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First-in-disease therapies for patients with rare diseases



Corporate Presentation
September 2026

Forward Looking Statements

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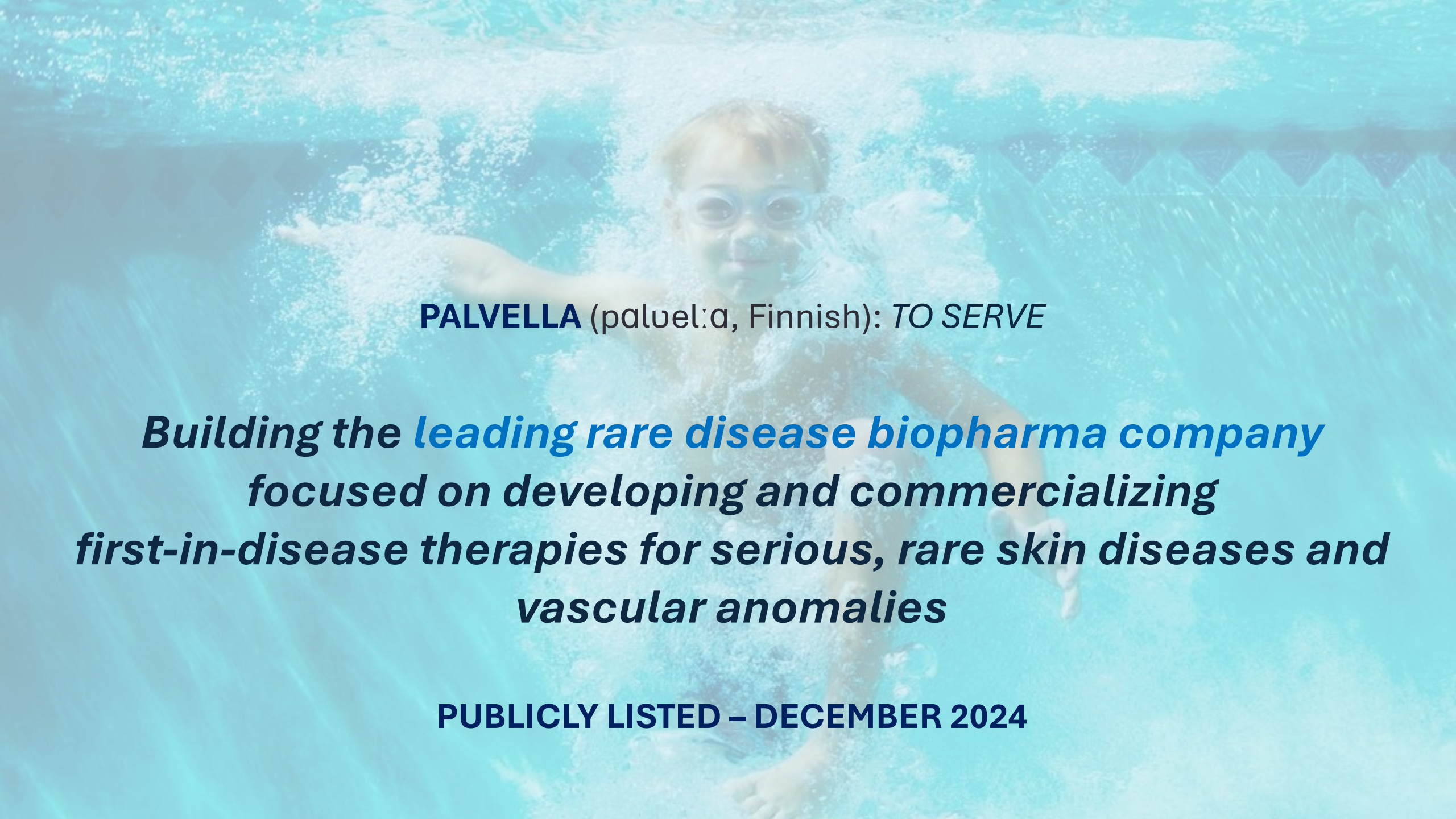
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PALVELLA (palʋelːa, Finnish): *TO SERVE*

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

PUBLICLY LISTED – DECEMBER 2024

What Makes Palvella Stand Apart

Veteran team executing rare disease model designed to reduce time and capital to FDA approval

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Repeatably unlocking multi-billion dollar market opportunities in previously untreated orphan diseases

1. **First-in-Disease Focus**
+
2. **Rare Diseases with Clear Disease Biology**
+
3. **Leveraging Existing Human Proof-of-Concept and Safety Data**
+
4. **Innovative QTORIN™ Platform: Durable IP Generation**

Palvella's Focus:
High Unmet Need, Low Competitive Intensity Corridors of the Orphan Universe

679
Rare Skin Diseases and Vascular Anomalies[1]

Often serious and chronically debilitating

Approximately
98%
Have **No** Approved Therapies[1]

Lower industry innovation and investment compared to other therapeutic areas (e.g., oncology, neurology, pulmonary)



"We have begun to see interest from investors and companies in developing treatments for a rare disease such as epidermolysis bullosa, but there are many other diseases within dermatology that remain unaddressed"

John Doux, M.D., Barriers and Opportunities Across the Development Divide,
The Society of Investigative Dermatology, 2015

Note: John Doux, M.D. is a member of the Palvella Board of Directors. 1. Licholai G., Rare Dermatology Orphan Disease Drug Development Opportunities & Challenges, Presented at Dermatology Summit, Jan 2019. Updated 2026 to include rare vascular anomalies (ISSVA 2025) and approvals through 2026.

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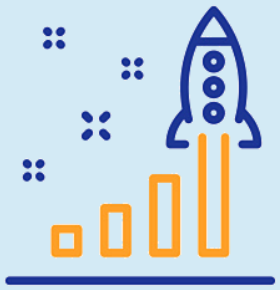
Palvella: Driving the Next Wave of Innovation in Under-Appreciated Rare Skin Diseases and Vascular Anomalies

	Microcystic LMs	Cutaneous VMs	Clinically Significant Angiokeratomas	Disseminated Superficial Actinic Porokeratosis
Diagnosed **US Prevalence** (Estimated)[1]	30k+	75k+	50k+	50k+
Number of **FDA Approved** Therapies	None			
% Physicians Who Would Consider QTORIN™ as **First-line Therapy** (Market Research)[2]	> 80%			

1. Clarity Pharma, Trinity Life Sciences, MedaCorp, and ZS Associates. 2. MedaCorp market research.

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What's New at Palvella – Six Rare Disease Programs by YE 2026



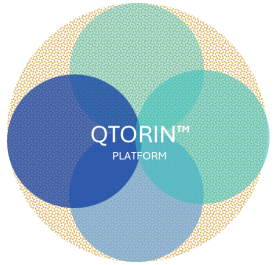
U.S. launch planning is accelerating for microcystic LMs (first indication)

- Multi-billion dollar uncontested TAM with estimated U.S. peak sales > $1 billion
- Sales force planned at ~40 reps, the upper end of prior ~20-40 rep range, to strengthen launch execution, field coverage, physician education, and patient access from day one
- 1H 2026 commercial talent recruited: Matt Pauls, Board Director *(Savara, Soleno, Insmed)*, Jen McDonough, SVP Market Access *(prev. Krystal Biotech)*; Kent Taylor, SVP Sales *(prev. Arcutis)*
- Well-capitalized after $230mm oversubscribed Q1 2026 financing



NDA submission completed with potential 1H 2027 FDA approval in microcystic LMs

- Rolling NDA submission completed in August 2026 following pre-NDA meeting
- Previously granted FDA's Breakthrough Therapy, Fast Track, and Orphan Designations



Leveraging QTORIN™ to expand into additional first-in-disease opportunities in orphan indications that are commercially underappreciated

- Deep rare disease pipeline with three QTORIN™ product candidates across six rare diseases anticipated by year end 2026

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QTORIN™ Rapamycin: Opportunity to be First FDA-approved therapy for estimated > 30k microcystic LM patients in the U.S.

1

Microcystic Lymphatic Malformations

QTORIN™ 3.9% Rapamycin

Position as first-line and establish as the new standard of care

Broad applicability potential: pediatric and adult, all severities, treatment-naïve and experienced

Long-term chronic administration anticipated

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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Multiple High-Impact Pipeline Milestones in 2026:
Density of Catalyst Creation Driven by QTORIN™

2 Cutaneous Venous Malformations

- ✓ Positive Phase 2 data
- ✓ Fast Track Designated
- • P3 initiation expected Q4 2026



3 Clinically Significant Angiokeratomas

- ✓ Fast Track Designated
- ✓ Phase 2 initiated April 2026, ahead of schedule
- • Phase 2 data expected 2H 2027



4 Disseminated Superficial Actinic Porokeratosis

- ✓ QTORIN™ pitavastatin formulation developed, IP filed
- • Phase 2 initiation expected Q4 2026



5 QTORIN™ Third Program

- • Announcement expected Q4 2026
- • Potential pipeline-in-a-product



DAVID OSBORNE, PhD
Chief Innovation Officer

6 QTORIN™ Rapamycin Fourth Indication

- • QTORIN™ rapamycin fourth indication announcement expected Q4 2026



QTORIN™ 3.9% rapamycin anhydrous gel and QTORIN™ pitavastatin are 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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OUR LEAD PRODUCT CANDIDATE

QTORIN™ 3.9% RAPAMYCIN ANHYDROUS GEL

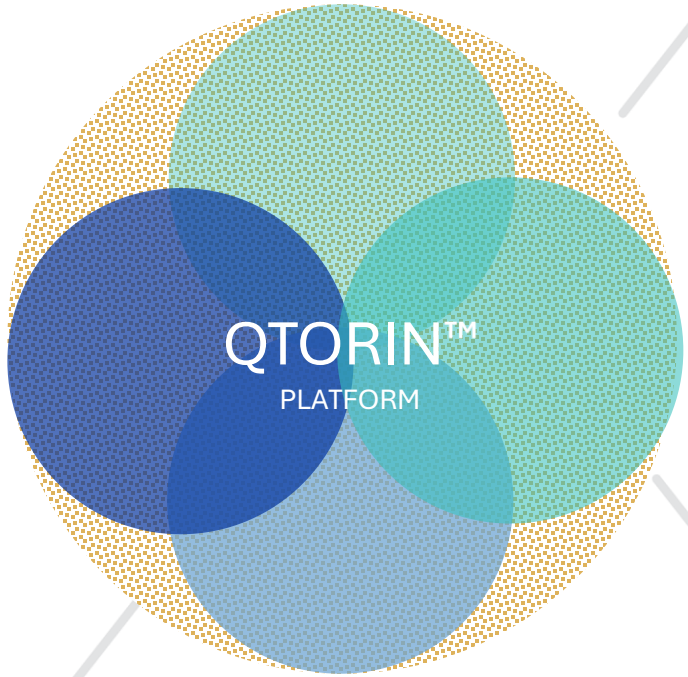
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QTORIN™: Reproducible Platform for Generating Novel, Topical Product Candidates in a Capital Efficient Manner



HIGH DRUG LOADING CAPACITY

High solubility → high concentrations → potential for rapid onset and large magnitude treatment effect





DERMAL ENGAGEMENT

Delivery to deeper layers of skin, often the site of disease pathophysiology



TOLERABILITY

Retaining active drug in the skin while minimizing systemic absorption



IP

Each QTORIN™ product candidate eligible for composition IP on formulation

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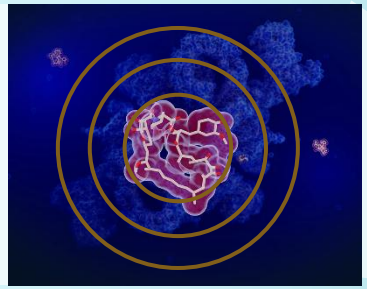
Our Breakthrough Innovation: QTORIN™ 3.9% Rapamycin Anhydrous Gel

Oral rapamycin is <u>not</u> a viable therapeutic option in skin diseases:

Systemic toxicities & low biodistribution to the skin

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]





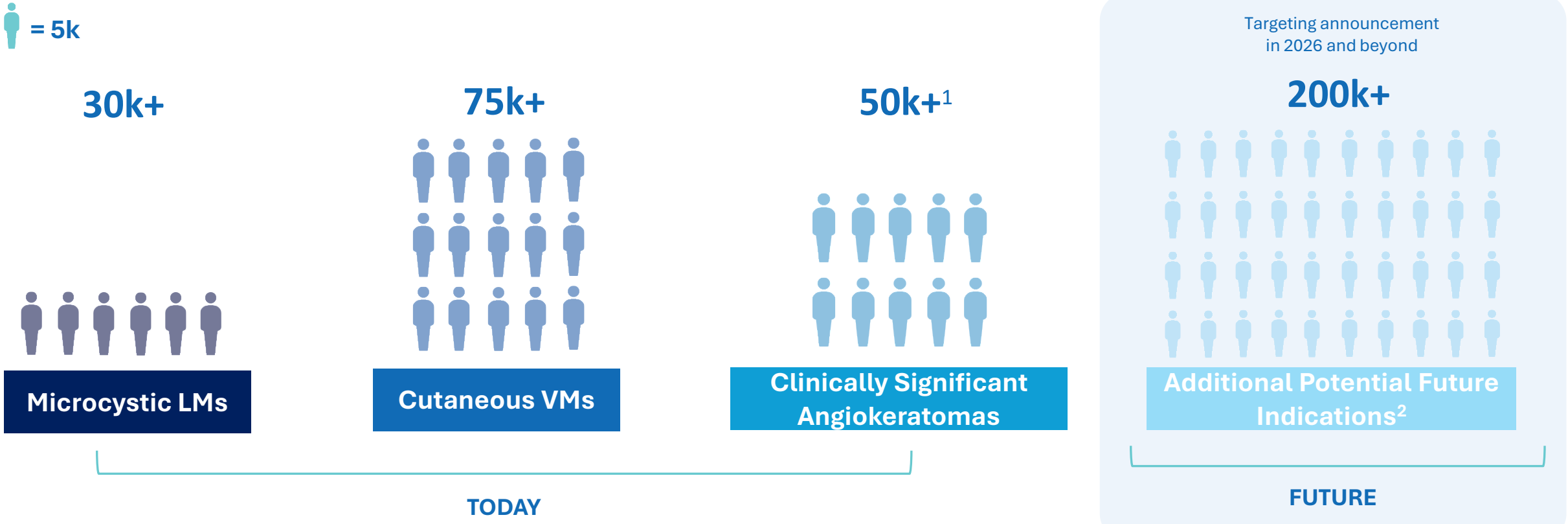
Intended for once daily at-home self-administration

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.

