palvella
THERAPEUTICS

First-in-disease therapies for patients with rare diseases

Phase 3 SELVA Topline Data in Microcystic Lymphatic Malformations
February 24, 2026

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 expected timing of the presentation of data from ongoing clinical trials, including the SELVA study, Palvella's marketing application plans, the potential clinical and safety benefits of QTORIN™ rapamycin and its potential to become a standard of care, the potential benefits of Palvella's designations for QTORIN™ rapamycin, including Breakthrough Therapy, Fast Track, and Orphan Drug, the potential for orphan drug exclusivity if approved, 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 and any additional indications or platform 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 Company's plan to meet with regulatory authorities, 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.

palvella THERAPEUTICS

Phase 3 SELVA Topline Data: Today's Presenters

Wes Kaupinen
Founder and CEO

Jeff Martini, PhD
Chief Scientific Officer

Matt Korenberg
Chief Financial Officer

Bohan Wei
VP Corporate Development & New Product Planning

Michael Kelly, MD, PhD

Cleveland Clinic

- Pediatric hematologist-oncologist at Cleveland Clinic
- Executive Director of Lymphangiomatosis & Gorham's Disease Alliance (LGDA)
- Study investigator in Phase 3 SELVA study
- Member of Palvella Medical and Scientific Advisory Board
- Consultant to Palvella

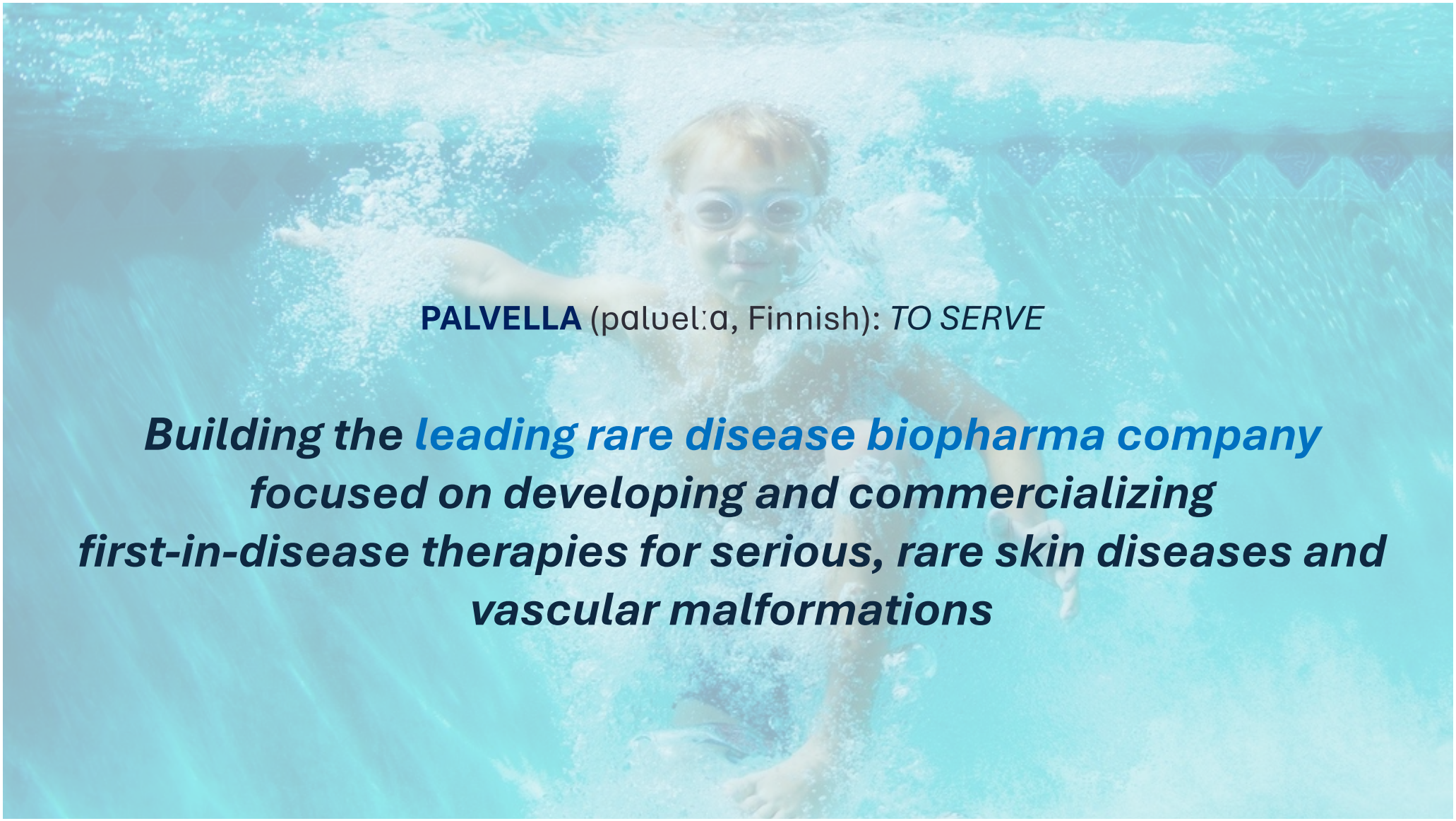
palvella THERAPEUTICS

QTORIN™ Rapamycin for
Microcystic Lymphatic Malformations

Opening Remarks

Wes Kaupinen
Founder and CEO

palvella
THERAPEUTICS



PALVELLA (pɑlʋelːɑ, Finnish): *TO SERVE*

Building the leading rare disease biopharma company focused on developing and commercializing first-in-disease therapies for serious, rare skin diseases and vascular malformations

selva : A Clear Path Forward for Patients

QTORIN™ Rapamycin at Week 24:

