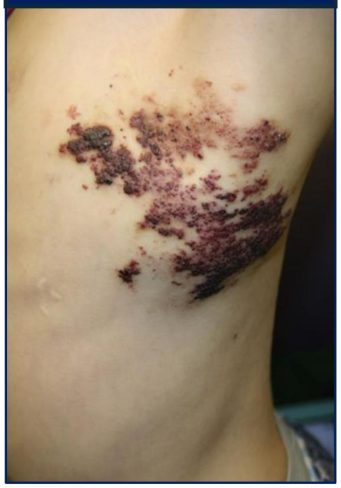
6 issued or pending U.S. patents through at least 2038

QTORIN™ 3.9% rapamycin anhydrous gel is for investigational use only and has not been approved or cleared by the FDA or by any other regulatory agency. The safety or efficacy has not been established for any use.

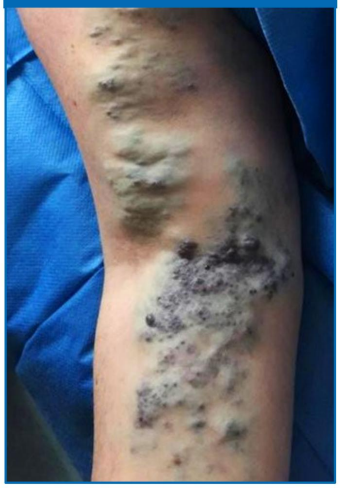
1. Data on file. 2.Clinical Study Report PALV-0609.

Multiple High-Impact Milestones Over Next 4 Quarters

Phase 3 SELVA data in microcystic LMs (Q1:26)



Phase 2 TOIVA data in cutaneous VMs (Q4:25)



Additional mTOR-driven indication for QTORIN™ Rapamycin (2H:25)

New QTORIN™ Program (2H:25)

QTORIN™ 3.9% rapamycin anhydrous gel is for investigational use only and has not been approved or cleared by the FDA or by any other regulatory agency. The safety or efficacy has not been established for any use.

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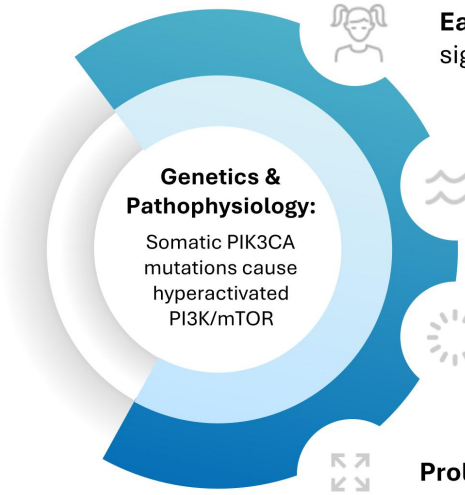
QTORIN™ 3.9% RAPAMYCIN

FOR

Microcystic Lymphatic Malformations

palvella THERAPEUTICS

Microcystic Lymphatic Malformations: *Serious, Debilitating, and Lifelong*



Early onset: Present at birth and significant impact to adolescents

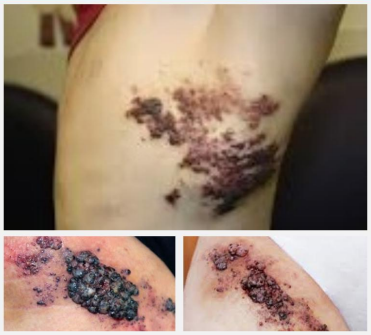
Lymphorrhea: Persistent discharge of lymphatic fluid through skin layers

Deep infections: Cellulitis and other serious infections

Proliferation of infiltrative lesions over time with no spontaneous resolution

> 30k patients

ESTIMATED DIAGNOSED IN THE US[1]



Leads to serious impact to quality of life and hospitalizations, with <u>no FDA approved therapies</u>

Current options: surgeries, sclerotherapy (chemotherapy injections), laser therapy, off label oral and topical mTOR inhibitors

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1. Primary prospective research conducted by Clarity Pharma and Sentero Pharma, claims analysis conducted by Trinity Life Sciences (June 2024). Includes microcystic LM and mixed LM (patients with both microcystic and macrocystic disease).