QTORIN™ Rapamycin: Fourth Indication Announcement in Q4 2026

Pipeline-in-a-product strategy expands addressable U.S. patient pool by 10x beyond initial indication



1. Clarity Pharma research (July 2025), n=643 physicians surveyed. 2. Lapa et al., *Journal of Cutaneous Medicine and Surgery*, (2025).

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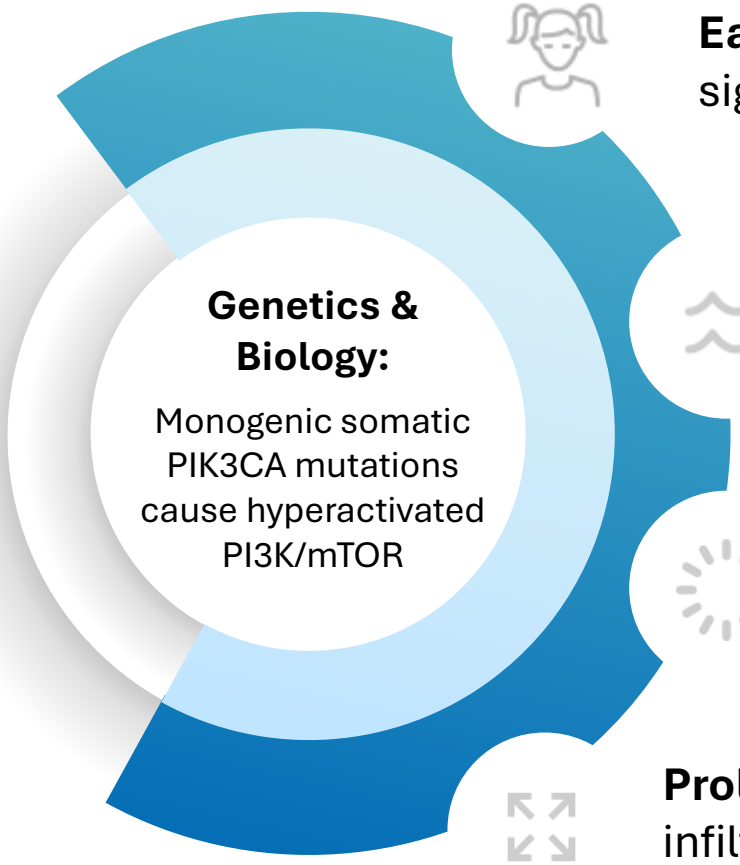
QTORIN™ 3.9% RAPAMYCIN

FOR

Microcystic Lymphatic Malformations

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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

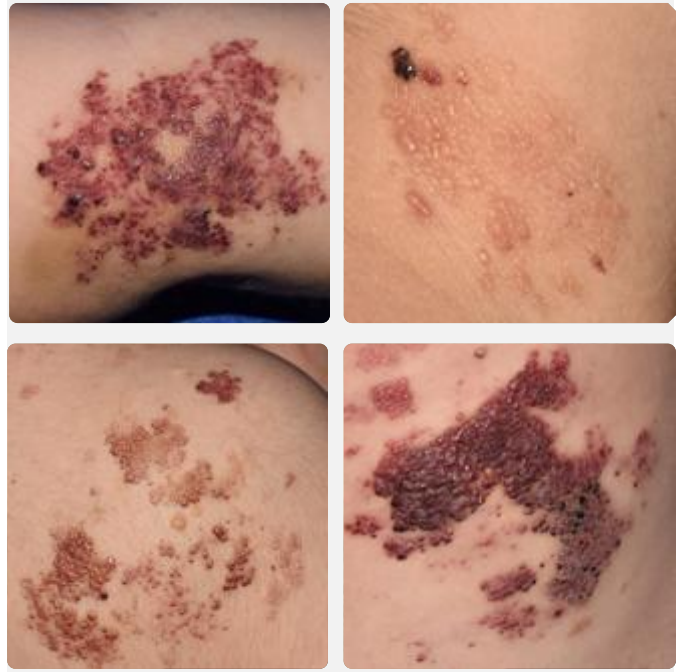
Deep infections: Recurrent cellulitis and serious soft tissue infections, resulting in hospitalizations

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

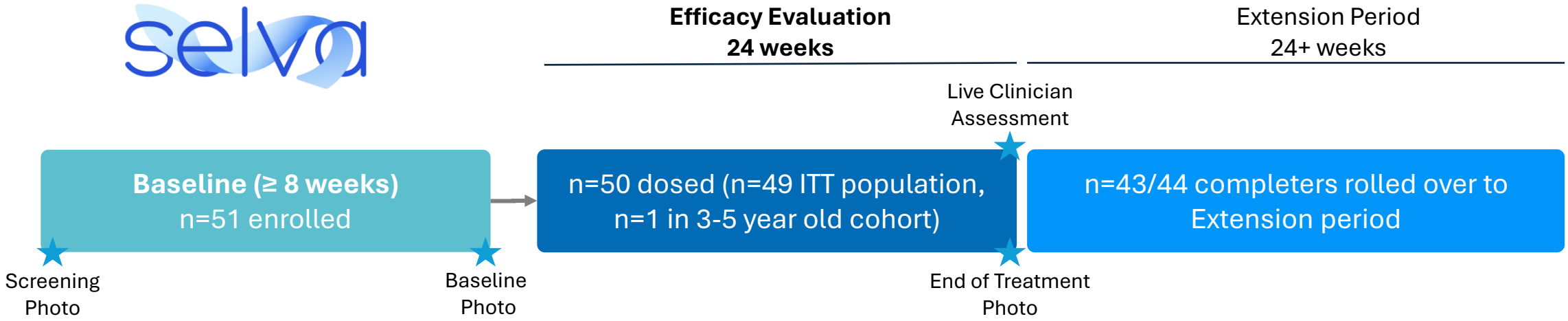
Orphan Drug Designation

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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).

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.

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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

Potential to be first and only FDA-approved therapy for >30,000 diagnosed U.S. patients

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).

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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)

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

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).

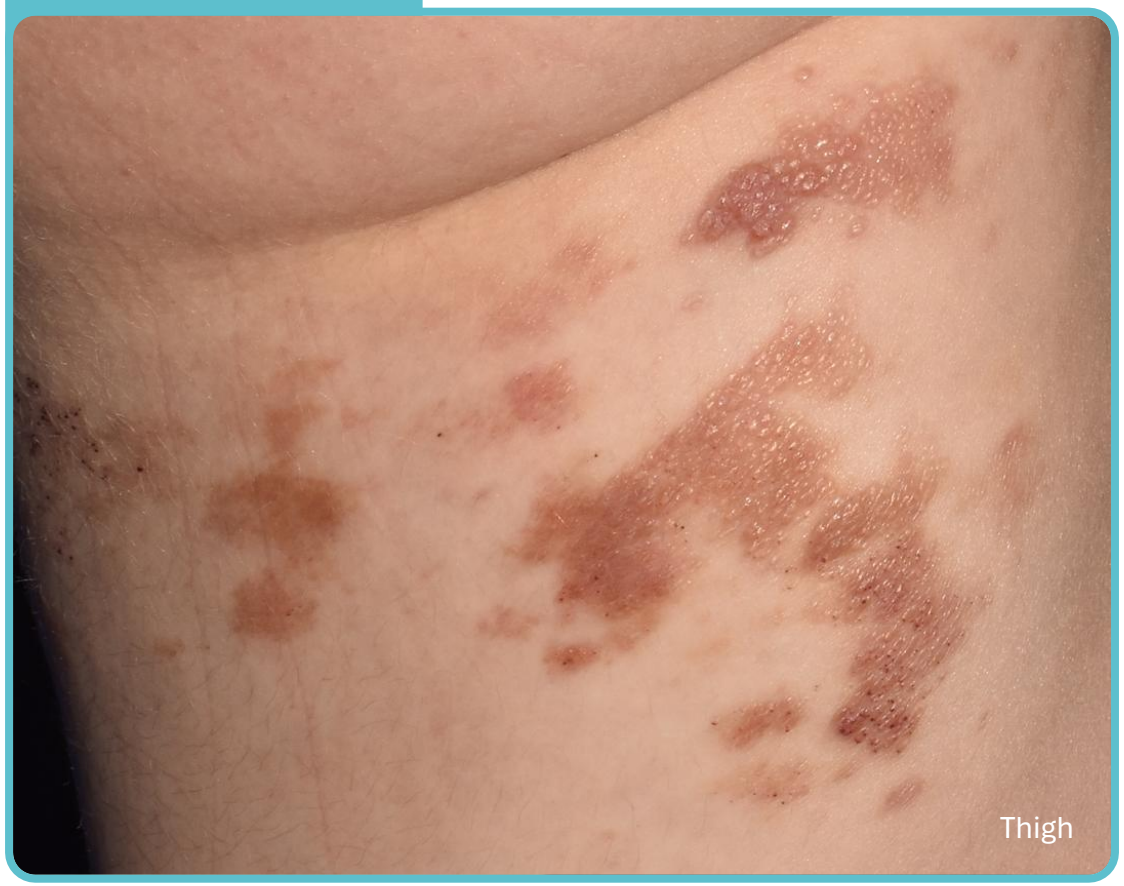
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Phase 3 Results: Age 10, Male, mLM-IGA: +2 “Much Improved”

Baseline



Week 24



Prior Interventions: *Multiple sclerotherapy procedures, antibiotics due to infection*

Note: Selective photos.

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

Baseline



Week 24



Prior Interventions: *Laser therapy, compounded topical rapamycin (discontinued due to skin irritation)*

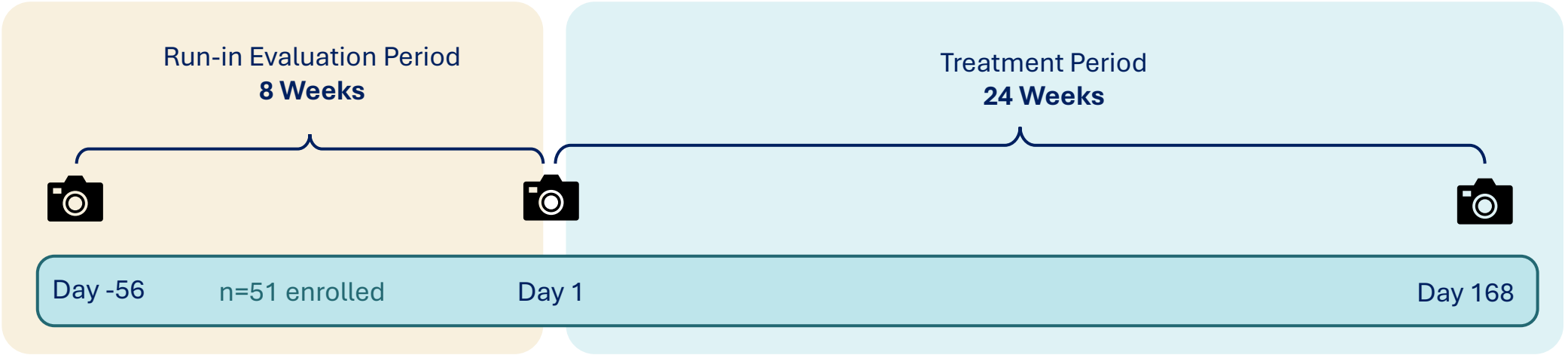
Note: Selective photos.

