



First-in-disease therapies for patients  
with rare diseases



Q1 2026 Financial Results & Corporate Update  
May 7, 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 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 planned regulatory framework for the Company’s product candidates, the Company’s expectations regarding the benefits of orphan drug designation and potential benefit of orphan drug exclusivity for QTORIN™ rapamycin for the treatment of microcystic lymphatic malformations, the Company’s ability and the ability of third-party manufacturers the Company engages to optimize and scale manufacturing, 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.

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# Q1 2026 Achievements Position Company for First Potential Approval and Launch of QTORIN™ Rapamycin

## Microcystic Lymphatic Malformations: Advancing Towards Potential 1H 2027 Approval



Phase 3 results exceeded upside case profile

NDA remains *on track* for 2H 2026; pre-NDA meeting *granted* for Q2 2026

Exceptional veteran commercial leadership recruited; launch planning accelerating

## Cutaneous VMs



Phase 3 initiation on track

## Angiokeratomas



Accelerated towards first patients dosed ahead of schedule

## DSAP



>40 patient inbounds for clinical trial  
Review published in *Clinical and Experimental Dermatology*

**\$230mm raised in gross proceeds in upsized equity financing**

# Palvella: Vision and Leadership in Treating Serious, Rare Skin Diseases

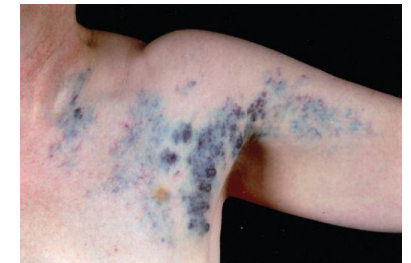
*“We have begun to see interest from investors and companies in developing treatments for a rare disease such as epidermolysis bullosa, but there are many other diseases within dermatology that remain unaddressed”*

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

**597**

Rare Skin  
Diseases

Often serious and chronically debilitating



Over  
**98%**

Have **No** Approved  
Therapies<sup>1</sup>

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



# Palvella: Driving the Next Wave of Innovation in Under-appreciated Rare Skin Diseases

## Diagnosed U.S. Prevalence<sup>1</sup>

30k+

Microcystic LMs

75k+

Cutaneous VMs

50k+

Clinically Significant  
Angiokeratomas

50k+

Disseminated Superficial  
Actinic Porokeratosis

## # of FDA-approved Therapies

None

## Market Research: % Physicians Who Would Consider QTORIN™ as First-line Therapy<sup>2</sup>

>80% across four indications

# Positive Clinical Data and Upsized Equity Financing Enable Accelerated U.S. Launch Planning

1

selva toiva

Positive Phase 3 data in microcystic LMs and positive Phase 2 data in cutaneous VMs

2

**\$230 million**

Gross proceeds in upsized equity financing

Attracting proven commercial talents



**Jen McDonough**  
SVP Market Access  
*Prev. Krystal Biotech*



**Kent Taylor**  
SVP Sales  
*Prev. Arcutis*

Targeting higher end of range for field sales force (30-40 reps) with plans to hire prior to PDUFA

Two MSLs hired, with plans for additional hires, to drive disease state awareness