Highly statistically significant across primary, key secondary, and all four secondary endpoints (all p<0.001)

+2.13 Mean improvement on mLM-IGA primary endpoint (p<0.001)

Participants aged ≥ 6 who completed the efficacy evaluation period:

95% Improved on mLM-IGA

86% "Much Improved" (+2) or "Very Much Improved" (+3) on mLM-IGA

Palvella plans to advance quickly towards NDA filing, with potential approval in 1H:2027

mLM-IGA = Microcystic Lymphatic Malformation Investigator Global Assessment. Note: Data analyzed per statistical analysis plan; non-completer data imputed per statistical analysis plan for primary endpoint; endpoints tested according to pre-specified hierarchical testing procedure; statistical significance (p<0.05).

palvella THERAPEUTICS

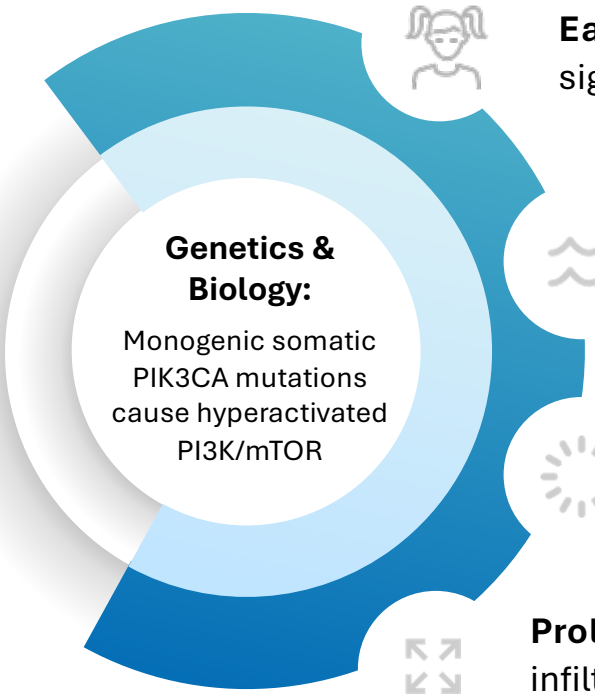
QTORIN™ Rapamycin for
Microcystic Lymphatic Malformations

Phase 3 SELVA Topline Results

Dr. Jeff Martini
Chief Scientific Officer

palvella
THERAPEUTICS

Microcystic Lymphatic Malformations:
Serious, Debilitating, and Lifelong



Early onset: Present at birth and significant impact to adolescents

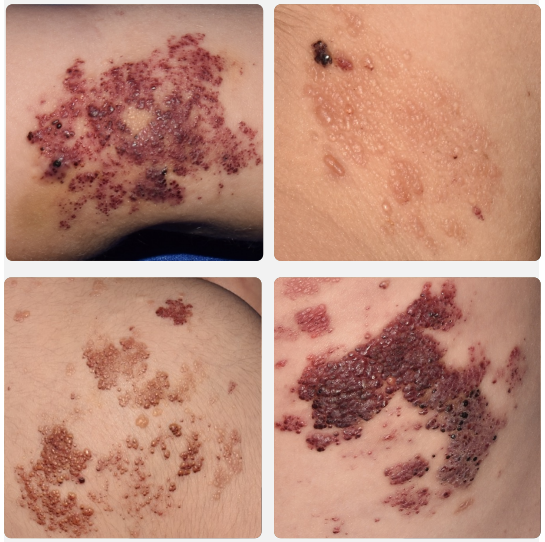
Lymphorrhea: Persistent discharge of lymphatic fluid through skin layers

Deep infections: Recurrent cellulitis and serious soft tissue infections, resulting in hospitalizations

Proliferative, progressive disease with infiltrative lesions and no spontaneous regression

> 30k patients
ESTIMATED DIAGNOSED IN THE U.S.[1]

No FDA-approved therapies



Breakthrough Therapy Designation

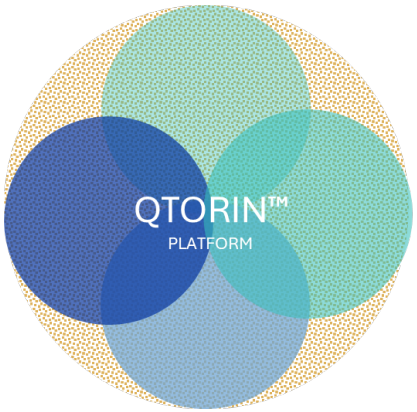
Fast Track Designation

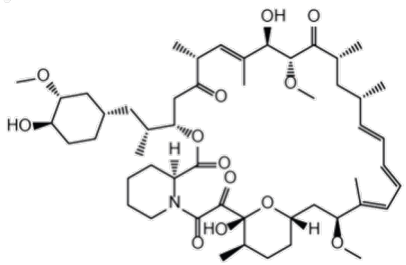
Orphan Drug Designation

palvella THERAPEUTICS

1. Incidence, prevalence, and care for patients with lymphatic malformations (LMs) in the U.S.: A claims-based analysis, Society of Investigative Dermatology, (2025) and Gallagher et al, *Orphanet Journal of Rare Diseases*, (2022). Includes microcystic LM and mixed LM (patients with both microcystic and macrocystic disease).