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Prespecified Blinded Independent Review Confirmed Disease Stability During Run-In and Marked Improvement with Treatment

mLM-MCSS: Sum of 3 static severity scales: Height, Leaking/Bleeding, Vesicle Appearance

Each scale rated 1 to 5; total score 3-15; Negative values indicate improvements from baseline



Pre-Treatment Change:

-0.1

Blinded mLM-MCSS

- Findings consistent with microcystic LM natural history study from Kato et al.[1] which demonstrated **no spontaneous regression**

Following 24 weeks of QTORIN™ rapamycin:

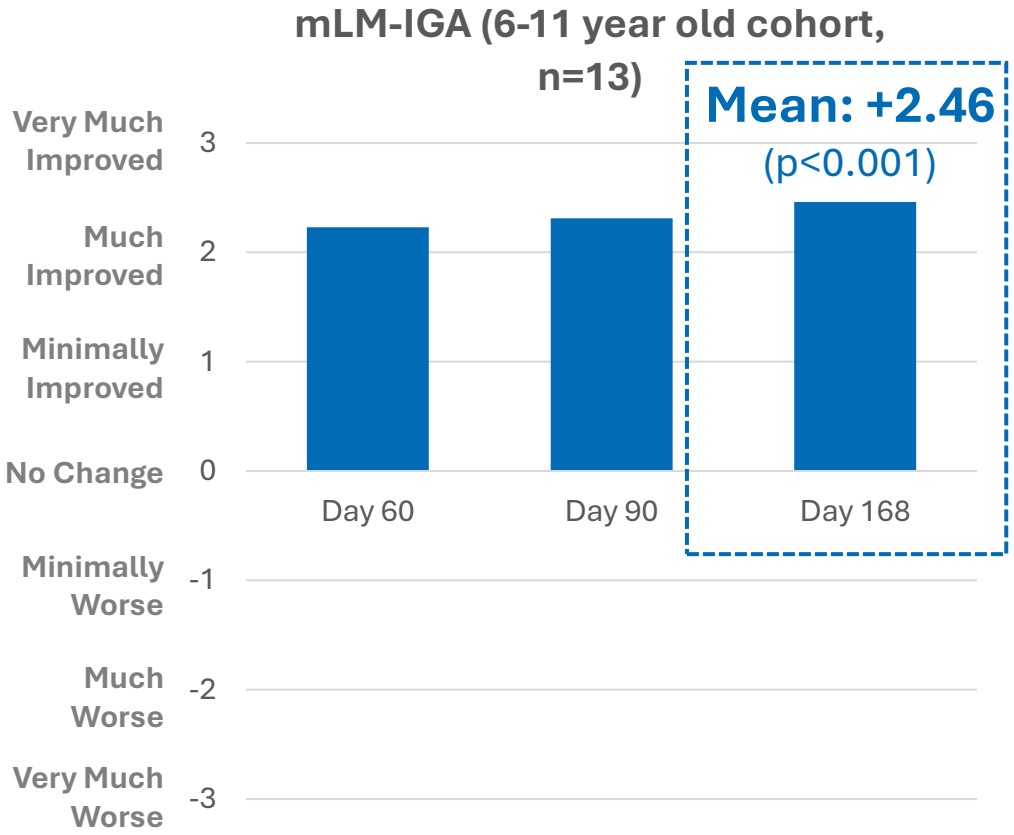
-3.4 (p<0.001)

Blinded mLM-MCSS Score: Day 1 (9.9) → Day 168 (6.6)

- Represents **48% of maximum potential improvement** from Day 1

Note: To preserve blinding, different raters were used to compare Day -56 vs Day 1 and Day 1 vs Day 168 photographs. 1. Kato M et al., *Plast Reconstr Surg Glob Open*. 2017 Sep 25;5(9):e1501.

New SELVA Data Highlight Benefits of Early Intervention and Chronic Disease Management in Children Aged 6–11



All pre-specified age cohorts in ITT were highly statistically significant



Age 7, Female

mLM-IGA: +3 "Very Much Improved"

- **100%** of the 6-11 y/o cohort were "***Much Improved***" (+2) or "***Very Much Improved***" (+3)
- All these patients rolled over into Treatment Extension, **supporting chronic disease management**

Dynamic change scale (7-point scale ranging from "Very Much Worse" (-3) to "Very Much Improved" (+3); positive values indicate improvements from baseline)

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 in systemic circulation on for all participants at all timepoints in the study

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).

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Regulatory Status: NDA Submission Completed in August 2026

Previously granted Breakthrough, Fast Track, and Orphan Designations, and FDA Orphan Drug Grant

Completed in-person Pre-NDA Meeting with FDA ▶ **Rolling NDA Review Granted** ▶ **First NDA Module Submitted** ▶ **NDA Submission Completed**

1. 505(b)(2) submission: leveraging FDA's prior findings for rapamycin to streamline review process
2. Phase 3 SELVA and Phase 2 studies highly statistically significant and clinically meaningful; real-world studies as supportive evidence
3. Seeking broad label and traditional approval (not accelerated) based on clinical endpoints

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Strong Barriers Through Multi-Layered Exclusivity Strategy

IP portfolio, trade secrets, and regulatory exclusivities through at least 2038

Granted U.S. Patents	**6 issued U.S. patents with claims through at least 2038** Including protection against 0.1% to 20% anhydrous gel compositions of rapamycin and other mTOR inhibitors
Trade Secrets	**Multiple trade secrets related to proprietary formulation processes and manufacturing know-how**
Regulatory	**Orphan drug designation and 7-year data exclusivity from anticipated FDA approval**

Pending patents could extend U.S. IP into mid-2040s



DAVID OSBORNE, PhD
Chief Innovation Officer

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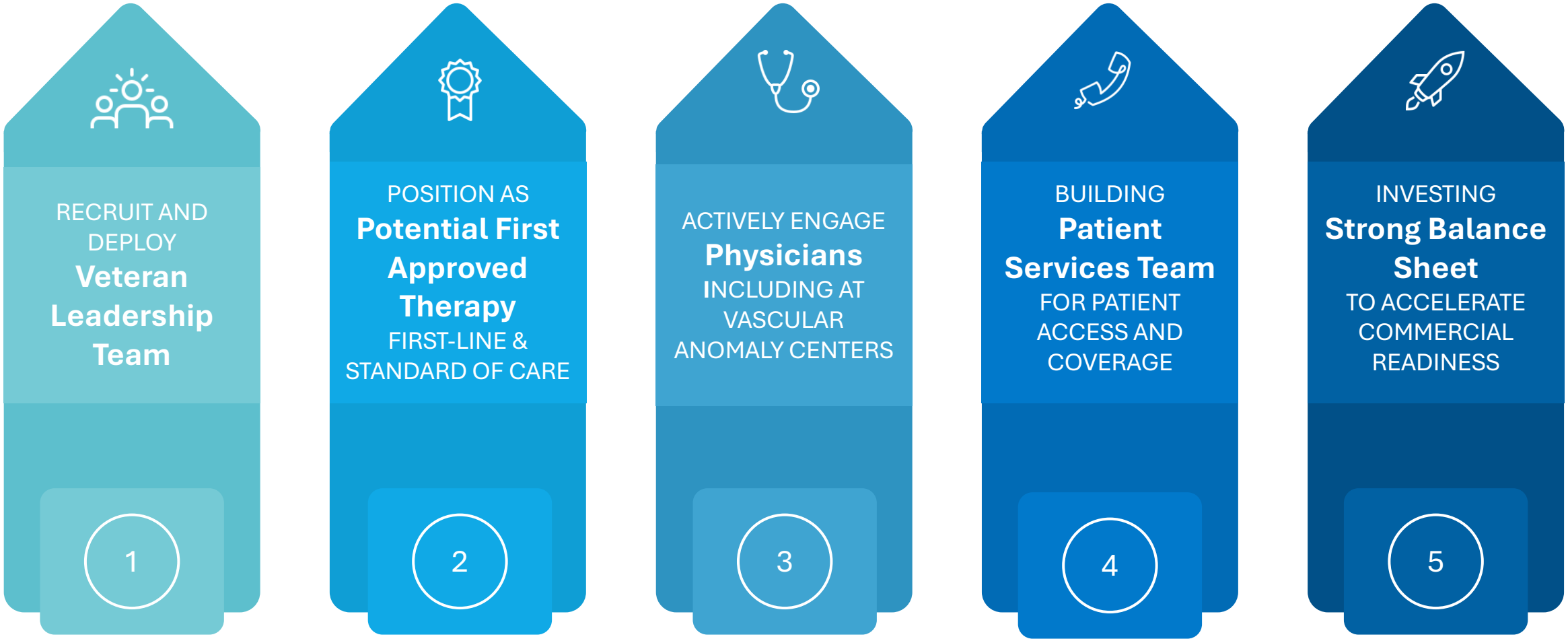
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U.S. Commercial Launch Planning

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QTORIN™ Rapamycin for Microcystic LMs:

Pre-Launch Initiatives Informed by Successful First-in-Disease Orphan Drug Precedents



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Leadership Executing on Key Pre-Launch Initiatives



Ashley Kline
Chief Commercial Officer
Led Oxervate® to >$500M; commercial leadership roles at Dompé and Genentech



Jen McDonough
SVP Market Access
Led VYJUVEK® market access; former SVP, Patient Access, Analytics & Operations at Krystal Biotech



Kent Taylor
SVP Sales
Led U.S. sales for ZORYVE® at Arcutis; built sales organization supporting OPZELURA® at Incyte



Vimal Patel, PharmD
SVP Medical Affairs
Led dermatology medical affairs at Incyte



Peter Finlayson
VP Marketing
Former Genentech marketing leader with extensive orphan disease and launch experience

Partnering with world-class executive search firms to recruit top talent

Sales force planned at ~40 reps (upper end of prior ~20 - 40 rep range) to strengthen launch execution, field coverage, physician education, and patient access from day one

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

Product X: Topical 3.9% rapamycin gel, including results from Phase 2 study of QTORIN™ rapamycin

Core Intent to Prescribe Insights

Would consider Product X as a **first-line** therapy

98% of physicians

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

75% of patients

"[Product X] would be an excellent safe option that I would readily prescribe"

Additional Insights into Pediatric Population

96% of physicians

See **advantages to targeted, localized delivery of Product X for pediatric patients** compared to oral mTOR or PI3K inhibitor

"Most parents would not like a young child treated with a systemic drug with so many potential long-term and serious side effects"

Physician Segmentation Insights

VAC* Physicians (n=21) — **73%** of patients

Non-VAC Physicians (n=31) — **77%** of patients

*Vascular Anomaly Centers

17 of 52 physicians stated they would **prescribe to 100% of patients**

Source: Medacorp Research, 2024. n=52 microcystic LM treaters.

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Highly Concentrated Patient Population: ~400 Established High Volume Centers for Treating Vascular Anomalies Comprise ~50% of Market

CLAIMS ANALYSIS SUPPORTS THREE SEGMENTS



Highly Concentrated Patients: ~50% of market at ~400 centers

- **Already established** Centers of Excellence for mLM
- **~96% of VACs today prescribe oral mTOR inhibitors**[1]: familiarity with class & mechanism of drug

Secondary Targets at Launch

Omni-channel approach

Inside sales can provide a high ROI

Apply additional learnings from Oxervate and other orphan launches

Long-tail Targets

All three market segments to be covered by orphan sales force of ~40 reps

Source: Palvella and ZS Associates claims analysis (2025). 1. Cohen-Cutler et al., *The Journal of Pediatrics,* (2023).

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Additional Pre-Launch Initiatives Accelerating

3

Physician Engagement

INCLUDING VASCULAR ANOMALY CENTERS

- Engaged over 200 of our initial 400 target clinics
- Meaningful presence at major medical congresses

4

Patient Services Team

FOR PATIENT ACCESS AND COVERAGE

- Hired field-based patient access liaison team leader, with team expansion ongoing
- Recent third-party payor research confirms likelihood of favorable coverage at orphan pricing ranges

5

Strong Balance Sheet

TO ACCELERATE COMMERCIAL READINESS

- Increased spending in 2026 following pre-NDA meeting to support commercial, marketing, and medical affairs launch preparations ahead of potential FDA approval and launch

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QTORIN™ Rapamycin Pricing: First-in-Disease Launches, Recent Topical Orphan Launches, and Payor Testing Support Potential Orphan Pricing

Rare Disease Medications	ARIKAYCE® (amikacin liposome inhalation suspension)	oxervate® (cenegermin-bkbj ophthalmic solution) 0.002% (20 mcg/mL)	TEPEZZA® teprotumumab-trbw
U.S. Annual List Price at Launch	~$135K[1]	~$190K[2]	~$200K-350K[3]
Indication	Refractory non-tuberculous mycobacterial (NTM) lung infections	Neurotrophic keratitis	Thyroid eye disease
Market Size	~12K-17K prevalent U.S. patients[4]	~50K prevalent U.S. patients[5]	~65K-100K prevalent U.S. patients[6]

1. Medscape Medical News interview with Mandy Fahey, Insmed Director of Corporate Communications (2018). 2. Assumes one course of 8-week therapy for both eyes. 3. Horizon Therapeutics Management guidance and Wall Street research. Price dependent on weight-based dosing. 4. Insmed corporate presentation (2025). 5. Sacchetti et al., *Clin Opth* (2014). 6. Stan et al., *Clin Endocrinol (Oxf)* (2024), Horizon Therapeutics investor presentation (2023).