Phase 3 SELVA Trial in Microcystic LM: Enrollment On Track

n=40; single-arm, baseline-controlled, QD dose, age 3+[1]

Joyce Teng, MD, PhD
Principal Investigator
Stanford MEDICINE

13 Sites Currently Activated

- Children's Hospital of Philadelphia (Sally Cohen-Cutler, MD)
- UNC HEALTH (Elizabeth Nieman, MD)
- MAYO CLINIC (Megha Tollefson, MD)
- Texas Children's Hospital (Ionela Iacobas, MD)
- Cincinnati Children's (Kiersten Ricci, MD)
- Cleveland Clinic (Mike Kelly, MD, PhD)
- MINNESOTA CLINICAL STUDY CENTER (Steve Kempers, MD)
- PennState Health (Andrea Zaenglein, MD)
- VANDERBILT HEALTH (Neeraja Swaminathan, MD)
- Children's Healthcare of Atlanta (Michael White, MD)
- CHOC (Maria Buethe, MD, PhD)
- HEALTH UNIVERSITY OF UTAH (Amy Theos, MD)

Primary Efficacy

- mLM-IGA (Investigator Global Assessment), a 7-point clinician change scale

Key Secondary

- Blinded mLM Multi-Component Static Scale (mLM-MCSS)

Data anticipated Q1 2026

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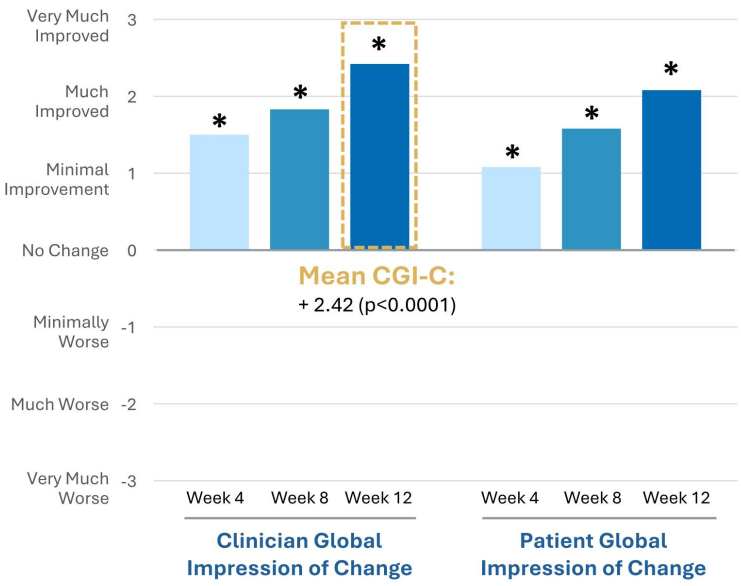
1. Patients ages 3-5 will be excluded from primary endpoint and will not be part of the n=40 database.

Phase 2: Clinically Meaningful, Statistically Significant Improvements

n=12; QD dose



Statistically significant across key clinician-assessed individual signs of microcystic LM at week 12

- Height (p<0.0001)
- Leaking (p<0.005)
- Bleeding (p<0.05)
- Erythema (p<0.005)
- Hyperkeratosis (p<0.005)