Engaging top 400 high-volume centers, including VACs\*

# QTORIN™ Rapamycin: >\$1bn U.S. Peak Sales Potential in Microcystic LMs



## Microcystic Lymphatic Malformations

>30k diagnosed U.S. patients,  
with concentration in vascular  
anomaly centers

Recent field checks, annual  
incidence estimates, published  
claims analysis

Annual ~\$100k-\$200k pricing range  
per patient

Supported by Phase 3 SELVA data,  
payor testing, analogues

**QTORIN™ Rapamycin Potential to Achieve >\$1bn Peak Sales**



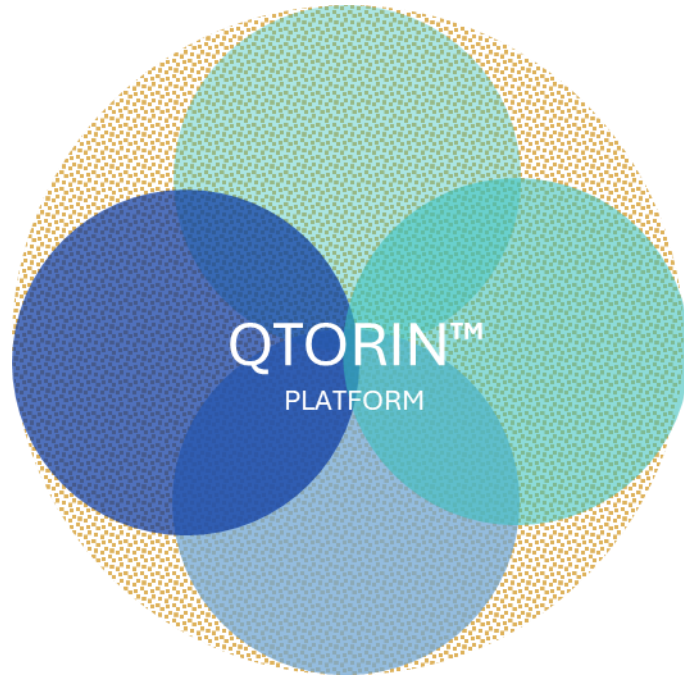
OUR PLATFORM

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QTORIN™

palvella  
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

Rapid pipeline expansion with capital- and time-efficient QTORIN™ programs

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# Rare Disease Pipeline Overview

# SELVA: Additional Analyses Support Clinical Meaningfulness

A pre-specified embedded patient interview sub-study provided qualitative evidence supporting the clinical meaningfulness of the improvements observed in SELVA

Week 24

“

*It doesn't bleed anymore and it's really clear... It just looks like my normal skin.*

*...it doesn't leak constantly like it used to.*

*[The lesion] became closer to the color of my skin tone before it fully went away*

”

## KEY INSIGHTS

**Improvements in disease burden were consistently reported by patients**

**Interviews reinforced that SELVA endpoints captured meaningful changes to patients' lives**

**Patient findings strengthen overall risk/benefit narrative for planned NDA submission**

# Planned 505(b)(2)-Enabled NDA Submission

*Pre-NDA meeting granted by FDA, with meeting expected Q2 2026*



**Planned 505(b)(2) submission: leveraging FDA's prior findings for rapamycin to streamline review process**



**Real-world studies from off-label rapamycin use as supportive evidence**



**Seeking traditional approval based on clinical endpoints (not biomarkers for accelerated approval)**



**Two prospective studies with statistical significance, with 95% of patients improving on mLM-IGA at Week 24 in SELVA**

**Breakthrough  
Therapy  
Designation**

**Fast  
Track  
Designation**

**Orphan  
Drug  
Designation**

**Supported by FDA Orphan Products Grant:**  
Two tranches of non-dilutive funding received

# Strong Medical Affairs Presence with Sponsorship and Presentations at Key Medical Congresses

## Platinum Sponsor at ISSVA World Congress (May 19-22, 2026)



**JAMES TREAT, MD**

Professor of Clinical  
Pediatrics and  
Dermatology, CHOP

### LATE-BREAKER PRESENTATION

- **Title:** *QTORIN™ 3.9% Rapamycin Anhydrous Gel: Statistically Significant, Clinically Meaningful Improvement in Microcystic Lymphatic Malformations (Phase 3 SELVA Study) and Cutaneous Venous Malformations (Phase 2 TOIVA Study)*
- **Date/Time:** Wednesday, May 20, 2026 at 4:30pm ET

### SCIENTIFIC SYMPOSIUM

- **Title:** *Clinical Development in Rare Cutaneous Vascular Disorders: Lessons Learned from SELVA and TOIVA Trials*
- **Date/Time:** Thursday, May 21, 2026 at 12:30pm ET

### BEYOND mLM LOUNGE

Attendees can participate in discussions on mLM and sign up to receive educational resources and communications for both themselves and their patients