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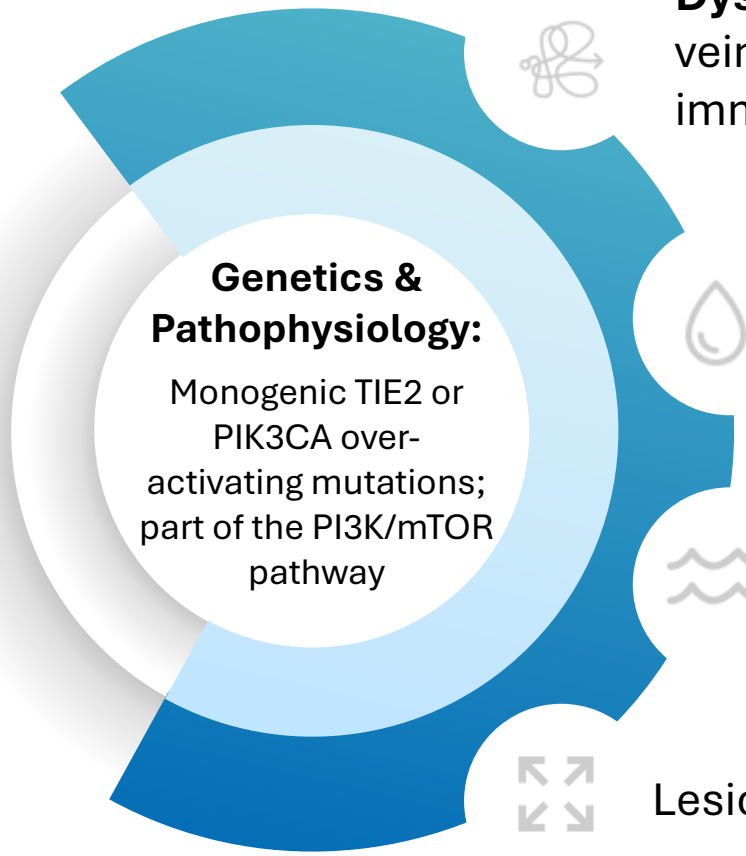
QTORIN™ 3.9% RAPAMYCIN

FOR

Cutaneous Venous Malformations

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Cutaneous Venous Malformations:
Serious, High Unmet Need



Dysregulated growth of malformed veins and **hyperproliferation** of immature venous endothelial cells

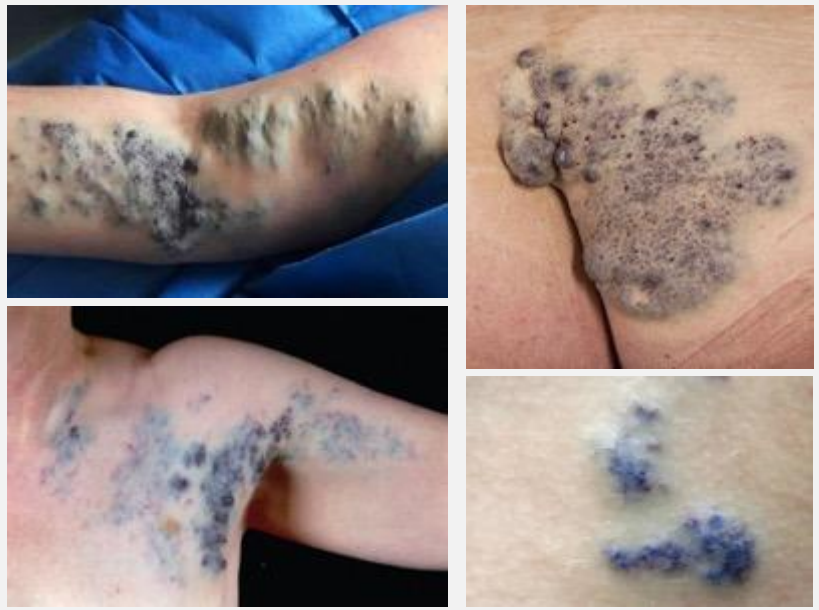
Dysfunctional venous architecture leads to bleeding, thrombosis, ulceration

Skin involvement in ~50-80% of venous malformation patients[2]

Lesions **do not resolve spontaneously**[3]

> 75k patients

ESTIMATED DIAGNOSED IN THE U.S.[1]



Most common vascular malformation with significant treater overlap with microcystic lymphatic malformations

1. Primary prospective research conducted by Clarity Pharma. 2. Rikihisa et al., *Journal of Vascular Surgery, (*2019); Boon et al., *Arch Dermatol, (*2004); Mazoyer et al., *Arch Dermatol, (*2008). 3. Fujino et al., *Lymphatic Research and Biology*, (2024).

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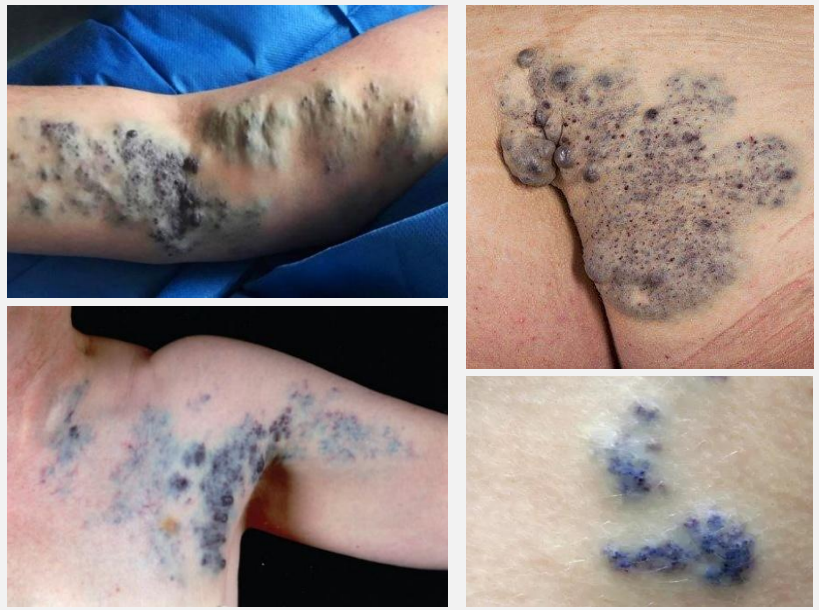
Cutaneous Venous Malformations:
Near-term Phase 3 Pivotal Study Planned

POSITIVE PHASE 2 DATA ANNOUNCED DEC 2025

- **New 24-week data presented at ISSVA World Congress (May 2026) by Dr. Jim Treat (CHOP)**
  - Height/Engorgement and Appearance improvements continued through 24 weeks demonstrating deepening of treatment effect over time
- **Phase 3 initiation expected in Q4 2026**
  - Planned EOP2 meeting with FDA to discuss Phase 3 trial design
  - Pipeline-in-a-product: supplemental NDA (sNDA) planned
- **Recent publication highlights off-label sirolimus as the most utilized medical therapy for VMs** *(Coulie et al, July 2026)*

> 75k patients

ESTIMATED DIAGNOSED IN THE U.S.



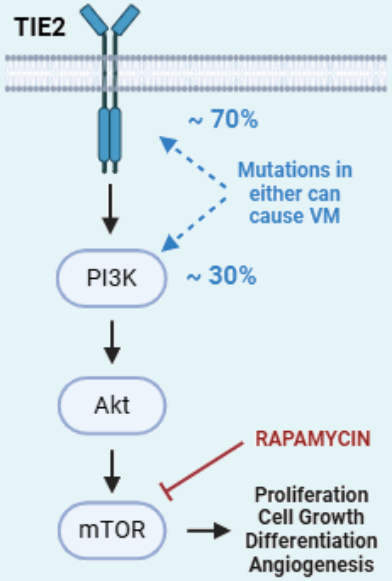
No FDA-approved therapies

FDA Fast Track Designation Granted

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Venous Malformations: Progress Towards the Potential First Targeted Therapy for Unaddressed Cutaneous Disease

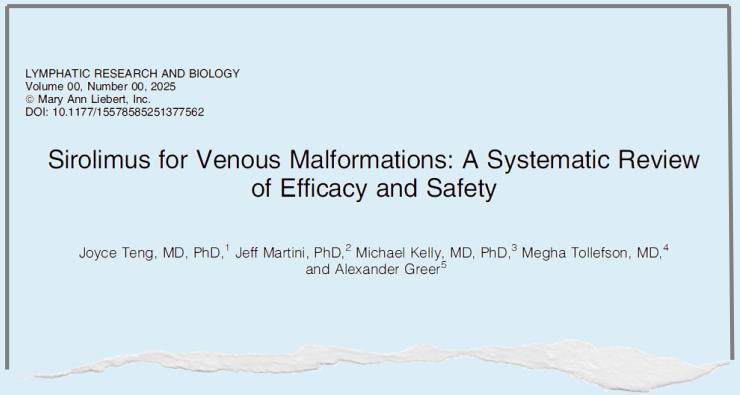
Known Genetics / Clear Biology



Plausible mechanism

Limaye et al (2009, 2015)

Real-world Evidence



Real-world evidence supports rapamycin's off-label use in primarily internal manifestations of VMs...

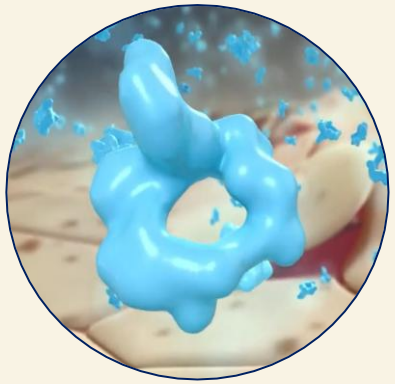
...however, poor patient outcomes persist in cutaneous disease

Teng et al (2025)

On Target, In Tissue

QTORIN™

RAPAMYCIN



3.9% concentration
Dermal penetration
Extensive CMC package

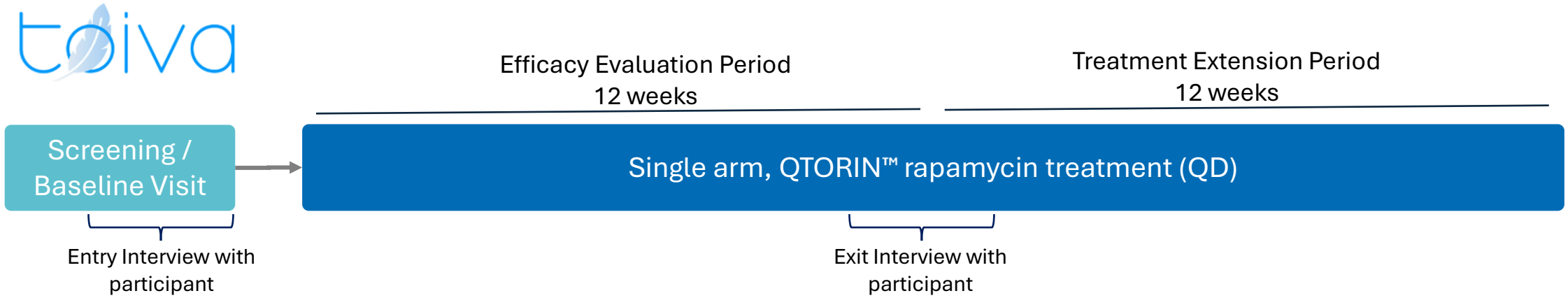
Phase 2 TOIVA Data (2025)

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 TOIVA Study in cVMs: 24-Week Study

Single-arm, baseline-controlled, QD dose, age 6+



Safety and Tolerability

Efficacy (no pre-specified primary endpoint): cVM-IGA (7-point clinician change scale), cutaneous VM multi-component static scale (cVM-MCSS), other clinician- and patient-reported outcomes

Statistics: Intent-to-Treat (ITT) population, based on available data at each time point and analyzed per statistical analysis plan

Key Entry Criteria: Enriched for patients with cutaneous disease and confirmed by third party eligibility consult team; genetics not required

Enrollment: 16 participants enrolled and dosed

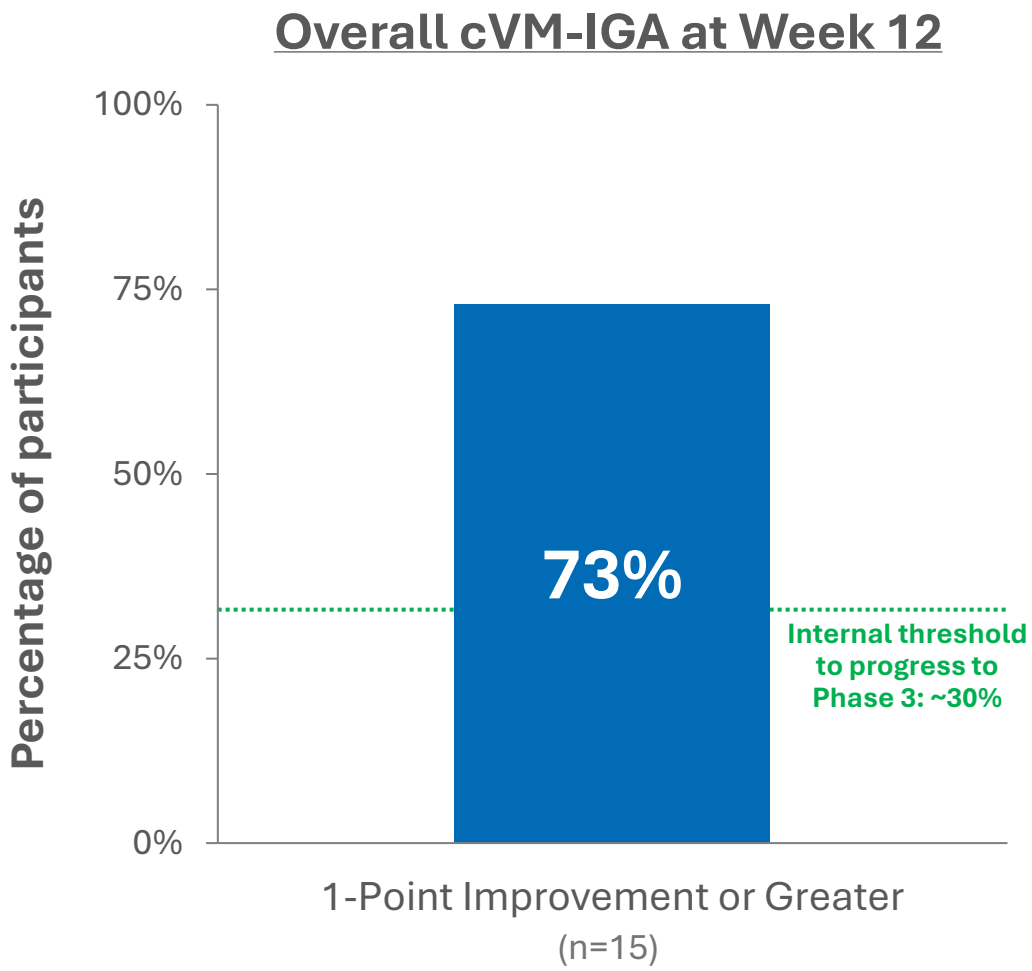
Sites: Mayo Clinic; Cleveland Clinic; Children's Hospital of Philadelphia; Stanford Medicine; UCI; Children's Hospital Colorado; Children's Healthcare of Atlanta; Johns Hopkins Medicine; University of Utah Health; UNC Health; Minnesota Clinical Study Center

Note: Statistical significance ($p<0.05$) is nominal as there was no adjustment for multiplicity amongst efficacy endpoints. Data analyzed per statistical analysis plan; ITT analyzed with no imputation of values for missing data.