QTORIN™: Targeted mTOR Inhibition with Dermal Target Engagement





QTORIN™ 3.9% Rapamycin Anhydrous Gel

3.9% concentration enabled through synergistic multi-excipient anhydrous solubilization system

Single-phase gel maintaining dissolved rapamycin without crystallization or suspension

Elevated thermodynamic activity drives rapamycin vehicle release and skin bioavailability

Deep dermal penetration & sustained dermal retention for prolonged inhibition of hyperactivated mTOR signaling

Adherence-optimized vehicle designed for chronic dosing in pediatric and adult populations

palvella THERAPEUTICS

selva: QTORIN™ Rapamycin Exceeded Upside Case Profile

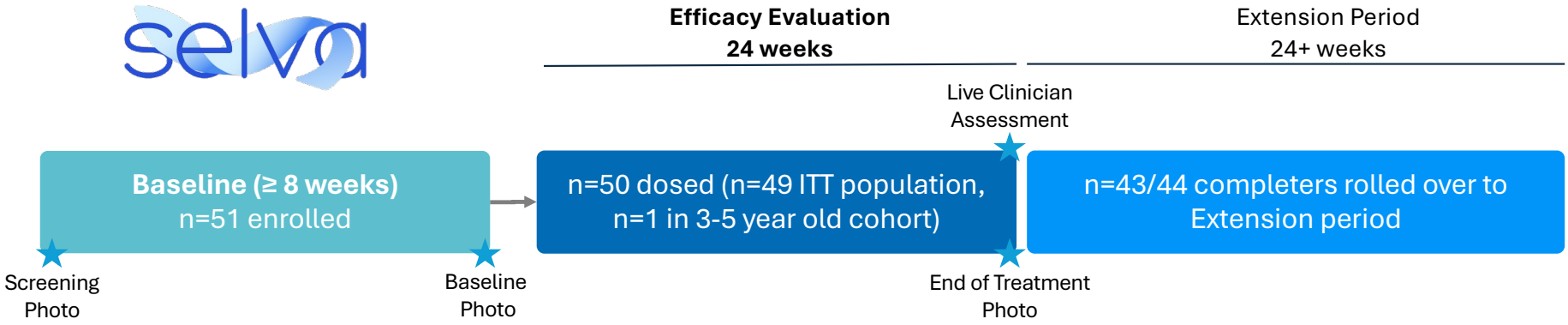
Upside Target Clinical Profile	SELVA Outcome
Statistically significant primary endpoint with mean mLM-IGA of ≥+1.5 at Week 24	☑ **Highly statistically significant with mean mLM-IGA of +2.13 at Week 24 (p<0.001)**
Statistical significance on independent, blinded key secondary endpoint	☑ Highly statistically significant (p<0.001), including on all three clinical signs: vesicle appearance, height, leaking/bleeding
Rollover into extension period in line with best-in-class drugs for rare diseases	☑ 98% of Week 24 completers (43/44) rolled over to Extension period
Safety profile: well-tolerated and similar to previous clinical trials	☑ Well-tolerated across both adult and pediatric patients, supporting chronic administration

Note: Data analyzed per statistical analysis plan; non-completer data handled via multiple imputation per statistical analysis plan; endpoints tested sequentially according to pre-specified hierarchical testing; statistical significance (p<0.05). Week 24 completers included 43 participants ≥6 years old and 1 participant 3-5 years old.

palvella THERAPEUTICS

Phase 3 SELVA Trial Design

Single-arm, baseline-controlled, QD dose



Primary Endpoint: mLM-IGA (Investigator Global Assessment)

Key Secondary Endpoint: Blinded mLM Multi-Component Static Scale (mLM-MCSS): independent, blinded review of randomized Baseline and Week 24 photos evaluating three key signs of disease: Vesicle Appearance, Height, Leaking/Bleeding

Secondary Endpoints: Live mLM-MCSS, Patient Global Impression of Change (PGI-C), Clinician and Patient Global Impression of Severity (CGI-S, PGI-S), and Incidence and Severity of Adverse Events

Supported by FDA Orphan Products Grant:

Two tranches of non-dilutive funding received (most recent in Oct '25)

Note: Of the 51 participants enrolled, 50 initiated treatment, including 49 aged ≥6 years (ITT population). During the 24-week efficacy evaluation period, six participants discontinued treatment: four participants discontinued for reasons unrelated to adverse events, one participant discontinued due to an adverse event not related to study drug, and one participant discontinued due to an adverse event possibly related to study drug.

palvella THERAPEUTICS

Phase 3 SELVA: Baseline Characteristics

	ITT Population (n=49)[1]
Age, Mean [Range]	19.4 [6-57]
Sex M:F	24:25
Prior Medical Interventions for mLM[2]	34 (69%)
Laser	17 (35%)
Sclerotherapy	14 (29%)
Surgery	13 (27%)
Topical Sirolimus [Rapamycin]	13 (27%)
Oral Sirolimus [Rapamycin]	2 (4%)

1. 51 participants entered Baseline period; 50 dosed with QTORIN™ rapamycin (49 participants ≥6 years old = ITT population; 1 participant 3-5 years old included in safety database). 2. Percentages may not sum to 100% due to some patients having more than one intervention.

palvella THERAPEUTICS

SELVA: Primary and Key Secondary Endpoints Achieved

	Mean Change at Week 24 (95% CI)	**p-value**
Primary Endpoint: Microcystic Lymphatic Malformation Investigator Global Assessment (mLM-IGA)*	**+2.13** (1.88, 2.38)	p<0.001
Key Secondary Endpoint: Blinded Microcystic Lymphatic Malformation Multi-Component Static Scale**	**-3.36** (-4.34, -2.38)	p<0.001