* = p-value <0.0001

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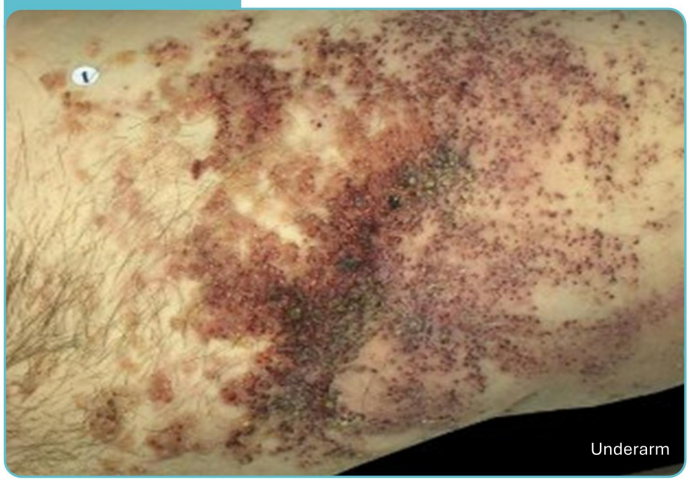
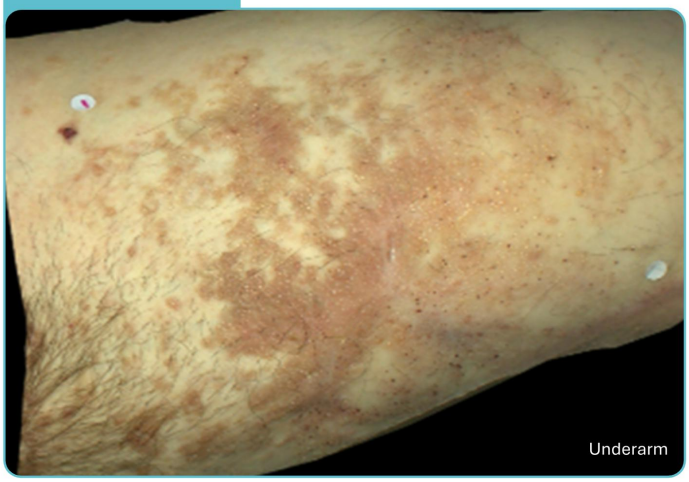
Phase 2 Results: Visible Improvement

Baseline



Week 12



Patient CGI-C: Very Much Improved (+3).

QTORIN™ 3.9% rapamycin anhydrous gel is for investigational use only and has not been approved or cleared by the FDA or by any other regulatory agency. The safety or efficacy has not been established for any use.

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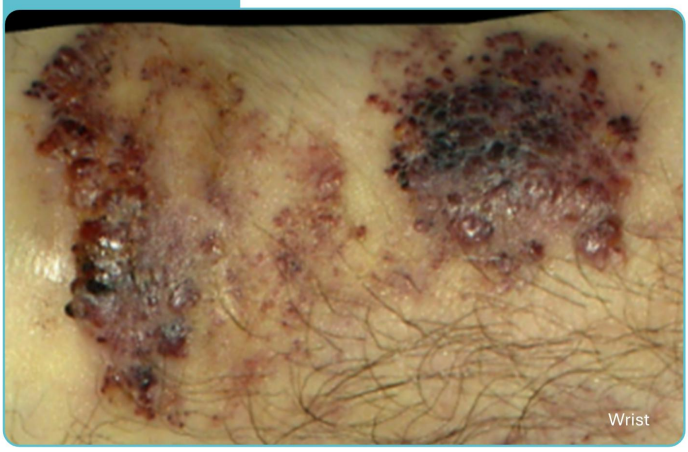
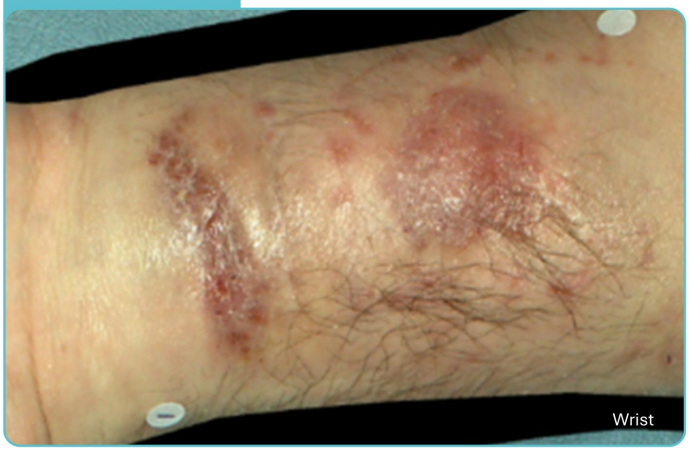
Phase 2 Results: Visible Improvement

Baseline



Week 12



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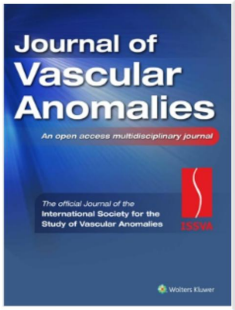
Phase 2 Study Results Published in *Journal of Vascular Anomalies (JoVA)*