### Planned Presentations at Additional Medical Congresses:



**Epidermal Differentiation  
Disorders Symposium (Platinum Sponsor)**  
May 12, 2026, Chicago, IL



Jul 22-25, 2026  
Minneapolis, MN



Oct 15-17, 2026  
Alexandria, VA

# Cutaneous Venous Malformations: 73% of Patients Improved in Phase 2

POTENTIAL TO BE FIRST FDA-APPROVED THERAPY

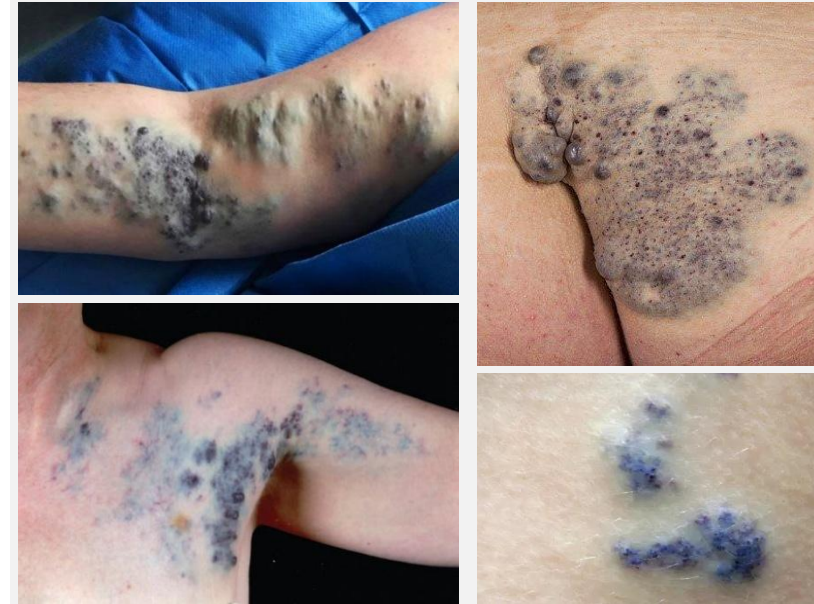
- **Why this fits Palvella's strategy:**
  - Serious, rare, mTOR-driven
  - Meaningful overlap with microcystic LM physicians at centers of excellence
- **On track for Phase 3 trial initiation in 2H 2026**
- **Additional findings from Phase 2 TOIVA study to be presented at ISSVA World Congress later this month**

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Market research (n=50 physicians): **86%** would consider QTORIN™ rapamycin as first-line therapy for cutaneous VMs

> 75k patients

ESTIMATED DIAGNOSED IN THE U.S.



**No FDA-approved therapies**

Fast Track Designation Granted

Breakthrough Therapy Designation  
application submitted

Pipeline-in-a-product:  
sNDA planned

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# Clinically Significant Angiokeratomas: Superficial Lymphatic Malformations

## Shared Clinical Features between Clinically Significant Angiokeratomas and Microcystic LMs



**Angiokeratomas**



**Microcystic LMs**



Lymphatic origin<sup>1,5</sup>



Scientific rationale and real-world evidence supporting targeted therapy with rapamycin<sup>2,5</sup>



Superficial dermal location<sup>3,5</sup>



Clinically impactful, causing bleeding, functional impairment, and risk of infection<sup>4,5</sup>



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.

# Clinically Significant Angiokeratomas: Superficial Lymphatic Malformations



> 50k patients

ESTIMATED DIAGNOSED IN THE U.S.

FIRST PATIENTS DOSED IN PHASE 2 TRIAL MAY 2026

- **A type of isolated lymphatic malformation: direct scientific adjacency to microcystic LMs**
- FDA granted Fast Track Designation in Dec 2025
- Phase 2 LOTU study is a single-arm, baseline-controlled clinical trial evaluating QTORIN™ rapamycin applied topically once daily
  - Enrolling up to 15 patients
- Topline results are expected in 2H 2027

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Market research (n=50 physicians): **96%** would incorporate QTORIN™ rapamycin into their practice



**No FDA-approved therapies**

**Fast Track Designation  
Granted**

**Pipeline-in-a-product:  
sNDA planned**

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

POTENTIAL TO BE FIRST FDA-APPROVED THERAPY AND STANDARD OF CARE

QTORIN™  
PITAVASTATIN

- **First pathogenesis-directed therapy targeting the casual mevalonate pathway**
- **Phase 2 initiation on track for 2H 2026**
- **Strong patient interest for planned Phase 2 study; over 40 inbound patient inquiries, including:**

“I tried several unsuccessful treatments. This is a disfiguring... potentially cancerous condition. After years of waiting, the prospect of a successful treatment is exciting. If I can be part of the solution by participating in a clinical trial, count me in.”

“Having tried just about everything to treat this rare disorder, I am EXCEPTIONALLY interested in your clinical trial...I am desperate for any treatment”

> 50k patients

ESTIMATED DIAGNOSED IN THE U.S.