*Dynamic change scales (7-point scales ranging from "Very Much Worse" (-3) to "Very Much Improved" (+3); positive values indicate improvements from baseline)
**mLM-MCSS (Sum of three static severity scales: Height, Leaking/Bleeding, Vesicle Appearance: Each scale rated "Clear or Almost Clear" (1) to "Very Severe" (5); total score 3-15. Test baseline to Week 24 change; negative values indicate improvements from baseline)

Note: Data analyzed per statistical analysis plan; non-completer data imputed per statistical analysis plan for primary endpoint; endpoints tested according to pre-specified hierarchical testing procedure; statistical significance (p<0.05).

palvella THERAPEUTICS

SELVA: All Additional Secondary Endpoints Achieved

	Mean Change at Week 24 (95% CI)	**p-value**
Patient Global Impression of Change*	**+1.9** (1.66, 2.16)	p<0.001
Live mLM-MCSS**	**-4.6** (-5.20, -3.92)	p<0.001
Clinician Global Impression of Severity***	**-1.7** (-1.91, -1.39)	p<0.001
Patient Global Impression of Severity***	**-1.0** (-1.26, -0.74)	p<0.001

*Dynamic change scales (7-point scales ranging from "Very Much Worse" (-3) to "Very Much Improved" (+3); positive values indicate improvements from baseline)
**mLM-MCSS (Sum of three static severity scales: Height, Leaking/Bleeding, Vesicle Appearance: Each scale rated "Clear or Almost Clear" (1) to "Very Severe" (5); total score 3-15. Test baseline to Week 24 change; negative values indicate improvements from baseline)
***Static severity scales (5-point scales ranging from 1 to 5; negative values indicate improvements from baseline)

Note: Data analyzed per statistical analysis plan; non-completer data imputed per statistical analysis plan for primary endpoint; endpoints tested according to pre-specified hierarchical testing procedure; statistical significance (p<0.05).

palvella THERAPEUTICS

Phase 3 Results: Well-Tolerated and Favorable Safety Profile

	Number of Participants (%)
Any Treatment-Emergent Adverse Event	35 (70%)
Severe (not related to study drug)	1 (2%)
Serious (not related to study drug)	4 (8%)
Any Treatment-Related[1] Adverse Event	17 (34%)
Severe	0 (0%)
Serious	0 (0%)
Treatment-Related[1] AEs with ≥ 5% Incidence	
Application site acne	3 (6%)
Application site discoloration	3 (6%)
Application site pruritus	3 (6%)
Possibly Treatment-Related AE Leading to Discontinuation	1 (2%)

Rapamycin levels were below 2 ng/mL[2] in systemic circulation on for all participants at all timepoints in the study

palvella THERAPEUTICS

Note: n=50 participants in efficacy evaluation evaluated for safety. 1. Denotes related or possibly related. 2. Validated assay of lower limit of quantification (100 pg/mL).

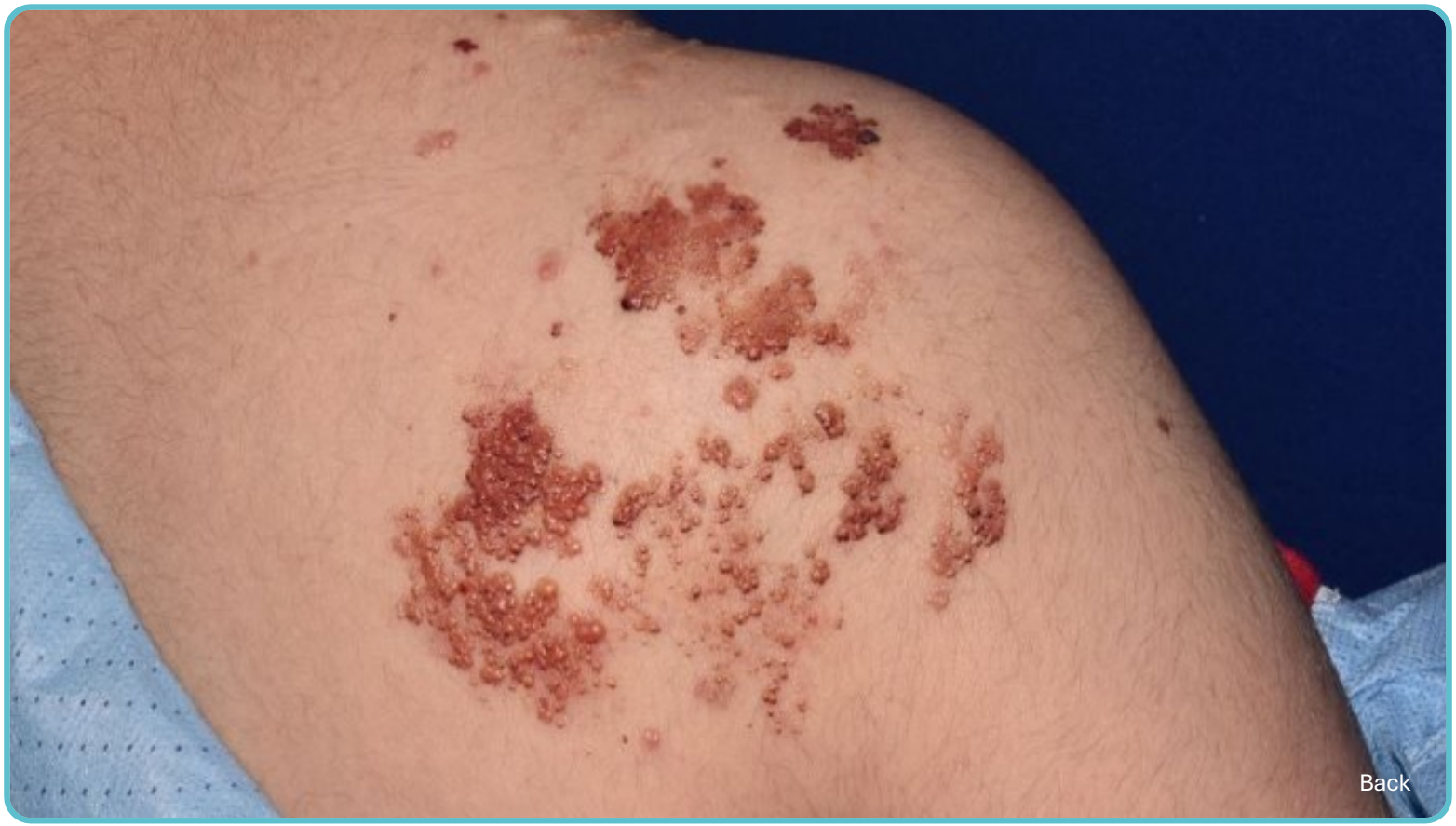
QTORIN™ Rapamycin for
Microcystic Lymphatic Malformations