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Overall cVM-IGA: 73% of Participants Demonstrated Improvement at Week 12

Single-arm, baseline-controlled, QD dose, age 6+



- **Overall cVM-IGA:** 7-point clinician-assessed dynamic change scale ranging from “Very Much Worse” (-3) to “Very Much Improved” (+3)
  - Mean effect size at week 12: **+1.5 (p<0.001)**
  - Median effect size at week 12: **+2.0**
- **73%** of participants (11/15 participants) demonstrated at least a 1-point improvement on the Overall cVM-IGA at Week 12
- **67%** of participants (10/15 participants) were rated as either “Much Improved” (+2) or “Very Much Improved” (+3) on the Overall cVM-IGA at Week 12

Note: Statistical significance (p<0.05) is nominal as there was no adjustment for multiplicity amongst efficacy endpoints. Data analyzed per statistical analysis plan; ITT analyzed with no imputation of values for missing data.
1. Genetic testing was not required as part of the protocol; Palvella is continuing efforts to collect genetic data on trial participants.

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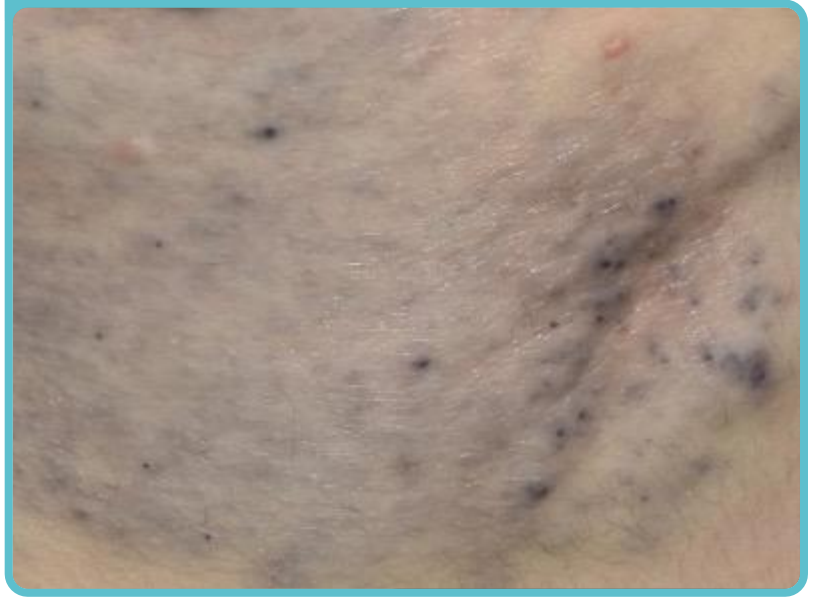
Phase 2 Results: Meaningful Improvement in Patient QoL



Site: CHOP
Investigator: Dr. Denise Adams

Participant Age: 17
Mutation: TEK
cVM-IGA at Week 12:
Very Much Improved (+3)

Baseline



Week 12



Participant Qualitative Interview: *"I've definitely noticed some improvements…it's definitely had a positive effect…It's more comfortable to wear a bra now…I've had less pain in that specific area"*

Note: Selective photos.

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Phase 2 Results: Safety and Tolerability

- QTORIN™ rapamycin was generally well-tolerated, similar to previous clinical trials
- Most common Treatment-Emergent Adverse Events were application site reactions (erythema, 25%)
- All Treatment-Related Adverse Events were moderate or mild (no severe events)
  - Majority of AEs were mild
  - No SAEs related to study drug
  - No unexpected AEs

Rapamycin levels were below the lower limit of quantification (2 ng/mL) in systemic circulation on a standard lab assay for all participants at all timepoints in the study

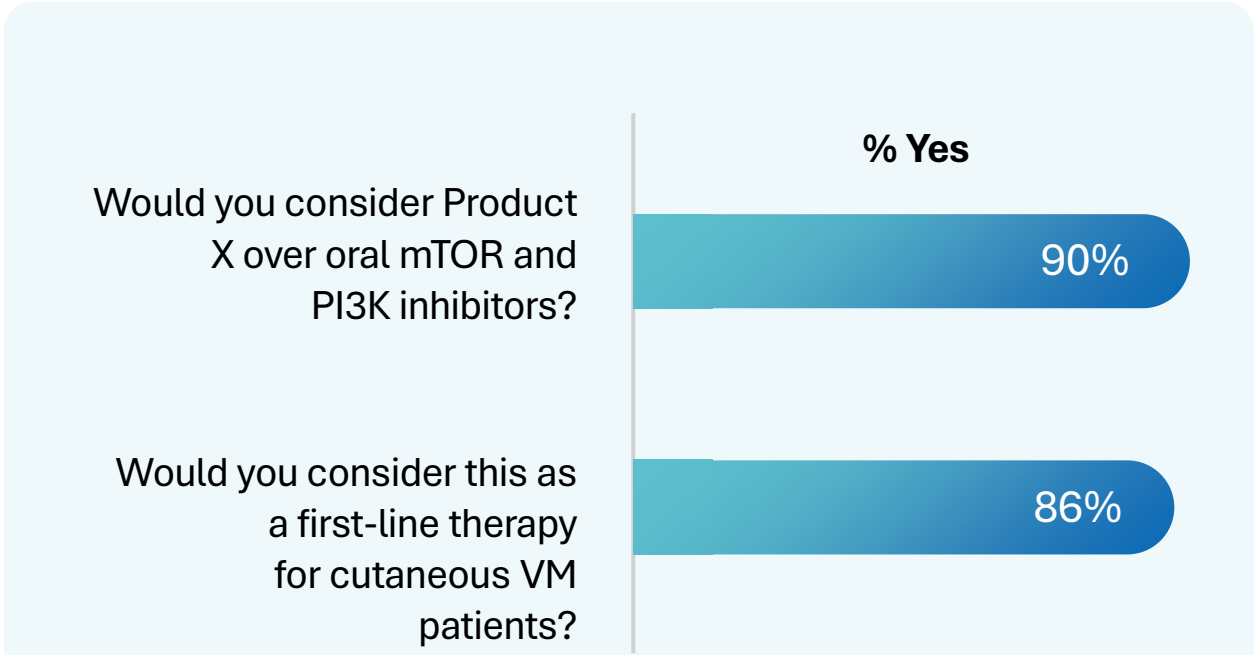
Significantly below 5 ng/mL which is the lower boundary where rapamycin begins to exert immunosuppressive effects

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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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QTORIN™ 3.9% RAPAMYCIN

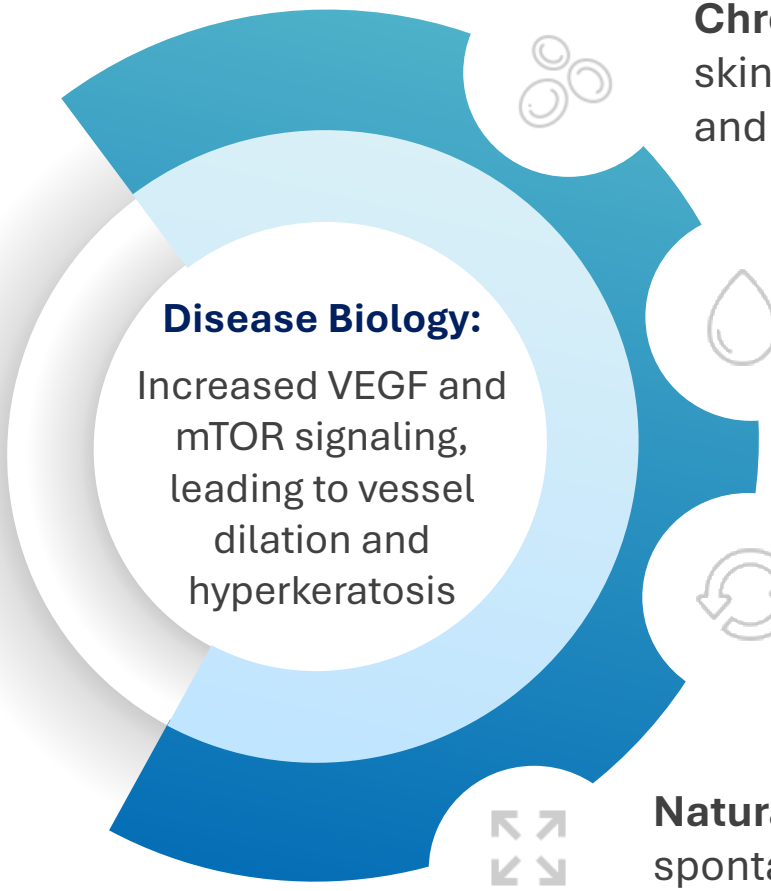
FOR

Clinically Significant Angiokeratomas

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Clinically Significant Angiokeratomas:
Superficial Lymphatic Malformations

Palvella's focus to include Fordyce, Solitary, Mibelli, and Circumscriptum subtypes



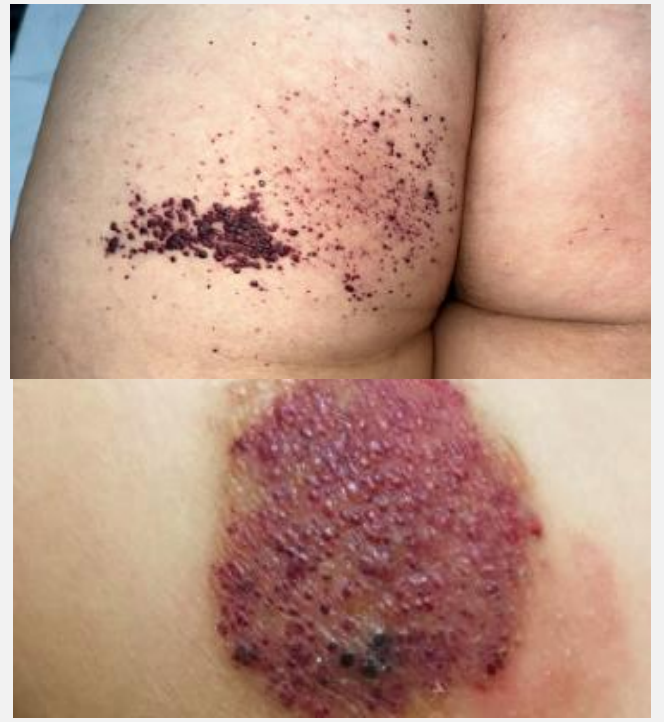
Chronically debilitating lymphatic-derived skin lesions associated with bleeding, pain, and functional impairment

Recurrent bleeding: Friction can cause fragile lesions to frequently bleed

Persistent and extensive: Lesions can be large and increase in size, number, and extent over time

Natural history: No tendency for spontaneous regression

> 50k patients
ESTIMATED DIAGNOSED IN THE U.S.[1]



Phase 2 study of up to n=15 patients underway

Topline results expected 2H 2027

Fast Track Designation granted

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Wang et al., *Journal of Cutaneous Pathology*, (2014); Trindade et al., *Am J Dermopathol*, (2014); Prindaville et al., *Pediatric Dermatology*, (2017); Singh et al, *Indian Journal of Dermatology*, (2023); Caraffa et al, *International Journal of Infection*, (2025); Molla, *Clinical, Cosmetic and Investigative Dermatology,* (2024). Ivy H, Julian CA. Angiokeratoma Circumscriptum. Treasure Island (FL): StatPearls Publishing; 2025 Jan; Lapa et al., *Journal of Cutaneous Medicine and Surgery*, (2025).
1. Clarity Pharma research (July 2025), n=643 physicians surveyed.

Commercial Opportunity for Clinically Significant Angiokeratomas

Estimated Diagnosed U.S. Prevalence	>50k clinically significant angiokeratomas in the U.S. based on nationally representative, blinded, real-world observational study conducted July 2025 (n=643 physicians)[1]
Pricing	Anticipate drug pricing similar to QTORIN™ rapamycin for Microcystic LMs and Cutaneous VMs based on disease severity and lack of FDA-approved therapies
Market Research[2] *(n=50 physicians)*	**96%** would incorporate Product X (topical 3.9% rapamycin gel) into their practice **85%** believe there is unmet need for a novel treatment across all subtypes *"A topical application that was basically asymptomatic in terms of its application…would be ideal for these intractable cases"*

1. Clarity Pharma research (July 2025), n=643 physicians surveyed. 2. Medacorp Survey (September 2025), n=50 physicians surveyed.