Clinical Study (Prospective, Retrospective, Case Series)

OPEN

Journal of Vascular Anomalies
An open access multidisciplinary journal ISSVA

Phase 2 study of the safety and efficacy of QTORIN rapamycin in the treatment of microcystic lymphatic malformations

James Treat[a], Jeffrey Martini[b], Jason T. Connor[c], Alison Small[a], Tracy Funk[d], Milton Waner[e], Joyce Teng[f]

*"Efficacy from this phase 2 study showed a **robust clinical response** as measured from **both the clinicians' and patients' perspective** All 12 patients in the study demonstrated clinical and statistical improvements across a variety of endpoints, including **remarkable visual improvement in disease symptoms** from photographs of microcystic LM lesions. In addition, patient exit interviews that assessed baseline disease severity and changes in disease severity after treatment confirmed the results from this study."*

QTORIN™ 3.9% rapamycin anhydrous gel is for investigational use only and has not been approved or cleared by the FDA or by any other regulatory agency. The safety or efficacy has not been established for any use.

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Regulatory Overview: NDA Submission Planned for 2026[1]

Phase 3 study in microcystic LMs ongoing; data expected in Q1 2026

Seeking **full FDA approval** based on clinical endpoints utilized in prospective Phase 2 and Phase 3 studies

Leveraging **505(b)(2) pathway** and **real-world clinical evidence** of rapamycin

Rolling NDA submission with potential for six-month priority review planned for 2026

- Breakthrough Therapy Designation
- Fast Track Designation
- Orphan Drug Designation

1. NDA submission planned if Phase 3 study meets primary endpoint.

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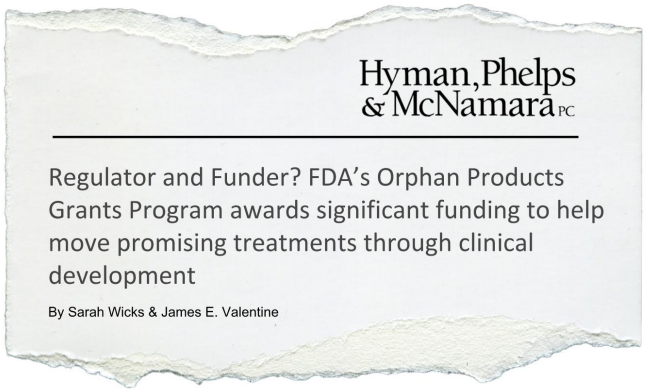
FDA Orphan Products Grant Recipient: Announced November 2024

Based on scientific and technical merit as determined by rare disease and regulatory experts

Out of 51 grant applications received by the FDA Orphan Products Grants Program in fiscal year 2024, Palvella's clinical trial was one of seven new clinical trials and only Phase 3 program that was awarded a grant (up to $2.6 million)



Hyman, Phelps & McNamara PC

Regulator and Funder? FDA's Orphan Products Grants Program awards significant funding to help move promising treatments through clinical development

By Sarah Wicks & James E. Valentine

"We would not expect clinical trials to be funded if there was not a meaningful degree of alignment between the FDA review division on the trial design, particularly for later stage trials. Receiving a Clinical Trials Grant provides insight that the FDA review team likely considered the proposed study as being capable of providing acceptable data that could contribute to product approval."

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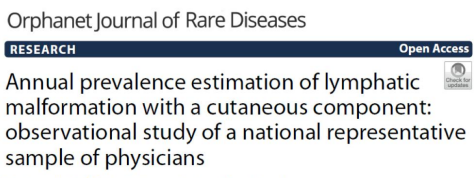
Microcystic LMs: Large U.S. Market Opportunity

MULTIPLE APPROACHES SUPPORT > 30K DIAGNOSED PATIENTS IN U.S.