KOL Perspective

Dr. Michael Kelly
Cleveland Clinic

palvella
THERAPEUTICS

Phase 3 Results: Age 10, Female

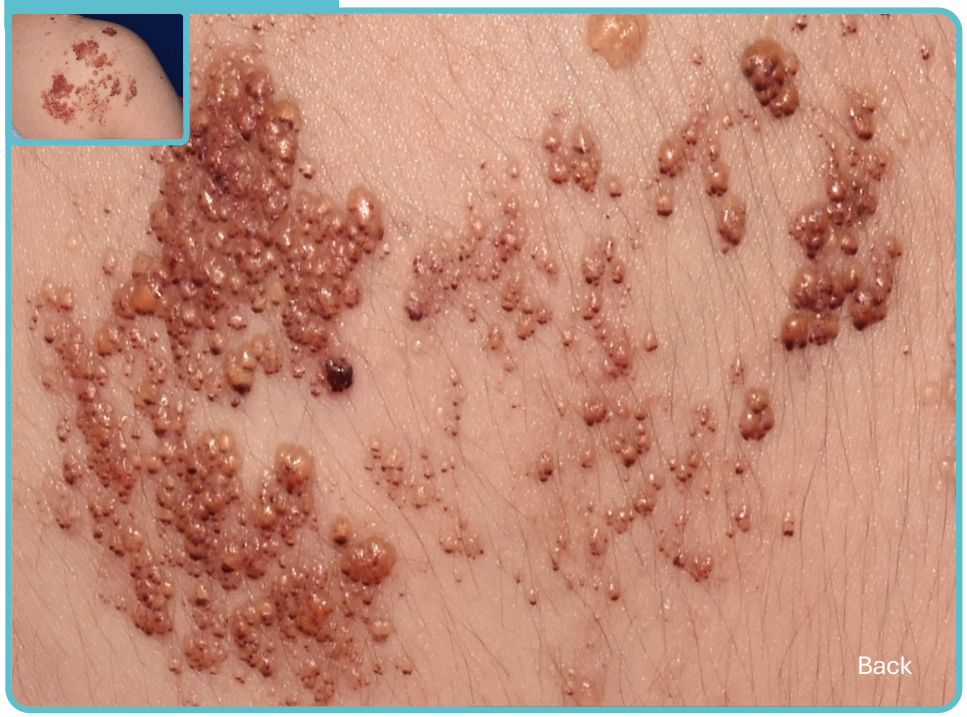


Note: Selective photos.

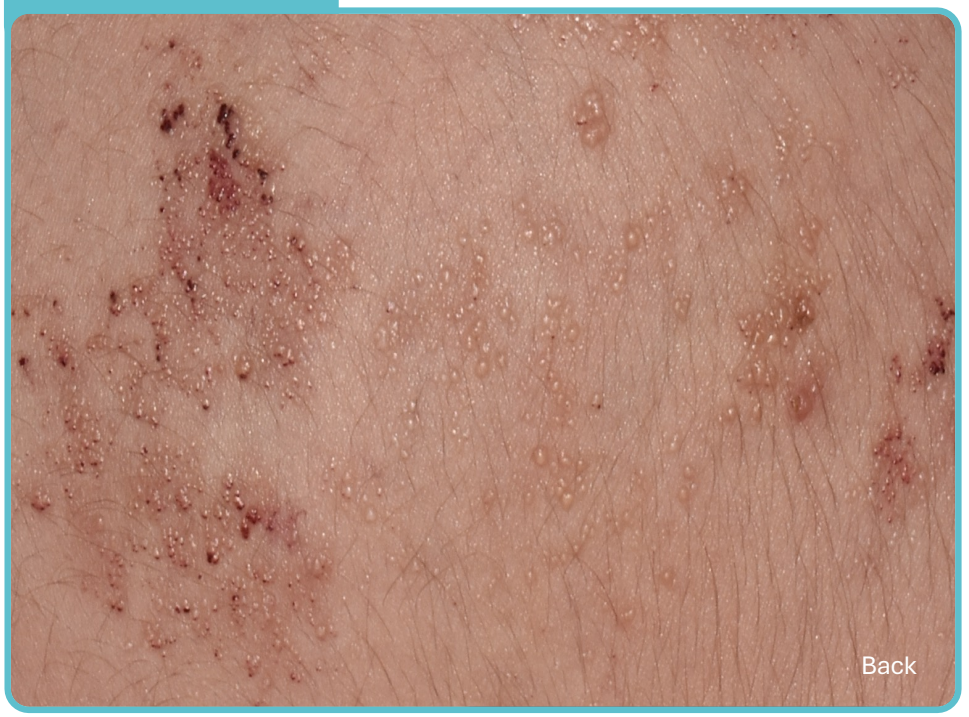
palvella THERAPEUTICS

Phase 3 Results: Age 10, Female, mLM-IGA: +2 “Much Improved”