## **No FDA-approved therapies**

### **Current options:**

Laser, surgery, and off-label topical chemo agents & mevalonate pathway inhibitors

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

## QTORIN™ Rapamycin

Microcystic LMs, Cutaneous VMs, Clinically Significant Angiokeratomas
✓
✓
✓
✓
✓
✓

## QTORIN™ Pitavastatin

DSAP
Chronic, extensive lesions, malignant transformation
>50k U.S. patients
None
Mutations in mevalonate pathway lead to accumulation of toxic intermediates
Multiple published case studies + use in academic centers
Opportunity to be first-in-disease and SOC

- Serious
- Rare
- No FDA-approved Therapies
- Strong Scientific & Biologic Rationale
- Published Case Studies & Off-label Use of API
- Commercially Attractive

Significant unmet medical need

Optimizing likelihood of clinical success

Multi-billion dollar U.S. TAM<sup>1</sup>



1. Based on internal and third-party estimates.

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# Finance & Upcoming Catalysts

# Well-Capitalized with High-Quality Investor Participation in \$230mm Oversubscribed Feb. 2026 Financing Following Positive SELVA Data

# \$262 million

3/31/26 cash

## Potential to Fund Through:

- NDA filing, FDA approval, and, if approved, U.S. launch for QTORIN™ rapamycin in microcystic LMs
- NDA filing for QTORIN™ rapamycin in cutaneous VMs
- Multiple Phase 2 data readouts from pipeline programs

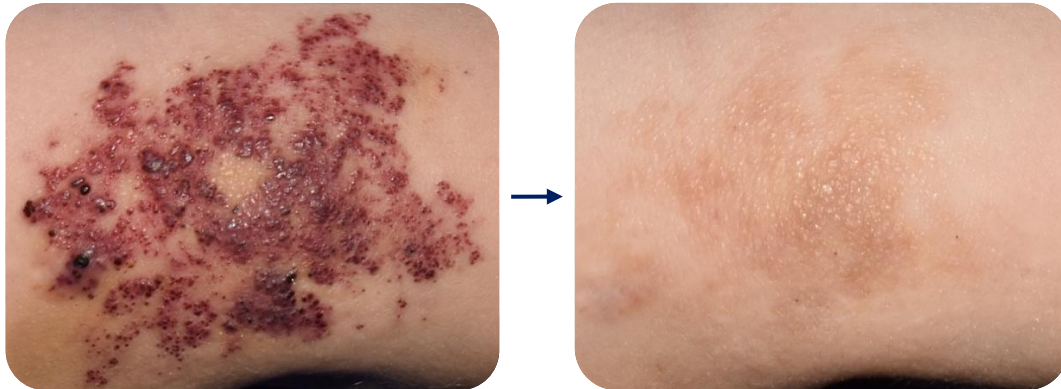
**QTORIN™ + 505(b)(2) + rare disease focus offers potential for attractive ROI**

# Microcystic Lymphatic Malformations: NDA Submission on Track for 2H 2026 with Anticipated U.S. Commercialization in 1H 2027

1

## Microcystic Lymphatic Malformations

- ✓ Positive Phase 3 SELVA data
- ✓ In-person pre-NDA meeting granted by FDA, to occur in Q2 2026
- Findings to be presented at ISSVA World Congress, May 2026
- NDA submission on track for 2H 2026



**Potential FDA approval and U.S. launch 1H 2027**

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

2

## Cutaneous Venous Malformations

- ✓ Positive Phase 2 data
- ✓ BTD application submitted Q2 2026
- P3 initiation expected 2H 2026



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- Phase 2 data expected 2H 2027



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## Disseminated Superficial Actinic Porokeratosis

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



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## QTORIN™ Third Program

- Announcement expected 2H 2026
- Potential pipeline-in-a-product



**DAVID OSBORNE, PhD**  
Chief Innovation Officer

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**DAVID OSBORNE, PhD**  
Chief Innovation Officer

6

## QTORIN™ Rapamycin Fourth Indication

- QTORIN™ rapamycin fourth indication announcement expected 2H 2026



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# Closing Remarks

# What Makes Palvella Stand Apart



**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**

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

A young child with light hair, wearing blue swimming goggles, is swimming in a pool. The child is smiling and has their arms outstretched. The water is clear and blue, with some bubbles around the child. The background shows the edge of the pool with a decorative border.

**PALVELLA** (paluel:a, 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***



# Thank You

*Striving to be first for rare disease patients*

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