1. Physician Survey
published 2022


Orphanet Journal of Rare Diseases

RESEARCH Open Access

Annual prevalence estimation of lymphatic malformation with a cutaneous component: observational study of a national representative sample of physicians

Jack Ray Gallagher[1*], J. Martini[2], S. Carroll[1], A. Small[3] and J. Teng[4]

*Estimated microcystic/mixed US prevalence**

79.9K

2. Claims Analysis
conducted 2024[1]

Study conducted in partnership with Trinity Life Sciences

*Estimated microcystic/mixed US prevalence**

38K

Additional claims analysis ongoing to support **commercial launch strategy** (annual incidence, patient concentration in centers of excellence)

Includes microcystic LM and mixed LM (patients with both microcystic and macrocystic manifestations)

1. Claims analysis conducted by Trinity Life Sciences (June 2024) based on a lookback period of 3 years.

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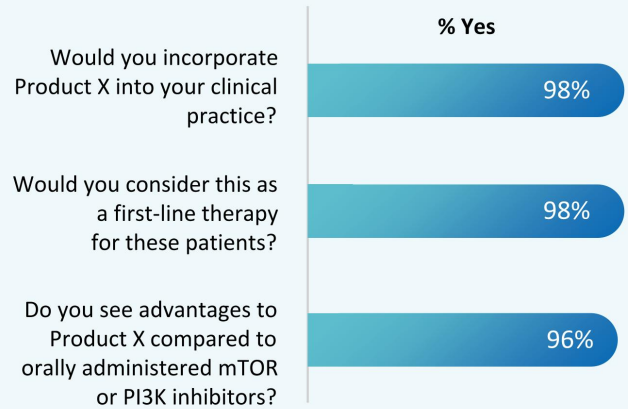
QTORIN™ Rapamycin:
Potential to be First Line, Standard of Care Therapy for Microcystic LMs

Current options: surgeries, sclerotherapy (chemotherapy injections), laser therapy, off label oral and topical mTOR inhibitors

Product X: topical 3.9% rapamycin gel



Percent of my microcystic LM patients I would prescribe Product X:

75%

Survey of 52 high-volume dermatologists and hematologists (average of 11.7 cutaneous microcystic lymphatic malformation patients)

Source: Medacorp.

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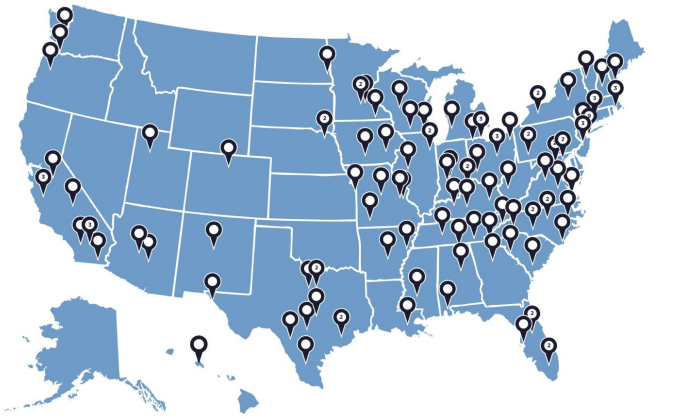
Streamlined and Efficient Commercial Strategy Targeting Concentrated Centers of Excellence (CoEs)

- 142 established Vascular Anomaly Centers across the U.S.
- Ideal for self-commercialization with focused sales force and medical affairs teams
- Second indication (cutaneous VMs) treated at same CoEs – able to leverage synergies with Microcystic LMs

Distribution of Vascular Anomaly Centers in the U.S.



Source: Cohen-Cutler et al., *The Journal of Pediatrics,* (2023).

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QTORIN™ 3.9% RAPAMYCIN

FOR

Cutaneous Venous Malformations

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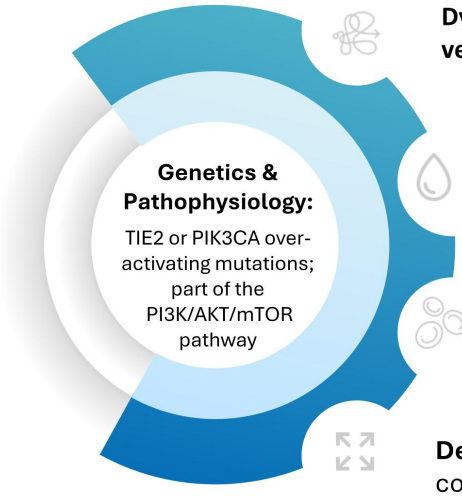
Cutaneous Venous Malformations: *Serious, High Unmet Need*