Baseline



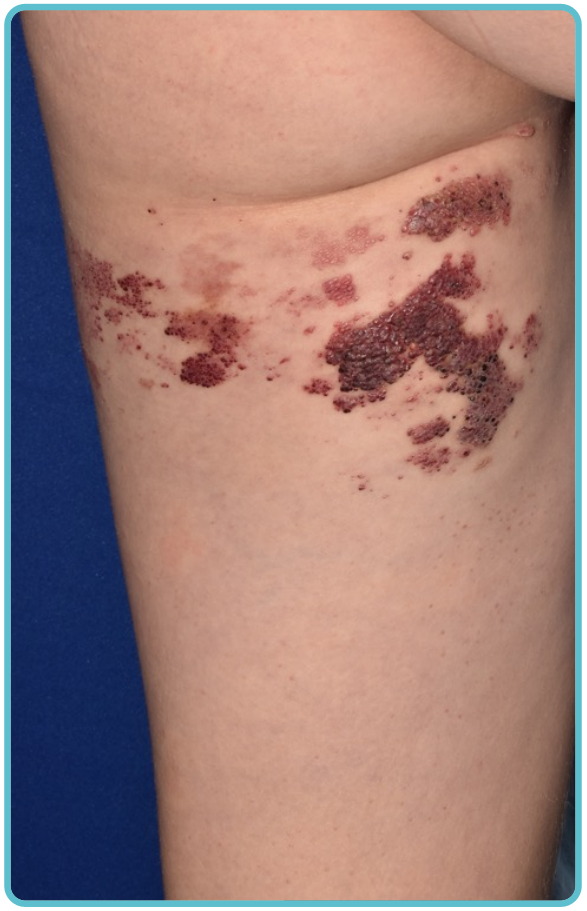
Week 24



Note: Selective photos.

palvella THERAPEUTICS

Phase 3 Results: Age 10, Male

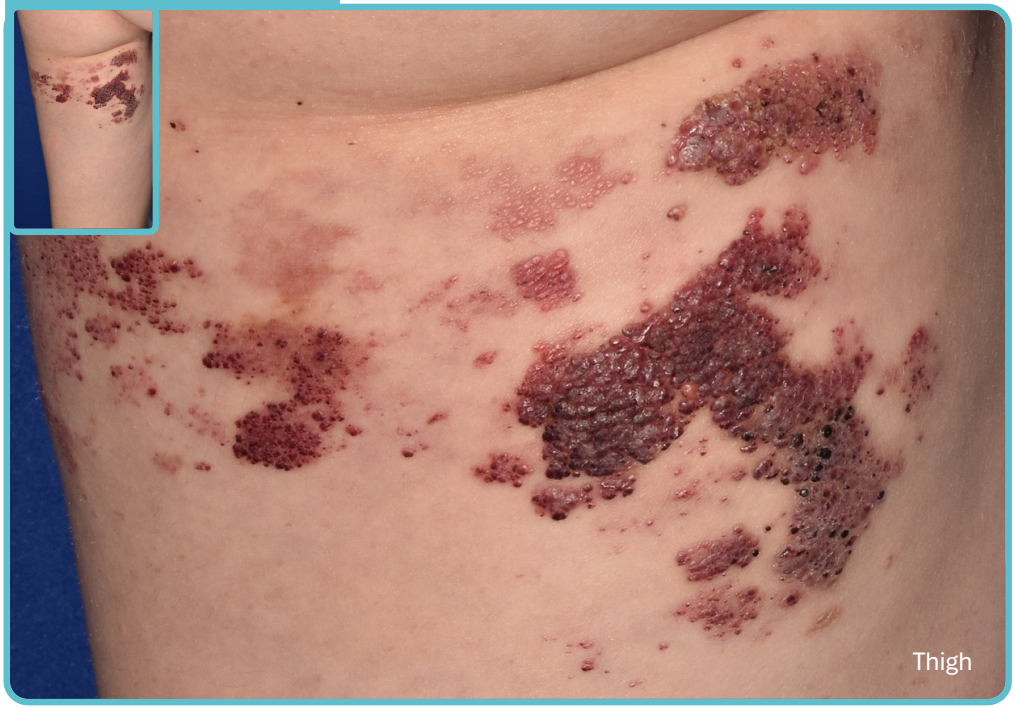


Note: Selective photos.