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QTORIN™ PITAVASTATIN

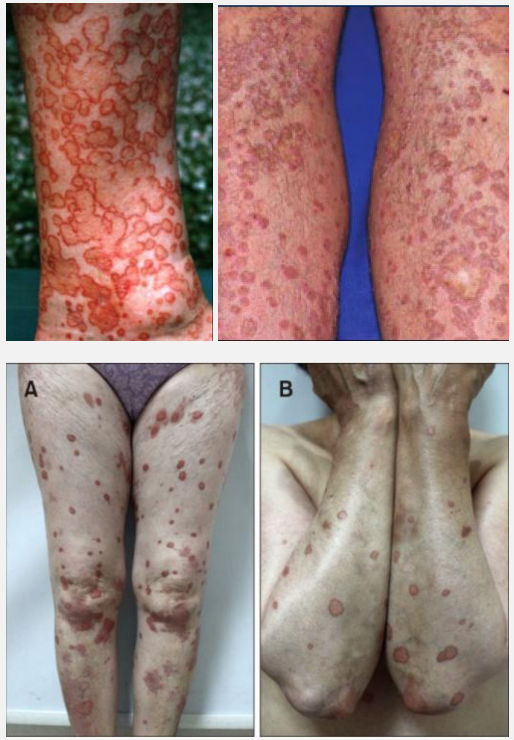
FOR

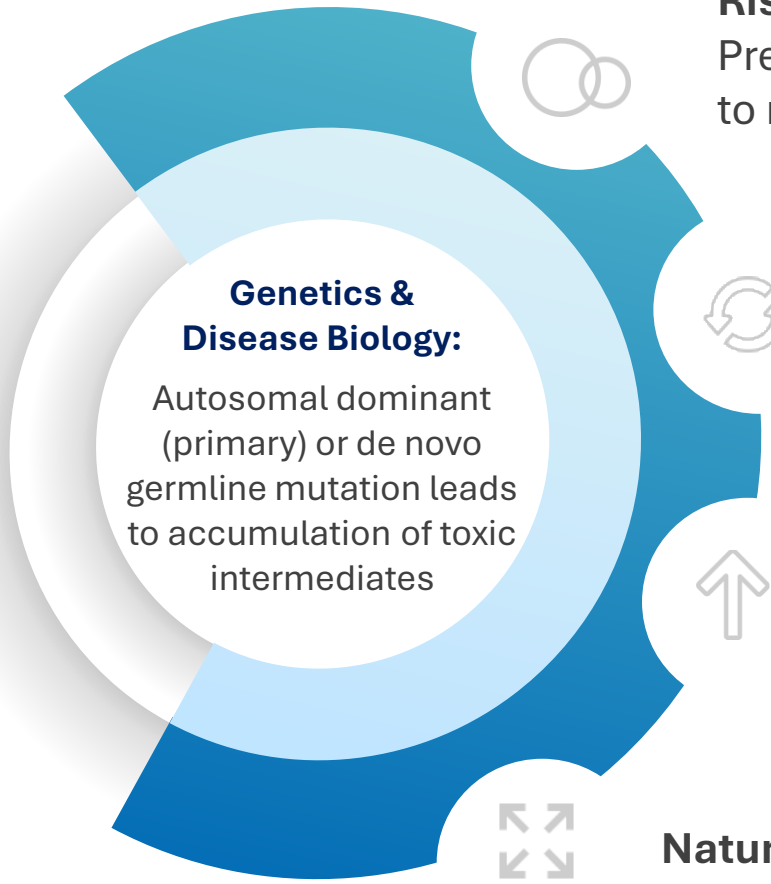
Disseminated Superficial Actinic Porokeratosis

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Disseminated Superficial Actinic Porokeratosis (DSAP):
Chronic, Pre-Cancerous, and Progressive

> 50k patients
ESTIMATED DIAGNOSED IN THE U.S.[1]





Risk of malignant transformation: Premalignant disease with transformation to non-melanoma skin cancers[2]

Significant impact to quality of life: clinical signs include skin disfigurement, burning, and persistent itch

Persistent and extensive: Clonal proliferation of abnormal keratinocytes leads to increased number and size of lesions

Natural history: Spontaneous regression is extremely rare[2]

No FDA-approved therapies

Phase 2 trial initiation expected 2H 2026

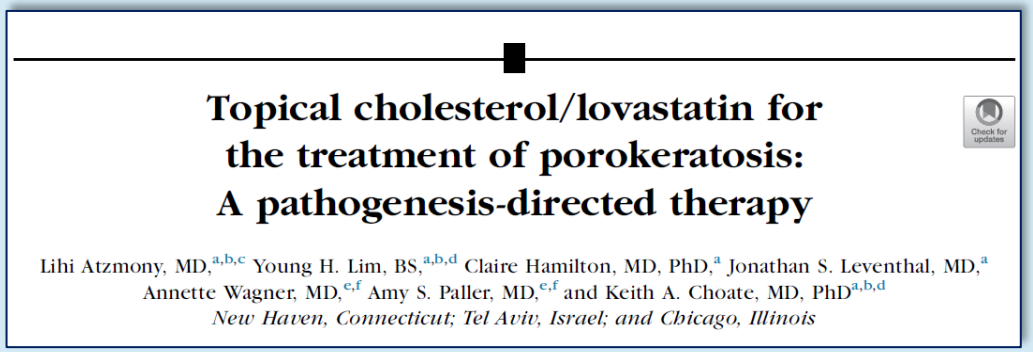
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1. Clarity Pharma research (April 2025), n=277 physicians surveyed. 2. Williams, Grant M., et al. “Porokeratosis.” StatPearls Publishing, 2024.

Unmet Need for First FDA-approved Topical Mevalonate Pathway Inhibitor for DSAP

Oral statins are not a viable therapeutic option in DSAP:

High first pass metabolism and/or sub-therapeutic biodistribution to the skin



Topical cholesterol/lovastatin for the treatment of porokeratosis: A pathogenesis-directed therapy

Lihi Atzmony, MD,[a,b,c] Young H. Lim, BS,[a,b,d] Claire Hamilton, MD, PhD,[a] Jonathan S. Leventhal, MD,[a] Annette Wagner, MD,[e,f] Amy S. Paller, MD,[e,f] and Keith A. Choate, MD, PhD[a,b,d]

New Haven, Connecticut; Tel Aviv, Israel; and Chicago, Illinois

Proof-of-concept study, demonstrating a plausible mechanistic approach

Recently published systematic review in *Clinical and Experimental Dermatology* (Martini et al, 2026) included **24 case studies** of off-label use of topical statin therapy in porokeratosis…

…however, today **poor patient outcomes persist** due to lack of access and known variability in unapproved formulations which can limit safety, efficacy, and quality

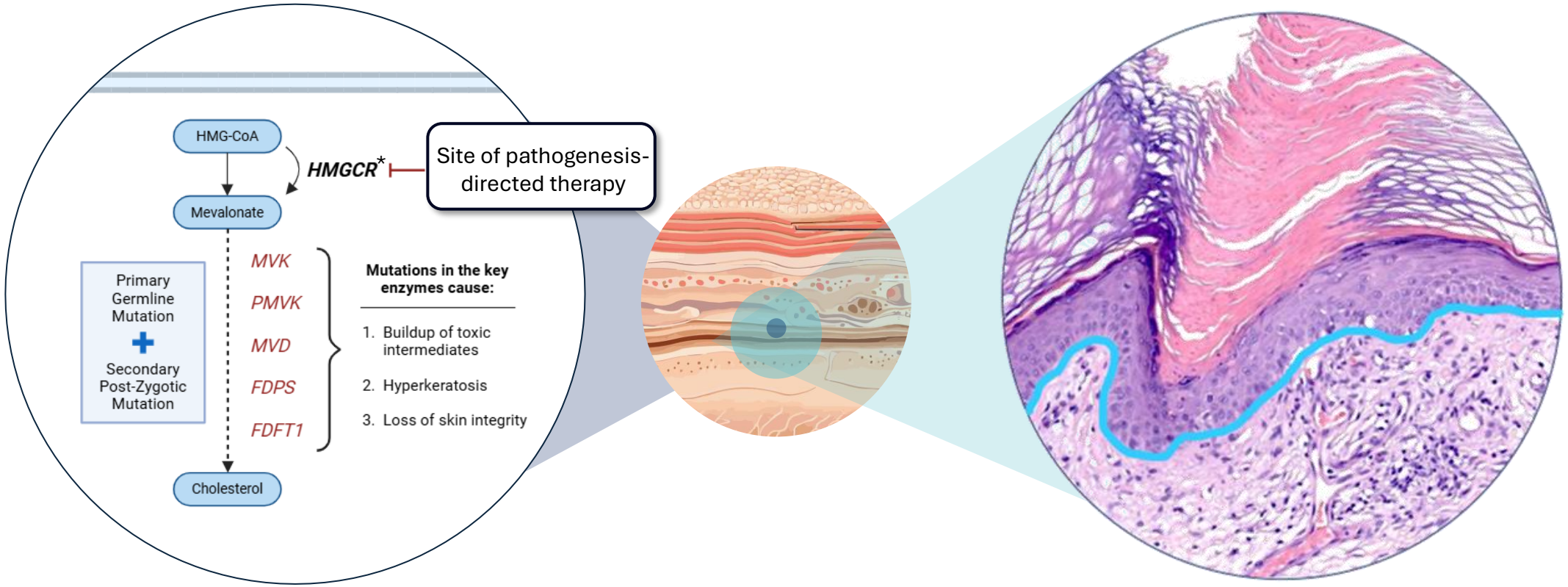
Significant need for an FDA-approved topical mevalonate pathway inhibitor

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Clear Biology: Targeting the Causal Mevalonate Pathway

Target: Mevalonate Pathway

Tissue: Epidermis & Dermis



An on-target, in-tissue approach could result in significant clinical improvement

*HMGCR = 3-hydroxy-3-methylglutaryl-coenzyme A reductase. Image sources: Milani D, Warbasse E, Chen WS. Porokeratosis. PathologyOutlines.com website.

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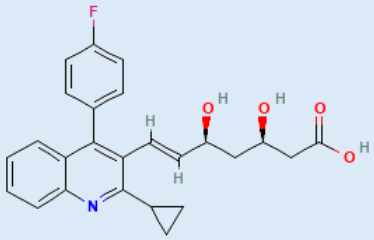
QTORIN™ Pitavastatin: On Target, In Tissue

Using QTORIN™, we considered and tested a wide range of mevalonate pathway inhibitors

Molecule	Potency	Optimal Skin PK	Stability
Pitavastatin	✓	✓	✓
Mev. Inhibitor 2	*Did not meet some or all pre-defined target product attributes*		
Mev. Inhibitor 3			
Mev. Inhibitor 4			
Mev. Inhibitor 5			
Mev. Inhibitor 6			
Mev. Inhibitor 7			

QTORIN™

PITAVASTATIN



- **Pitavastatin is an FDA-approved next-generation oral statin** for patients with primary hyperlipidemia and mixed dyslipidemia
- **Superior inhibition of the mevalonate pathway compared to all molecules evaluated**
- **Key characteristics:**
  - Payload: > 2% concentration achieved
  - Dermal penetration: *in vitro* penetration test confirms > IC90
  - Low systemic absorption
  - Encouraging preliminary drug stability
  - IP: Filed formulation & method of use IP and licensed Yale IP

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Disseminated Superficial Actinic Porokeratosis (DSAP):
Aligned With Palvella's Pipeline Strategy

	QTORIN™ Rapamycin	QTORIN™ Pitavastatin	
	Microcystic LMs, Cutaneous VMs, Clinically Significant Angiokeratomas	DSAP	
Serious	✓	Chronic, extensive lesions, malignant transformation	*Significant unmet medical need*
Rare	✓	>50k U.S. patients	
No FDA-approved Therapies	✓	None	
Strong Scientific & Biologic Rationale	✓	Mutations in mevalonate pathway lead to accumulation of toxic intermediates	*Optimizing likelihood of clinical success*
Published Case Studies & Off-label Use of API	✓	Multiple published case studies + use in academic centers	
Commercially Attractive	✓	Opportunity to be first-in-disease and SOC	*Multi-billion dollar U.S. TAM[1]*

1. Based on internal and third-party estimates.

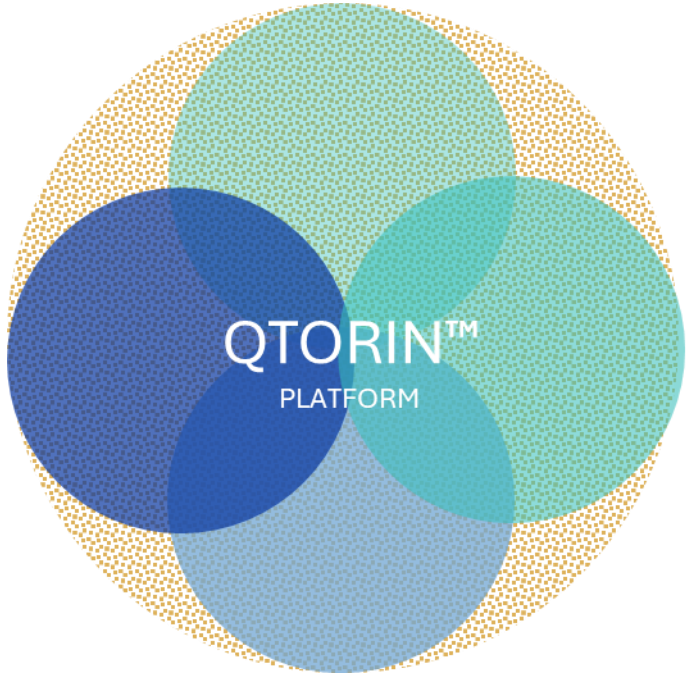
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QTORIN™

Platform

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THERAPEUTICS

QTORIN™: Repeatable New Product Development Engine



- **PARADIGM SHIFT** towards targeted and well-tolerated delivery to the dermis
- **BROAD PLATFORM CAPABILITY** across diverse range of molecules
- **NEW LONG-DURATION IP** opportunity with each product candidate

We plan to announce one new QTORIN™ program and one new QTORIN™ rapamycin indication later this year

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Finance

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U.S. Launch and Rare Disease Pipeline are Well-Funded with $251mm on the Balance Sheet as of 6/30/26

Upsized $230mm raise in February 2026 with high-quality investor participation

- Key strategic priorities accelerating:
  - NDA submission completed
  - Commercial and medical affairs teams expanding
  - Major QTORIN™ pipeline and platform value drivers advancing

$251 million

6/30/26 cash

Anticipated full-year 2026 cash expenses of $85 to $95 million

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What Sets Palvella Apart: Building The Leader in Rare Skin Diseases and Vascular Anomalies

Positive Phase 3 data in microcystic lymphatic malformations, focused on potential U.S. commercial launch post-NDA submission

QTORIN™ rapamycin: potential to be first approved therapy and SOC in U.S. for microcystic LMs, cutaneous VMs, and angiokeratomas

U.S. commercial opportunity: multi-billion-dollar TAM in mLMs, with potential to expand addressable pool of patients by 10x

Deep pipeline of rare disease therapies: six diseases anticipated by year end 2026

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Striving to be first for rare disease patients

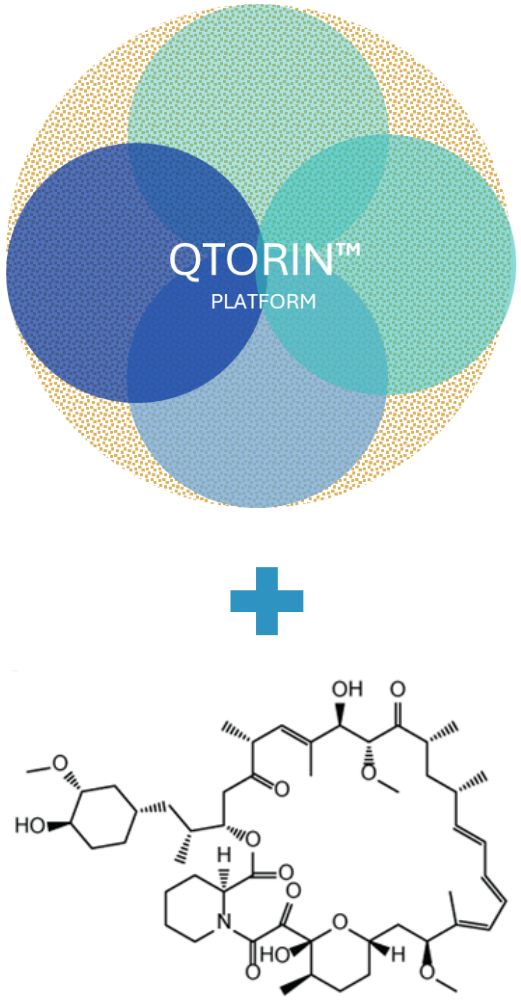
palvella THERAPEUTICS

Thank You

Striving to be first for rare disease patients

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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

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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.