Dysregulated growth of malformed veins impacting the skin

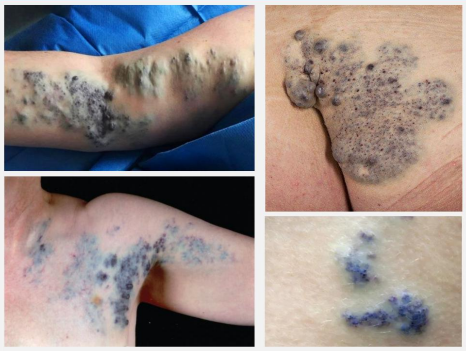
Hemorrhaging

Functional impairment

Deformity as lesions continue to grow

> 75k patients

ESTIMATED DIAGNOSED IN THE US[1]



Leads to physical & functional impairment, psychological distress, with <u>no FDA approved therapies</u>

Current options: laser treatment, off label systemic pharmacotherapies limited by toxicities

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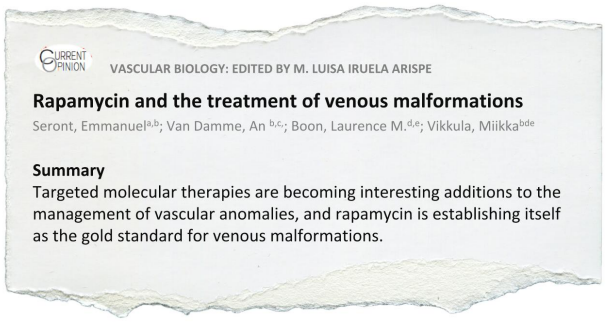
1. Primary prospective research conducted by Clarity Pharma.

Real World Clinical Evidence Supporting Rapamycin's Potential in VM Led to *FDA Fast Track Designation* for QTORIN™ Rapamycin



CURRENT OPINION VASCULAR BIOLOGY: EDITED BY M. LUISA IRUELA ARISPE

Rapamycin and the treatment of venous malformations

Seront, Emmanuel[a,b]; Van Damme, An [b,c]; Boon, Laurence M.[d,e]; Vikkula, Miikka[bde]

Summary

Targeted molecular therapies are becoming interesting additions to the management of vascular anomalies, and rapamycin is establishing itself as the gold standard for venous malformations.

Summary Takeaways

1. **Rapamycin recognized as a targeted molecular therapy for venous malformations**

   "Rapamycin is the first targeted therapy that improves considerably the QoL of these patients"

2. **Need for topical therapies**

   "Topical agents…could abolish the need for systemic treatments that have wider toxicity"

Current unmet need for targeted, localized therapy for Cutaneous Venous Malformations

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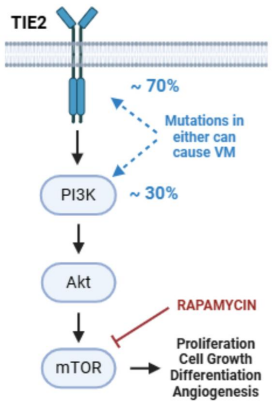
Data Supporting Rapamycin Use in TIE2 and PIK3CA Mutations

VENOUS MALFOMATION

Pathophysiology



Queisser 2021

TIE2 VENOUS MALFORMATION MODEL

Preclinical Evidence

The Journal of Clinical Investigation RESEARCH ARTICLE

Rapamycin improves TIE2-mutated venous malformation in murine model and human subjects

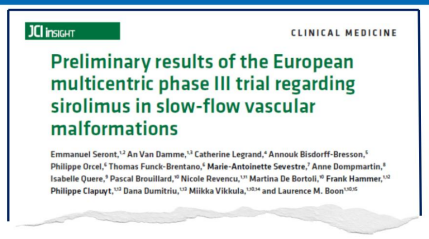
Elisa Boscolo, Nisha Limaye, Lan Huang, Kyu-Tae Kang, Julie Soblet, Melanie Uebelhoer, Antonella Mendola, Marjut Natynki, Emmanuel Seront, Sophie Dupont, Jennifer Hammer, Catherine Legrand, Carlo Brugnara, Lauri Eklund, Miikka Vikkula, Joyce Bischoff, and Laurence M. Boon