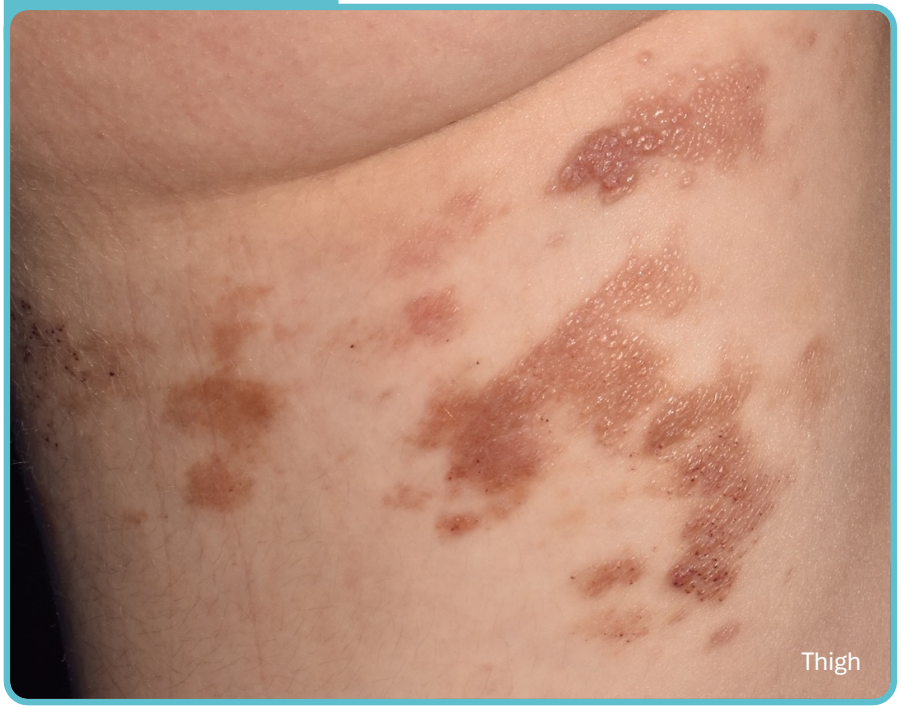
palvella THERAPEUTICS

Phase 3 Results: Age 10, Male, mLM-IGA: +2 “Much Improved”

Baseline



Week 24

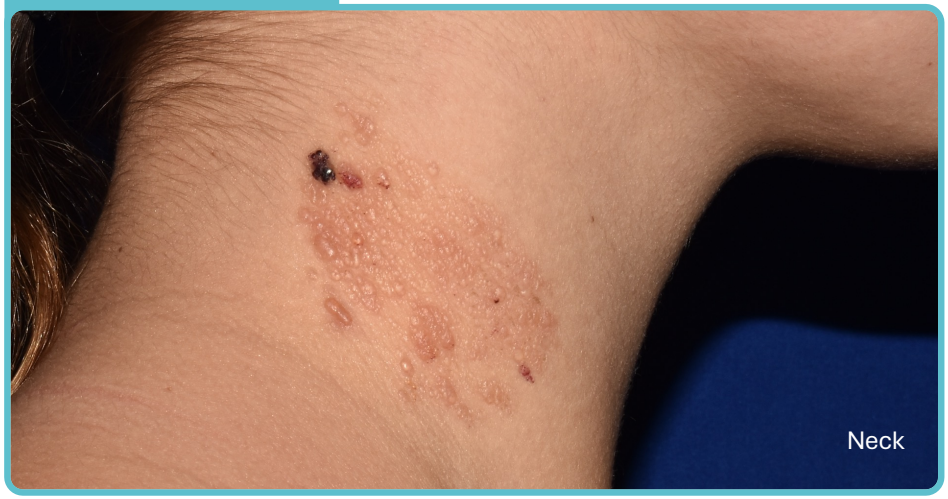


Note: Selective photos.

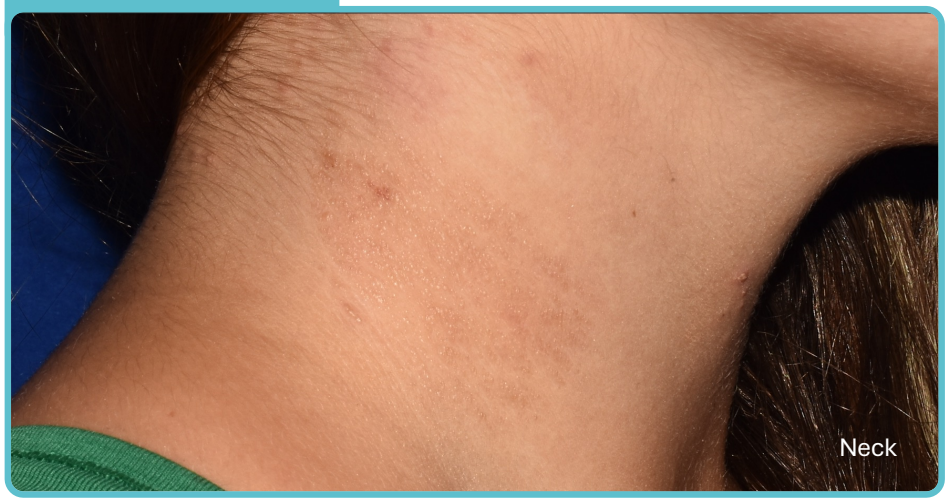
palvella THERAPEUTICS

Phase 3 Results: Age 14, Female, mLM-IGA: +3 “Very Much Improved”