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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).

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No Spontaneous Regression Well-Established in Microcystic LMs



OPEN

PRS GLOBAL OPEN ORIGINAL ARTICLE
Reconstructive

Spontaneous Regression of Lymphangiomas in a Single Center Over 34 Years

Motoi Kato, MD*
Shoji Watanabe, MD, PhD*
Reiko Kato, MD†
Hiroshi Kawashima, MD, PhD†
Takuya Iida, MD, PhD‡
Azusa Watanabe, MD, PhD*

Background: A lymphangioma, also called a lymphatic malformation, is a congenital condition that frequently occurs in young children. It is classified into 3 groups depending on the size of the cysts (macrocystic, microcystic, and mixed). Spontaneous regression occurs in some cases; however, the characteristics of patients who show regression have not been studied previously. Furthermore, the types and the timing of the initial treatment are still controversial. Therefore, we statistically analyzed the occurrence of short-term spontaneous regression, patient age at original occurrence, cyst types, cyst sizes, and cyst locations in patients diagnosed with peripheral localized lymphangiomas in a single children center over 34 years.
Methods: We retrospectively collected the data of 153 patients and reviewed the medical charts.
Results: Spontaneous regression occurred only in macrocystic or mixed type; regression was most frequent in patients who, at the time of onset, were more than 2 years old.
Conclusions: We concluded that elderly patients with macrocystic or mixed type lymphangioma may have to wait for treatment for over 3 months from the initial onset. Conversely, microcystic type could not be expected to show regression in a short period, and prompt initiation of the treatments may be required. The difference of the regression or not may depend on the characteristics of the lymph flow. *(Plast Reconstr Surg Glob Open 2017;5:e1501; doi: 10.1097/GOX.0000000000001501; Published online 25 September 2017.)*

MATERIALS AND METHODS

We retrospectively reviewed the medical charts of 501 patients who were diagnosed with lymphangiomas or lymphatic malformations in our hospital over 34 years (April 1983 to December 2016). Lymphangioma cases that showed peripheral localization and were observed for more than 3 months without medical or surgical intervention were included. The diagnosis was reconfirmed on the basis of radiological findings and the clinical course according to the vascular anomalies classification of the International Society for the Study of Vascular Anomalies.[1]

Patients diagnosed with lymphangiomatosis, Gorham disease, combined vascular anomalies (Klippel–Trenaunay syndrome, Proteus syndrome, and Maffucci syndrome), intraabdominal lesions, and/or intrathoracic lymphangiomas were excluded from this study. Additionally, patients who were misdiagnosed, those who did not undergo radiological assessments (ultrasound, computed tomography, or magnetic resonance imaging), those who were not followed for 6 months after onset, and those who were administered treatments for the lesions (medication, aspiration, sclerotherapy, and/or surgery) within 3 months after the original onset were excluded (Table 1; Fig. 1). Patients who were prescribed acetaminophen and/or antibiotics for pain and/or infection and those with peripheral lesions that connected to the intrapleural region were included.

Spontaneous regression was considered as an over 20% decrease in the lesion size over 3 months when compared with the size at the original onset. We analyzed the patient age at the original onset, original lesion size, and lesion location retrospectively. Congenital lesions were considered as having an onset at 0 years of age, even when diagnosed prenatally.

Statistical analyses involved the 2-sided *t* test for normally distributed data and the F-test for assessment of less than 5 patients. A receiver operating characteristic (ROC) curve was drawn using the SPSS software (IBM Corp., Ar-

*From the *Department of Plastic and Reconstructive Surgery, Lymph Clinic, Saitama Children's Medical Center, Saitma, Japan; †Department of Pediatric Surgery, Saitama Children's Medical Center, Saitma, Japan; and ‡Department of Plastic and Reconstructive Surgery, The University of Tokyo, Tokyo, Japan.*
Received for publication March 29, 2017; accepted July 26, 2017.
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DOI: 10.1097/GOX.0000000000001501

Disclosure: *The authors have no financial interest to declare in relation to the content of this article. The Article Processing Charge was paid for by the authors.*

A 34-year, 28-subject study confirmed no spontaneous regression

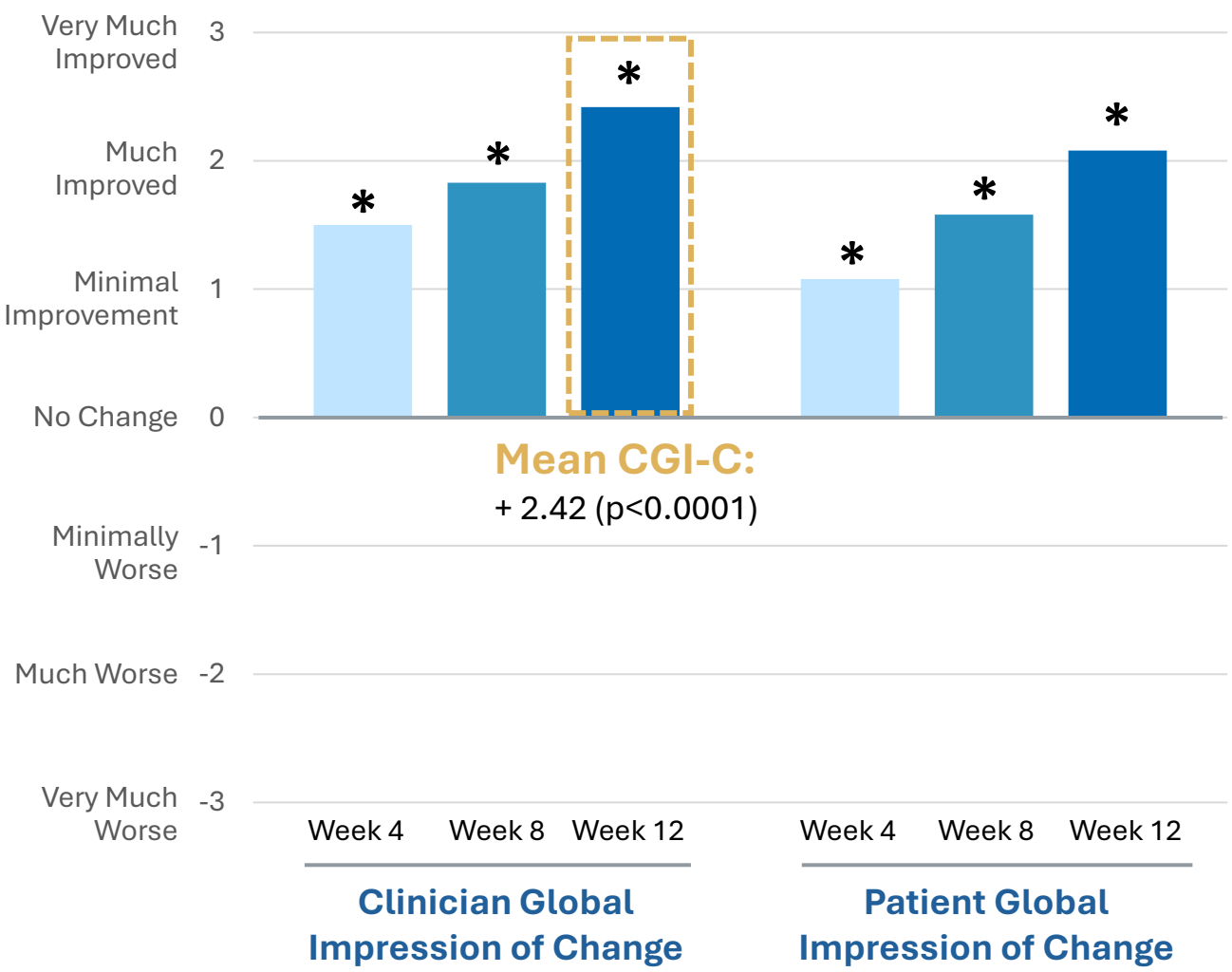
Types	Microcystic
Subtotal	28
Sex (F:M)	14:14
Age (y), mean ± SD	0.89 ± 1.4
Maximum diameter (cm), mean ± SD	—
Spontaneous regression	
Positive	0
Negative	28

** Consistent with well-established history of PI3K Related Overgrowth Spectrum, which includes microcystic LM*
***Kato M et al., Plast Reconstr Surg Glob Open. 2017 Sep 25;5(9):e1501.*

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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)

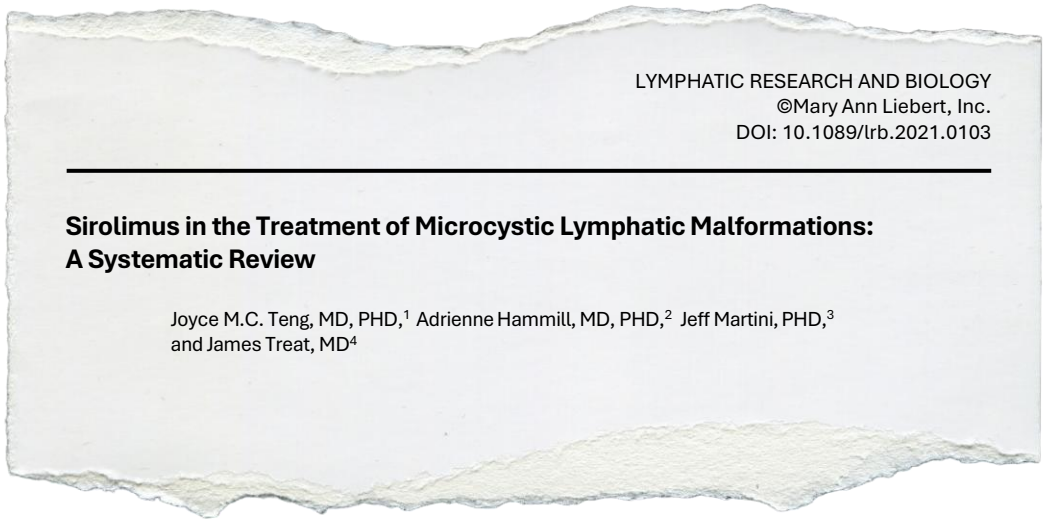
→ **100% of participants were either "Much Improved" or "Very Much Improved"** on CGI-C after 12 weeks of treatment

→ **Palvella was subsequently granted FDA Breakthrough Therapy Designation** based on this data

* = p-value <0.0001

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Regulatory: Real-world Evidence to Support NDA Submission



LYMPHATIC RESEARCH AND BIOLOGY
©Mary Ann Liebert, Inc.
DOI: 10.1089/lrb.2021.0103

Sirolimus in the Treatment of Microcystic Lymphatic Malformations: A Systematic Review

Joyce M.C. Teng, MD, PHD,[1] Adrienne Hammill, MD, PHD,[2] Jeff Martini, PHD,[3] and James Treat, MD[4]

"Micro LMs represent therapeutically challenging congenital vascular lesions. There is no universally accepted gold standard of care and there are no FDA approved therapies…this review examines clinical data over the last 10 years on the role of sirolimus [rapamycin]…a total of 16 studies were identified…clinically meaningful, long-term improvement (up to 3 years) was noted…however, developing a commercial topical sirolimus formulation faces important challenges."