*"Efficacy of rapamycin in this model and in patients in our clinical pilot study suggests **rapamycin as the first molecular therapy for VMs.**"*

Boscolo 2015

ORAL RAPAMYCIN

VASE Clinical Study



JCI insight CLINICAL MEDICINE

Preliminary results of the European multicentric phase III trial regarding sirolimus in slow-flow vascular malformations

Emmanuel Seront,[1,2] An Van Damme,[1,3] Catherine Legrand,[4] Annouk Bisdorff-Bresson,[5] Philippe Orcel,[6] Thomas Funck-Brentano,[4] Marie-Antoinette Sevestre,[7] Anne Dompmartin,[8] Isabelle Quere,[9] Pascal Brouillard,[10] Nicole Revencu,[11] Martina De Bortoli,[10] Frank Hammer,[12] Philippe Clapuyt,[13] Dana Dumitriu,[13] Miikka Vikkula,[1,10,14] and Laurence M. Boon[1,10,15]

*"A sustained benefit of sirolimus was observed in **84% of TIE2-mutated** and **83% of the PIK3CA-mutated** patients."*

Seront 2023

20+ published papers support rapamycin's clinical benefit in Venous Malformations[(1)]

1. Data on file.

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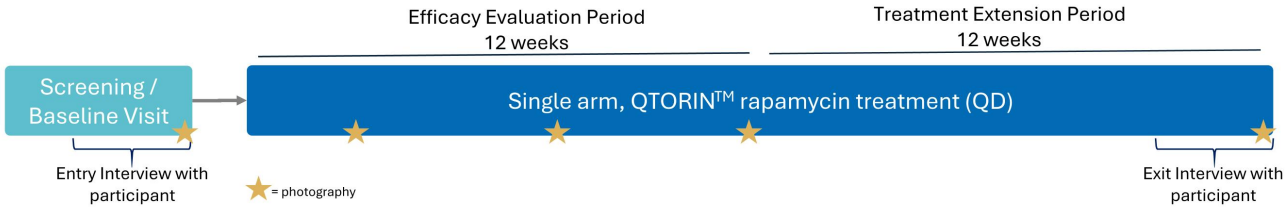
Cutaneous Venous Malformations Phase 2 TOIVA Study

n=~15; QD dose

Study objectives: evaluate safety and tolerability (incl. determining systemic concentration of rapamycin) and evaluate efficacy across multiple endpoints (no statistical hierarchy)



Safety

- Safety and tolerability

Efficacy

- Cutaneous venous malformation – investigators' global assessment (7-point clinician change scale)
- Cutaneous venous malformation - multicomponent static scale
- Other clinician and patient-reported outcomes

Data anticipated Q4 2025

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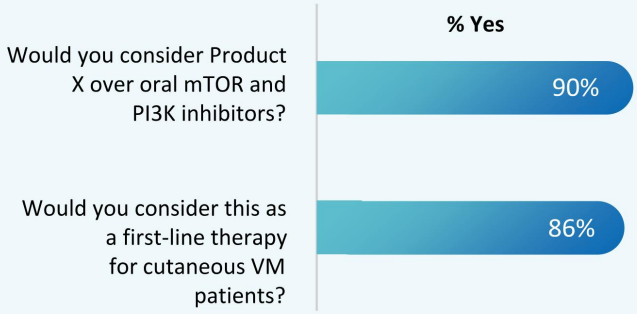
Market Research in Cutaneous VMs (Sept 2024): Strongly indicates QTORIN™ rapamycin's potential as first line therapy

Product X: topical 3.9% rapamycin gel

Current options: laser treatment, off label systemic pharmacotherapies limited by toxicities



"This product would be life changing for current patients with limited treatment options"

"Essentially all patients with VMs would be considered for Product X"

Survey of 50 high-volume dermatologists and hematologists with an average of 10.6 cutaneous VM patients seen per month

Source: Medacorp.

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Financial Overview

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2024 Financial Highlights and 2025 Outlook

$83.6 million
Cash at 12/31/2024

2+ years
Runway into 2H 2027

$14.1 million
R&D + G&A spend in 2024

>$55 million
Projected cash at year end

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Q&A

Striving to be first for rare disease patients

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