Baseline



Week 24

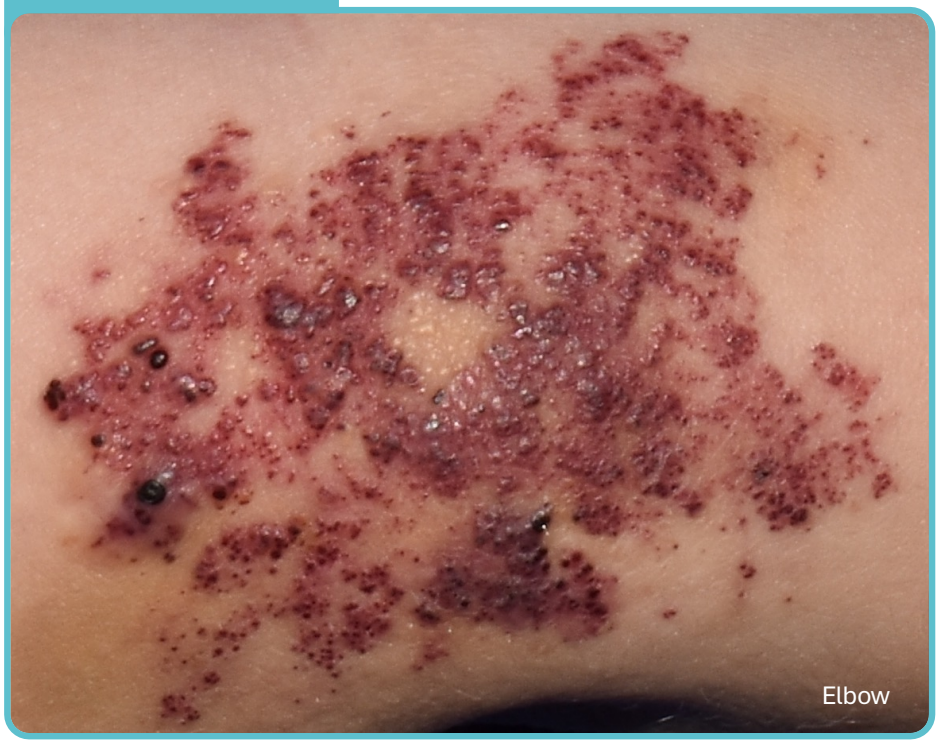


Note: Selective photos.

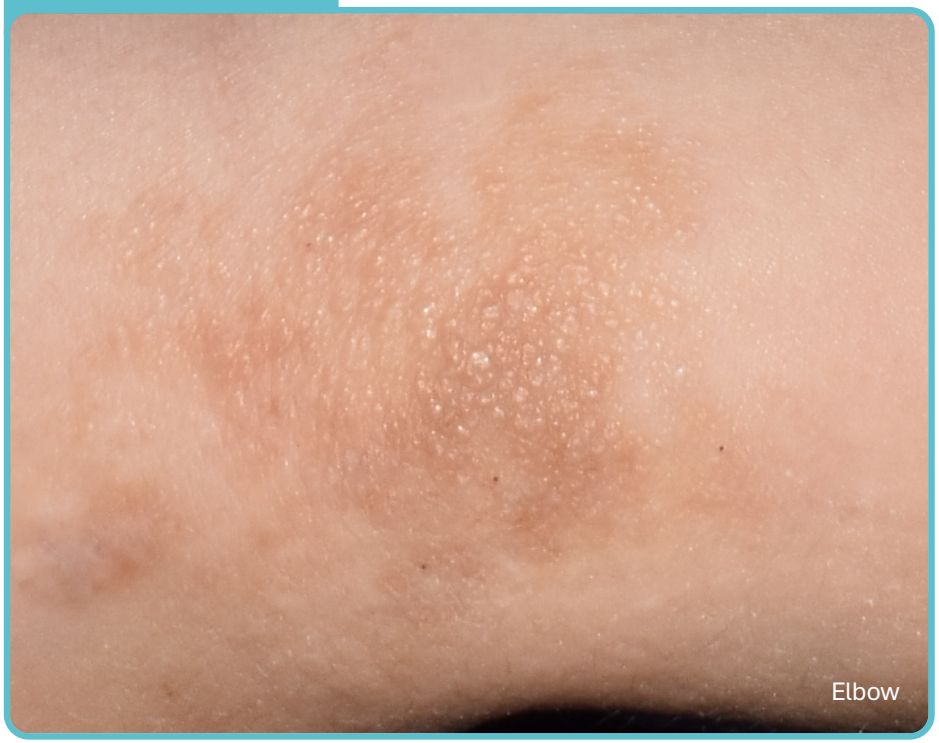
palvella THERAPEUTICS

Phase 3 Results: Age 7, Female, mLM-IGA: +3 “Very Much Improved”

Baseline



Week 24



Note: Selective photos.

palvella THERAPEUTICS

QTORIN™ Rapamycin Has the Potential to be First-Line and Standard of Care in Microcystic Lymphatic Malformations

QTORIN™ 3.9% Rapamycin

- Targets underlying pathobiology
- Large magnitude treatment effect in two prospective trials
- Favorable safety profile allowing for chronic therapy

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.

palvella THERAPEUTICS

QTORIN™ Rapamycin for
Microcystic Lymphatic Malformations

Summary

Wes Kaupinen
Founder and CEO

palvella
THERAPEUTICS

Palvella: Leading the Way in Addressing Rare Skin Diseases and Vascular Malformations

1

Planned NDA submission in 2H:2026

2

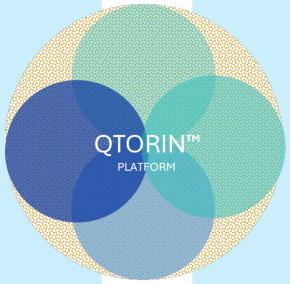
Potential first FDA-approved therapy in mLM; U.S. launch prep accelerating

3

$1-$3 billion U.S. peak sales potential (mLM + cVM)

4

Pipeline-in-a-product: Broad potential to address mTOR-driven diseases



5

Platform: QTORIN™ pitavastatin and new QTORIN™ program (2H:2026)

palvella THERAPEUTICS

Thank You

Striving to be first for rare disease patients

palvella
THERAPEUTICS