Leboulanger *et al.*
Orphanet Journal of Rare Diseases (2023) 18:10
https://doi.org/10.1186/s13023-022-02608-y

French national diagnosis and care protocol (PNDS, protocole national de diagnostic et de soins): cystic lymphatic malformations

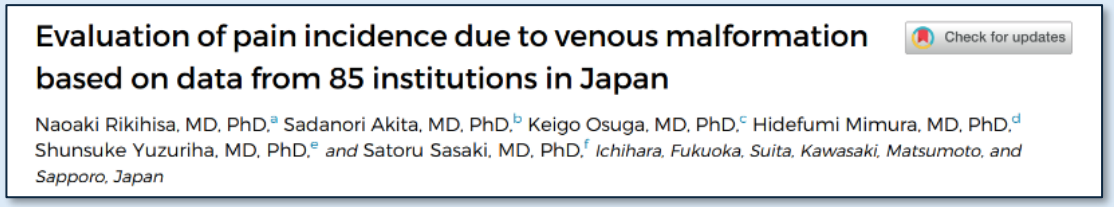
Nicolas Leboulanger[1,2*], Annouk Bisdorff[3], Olivia Boccara[4], Anne Dompmartin[5], Laurent Guibaud[6], Christine Labreze[7], Jacques Lagier[8], Bénédicte Lebrun-Vignes[9], Denis Herbreteau[10], Aline Joly[11], Julie Malloizel-Delaunay[12], Arnaud Martel[8], Stéphane Munck[13], Frédérique Saint-Aubin[14] and Annabel Maruani[15,16]

"Sirolimus [rapamycin] is the disease-modifying treatment of choice. It should be started early in life (early childhood) to prevent the increase in volume of the LM."

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Multiple Studies Indicate at Least Half of Venous Malformation Cases Have Cutaneous Involvement

Percentage of venous malformation cases with cutaneous involvement

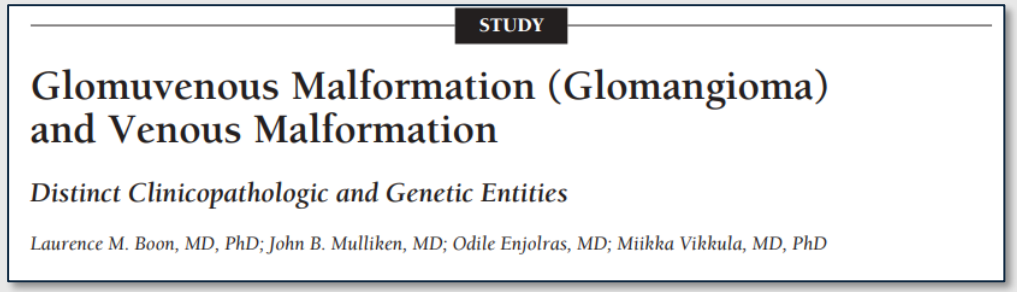


Evaluation of pain incidence due to venous malformation based on data from 85 institutions in Japan

Check for updates

Naoaki Rikihisa, MD, PhD,[a] Sadanori Akita, MD, PhD,[b] Keigo Osuga, MD, PhD,[c] Hidefumi Mimura, MD, PhD,[d] Shunsuke Yuzuriha, MD, PhD,[e] *and* Satoru Sasaki, MD, PhD,[f] *Ichihara, Fukuoka, Suita, Kawasaki, Matsumoto, and Sapporo, Japan*

~47% of 2,075 cases



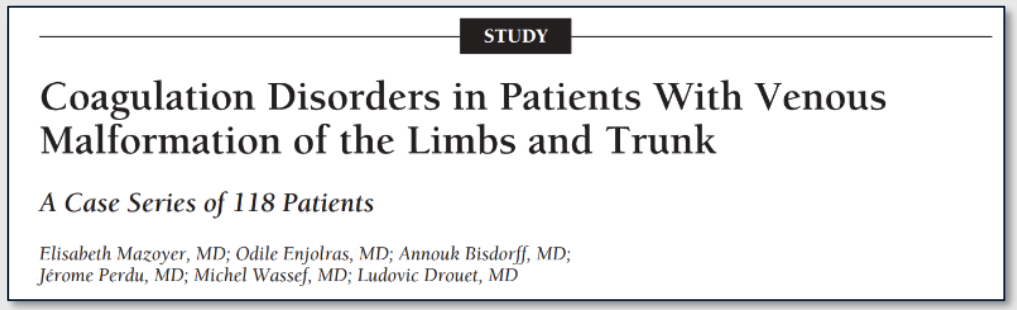
STUDY

Glomuvenous Malformation (Glomangioma) and Venous Malformation

Distinct Clinicopathologic and Genetic Entities

Laurence M. Boon, MD, PhD; John B. Mulliken, MD; Odile Enjolras, MD; Miikka Vikkula, MD, PhD

~80% of 1,517 cases



STUDY

Coagulation Disorders in Patients With Venous Malformation of the Limbs and Trunk

A Case Series of 118 Patients

Elisabeth Mazoyer, MD; Odile Enjolras, MD; Annouk Bisdorff, MD; Jérome Perdu, MD; Michel Wassef, MD; Ludovic Drouet, MD

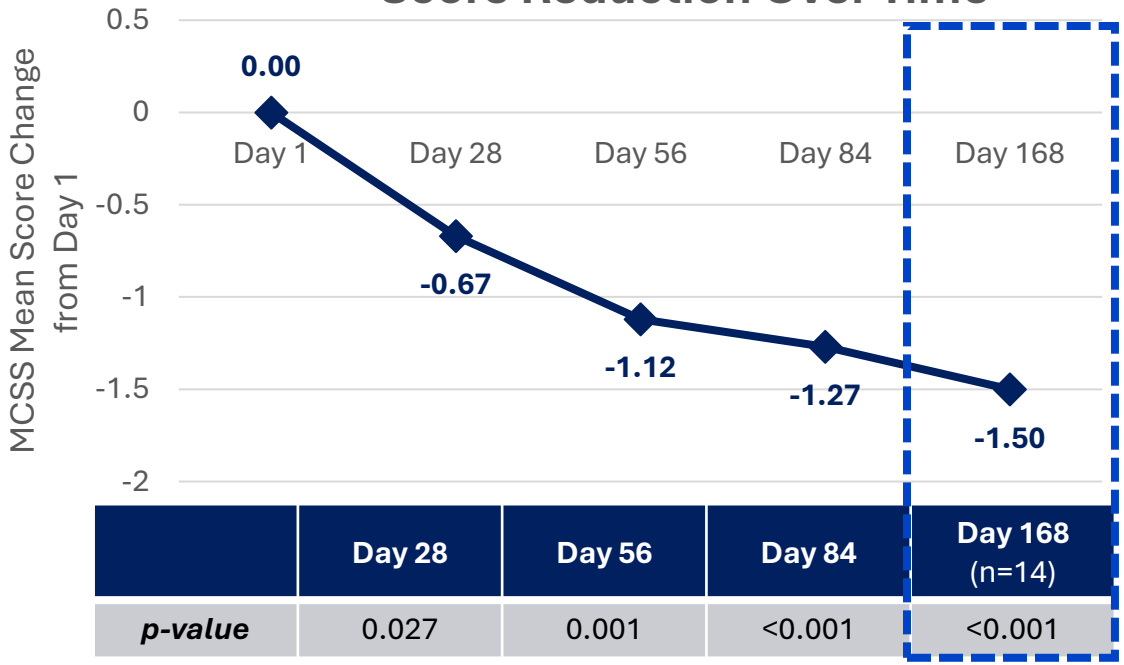
~65% of 118 cases

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New TOIVA Data: Height/Engorgement and Appearance Improvements Continued Through 24 Weeks Demonstrating Deepening of Treatment Effect Over Time

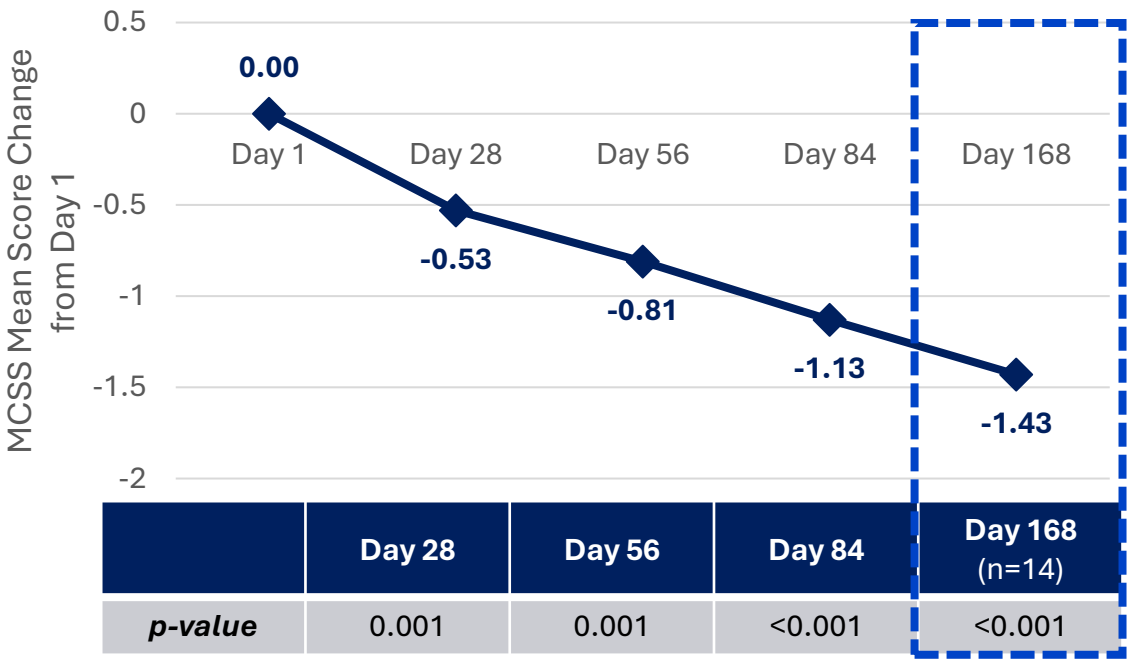
Lesion height/engorgement and appearance are key manifestations of cVM disease burden and clinically meaningful indicators of disease impact

cVM-MCSS Height/Engorgement Score Reduction Over Time



	Day 28	Day 56	Day 84	Day 168 (n=14)
p-value	0.027	0.001	<0.001	<0.001

cVM-MCSS Appearance Score Reduction Over Time



	Day 28	Day 56	Day 84	Day 168 (n=14)
p-value	0.001	0.001	<0.001	<0.001

cVM-MCSS (cutaneous VM multi-component static scale) Height and Appearance are rated on 5-point scales ranging from 1 to 5; negative values indicate improvements from Day 1

Note: Mean change and p-values collected on observed data only.

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Clinically Significant Angiokeratomas:
Superficial Lymphatic Malformations

Shared Clinical Features between Clinically Significant Angiokeratomas and Microcystic LMs



Angiokeratomas

✓	Lymphatic origin[1,5]	✓
✓	Scientific rationale and real-world evidence supporting targeted therapy with rapamycin[2,5]	✓
✓	Superficial dermal location[3,5]	✓
✓	Clinically impactful, causing bleeding, functional impairment, and risk of infection[4,5]	✓



Microcystic LMs

1. Trindade F, et al. *Am J Dermatopathol*, Sep 30, 2014. 2. Bell KA, et al. *JAAD Case Reports*, Nov 30, 2020; Camacho I, et al. *Dermatologic Therapy*, Jun 27, 2020; Moeineddin F, et al. *Clinical Case Reports*, May 31, 2024; Farajzadeh S, et al. *Indian J Dermatol Venereol Leprol*, Jun 20, 2023; Fernández Ginés Fd, et al. *Eur J Hosp Pharm*, Feb 28, 2018; Kang Y, et al. *J Korean Association of Oral Maxillofacial Surgery* July 12, 2014. 3. Trindade F, et al. *Am J Dermatopathol*, Sep 30, 2014. 4. Philip C, et al. *Dermatological Therapeutics* March 31, 2020; Hobbs et al, *Journal of Dermatology Surg Onco*, 1987. 5. Teng et al, *Lymphatic Research and Biology*, 2022.

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New Articles Emphasize the Unmet Need of Clinically Significant Angiokeratomas

Journal of Paediatrics and Child Health 62, no. 2 (2026): 298–300, https://doi.org/10.1111/jpc.70261.

Fordyce Angiokeratoma: An Unusual Localised Form in Childhood

M. F. Reis, J. Lage, C. Ferreira, B. Henriques, and C. Amaro

- Angiokeratomas proliferate during adolescence
- Present with bleeding, pain, pruritus
- Identifies mTOR inhibition as therapeutic option

Journal of the American Academy of Dermatology 2026 Jul 17:S0190-9622(26)03107-5. https://doi.org/10.1016/j.jaad.2026.07.033

Penile angiokeratomas (PEAKERs) and clitoral angiokeratomas (CLANKERs) are unique variants of genital angiokeratomas

Cohen PR

- Highlights the clinical spectrum of genital angiokeratomas
- Most common symptom is bleeding
- Treatment remains limited to destructive procedures underscoring the need for a non-invasive targeted therapy

Pediatric Dermatology 43, no. 1 (2026): 217–219 https://doi.org/10.1111/pde.70059

A Case of a Solitary Angiokeratoma Circumscriptum of the Lower Leg

C. Chang, C. Pak, M. Braniecki, O. B. Omoleye, and M. Bain

- Angiokeratoma circumscriptum are persistent, pruritic, hyperkeratotic plaques characterized by epidermal acanthosis and hyperkeratosis
- Lesions do not spontaneously resolve, and minor trauma can lead to recurrent bleeding

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Attractive U.S. Commercial Opportunity for DSAP

>50k

diagnosed DSAP patients in the U.S. based on two sources (Clarity Pharma, n=277 physicians; Zagoras et al, 2023) and confirmed by KOL calls

Orphan Pricing

anticipated based on disease severity and lack of FDA-approved therapies

Market research (n=55 physicians)[1]:

100%

of physicians would incorporate Product X (topical mevalonate pathway inhibitor) into their practice

96%

of physicians would consider Product X as a first line therapy for DSAP patients

1. MedaCorp Survey (August 2025), n=55 physicians surveyed.

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