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

Q1 2025 Financial Results & Corporate Update
May 15, 2025

# Forward Looking Statements

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

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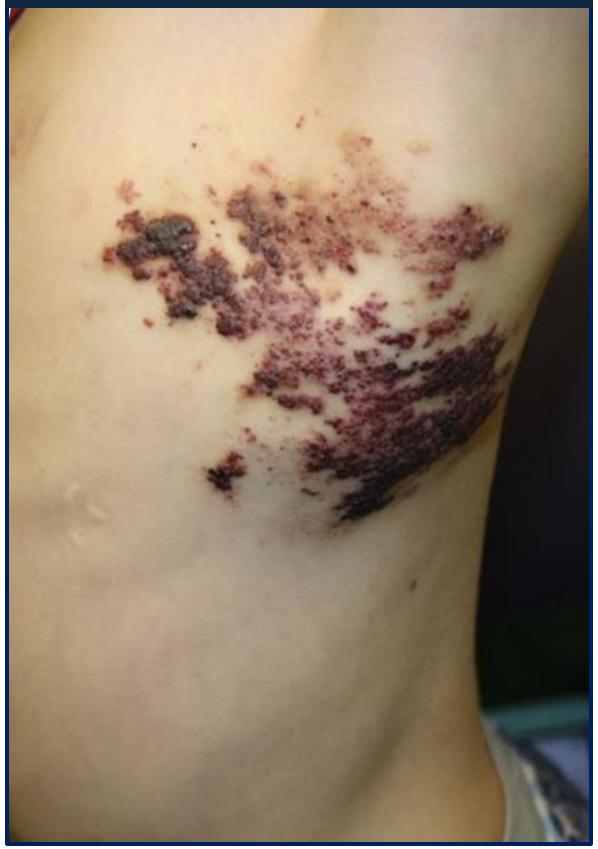
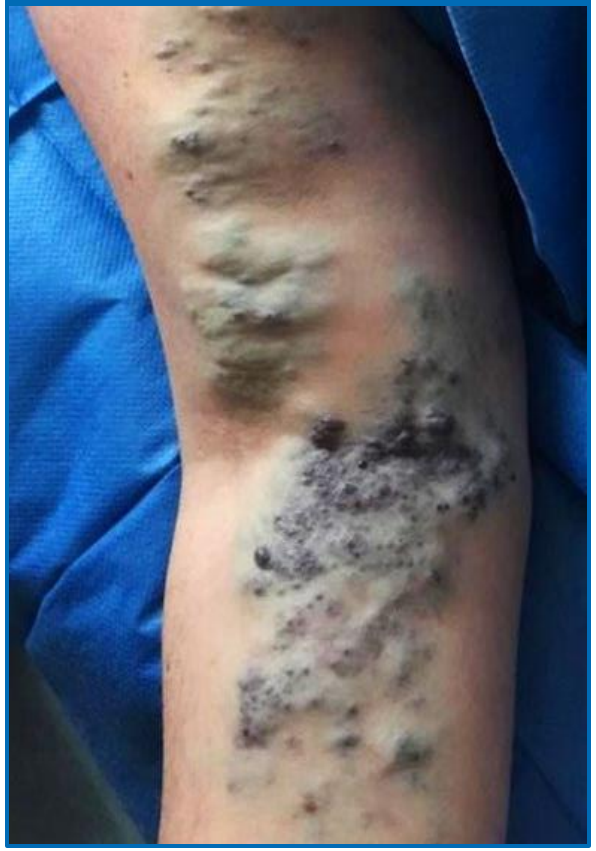
# Multiple High-Impact Milestones Over Next 4 Quarters

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



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



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


**New QTORIN™ Program** (2H:25)




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

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# Continued Strong Momentum at Palvella

- **QTORIN™ rapamycin for microcystic LMs: Phase 3 SELVA trial**
  - Exceeded enrollment target of 40 patients; enrollment expected to close in June 2025
  - Top-line readout anticipated Q1 2026

- **Phase 2 TOIVA study on track in cutaneous VMs**
  - 6 sites open and enrolling
  - Top-line readout anticipated Q4 2025
- **Insights from SID Meeting and ISSVA Conference: support significant unmet need and attractive commercial opportunity in microcystic LMs**
- **Fortifying leadership team in anticipation of potential U.S. commercialization**
  - Hired Jason Burdette as SVP, CMC & Technical Operations (Jan 2025)
  - Chief Commercial Officer recruitment ongoing; planned hire in 2H 2025
- **Strengthening patent position: 5th U.S. patent issuance with claims into 2038**
  - Patents augmented by trade secrets and anticipated seven-year orphan exclusivity



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LMs: lymphatic malformations. VMs: venous malformations. SID: Society of Investigative Dermatology. ISSVA: International Society for the Study of Vascular Anomalies.

QTORIN™ 3.9% RAPAMYCIN

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# Microcystic Lymphatic Malformations

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# Phase 3 SELVA Enrollment Exceeded Target of 40 Patients

## Single-arm, baseline-controlled, QD dose

**Enrollment expected to close in June 2025; top-line readout anticipated Q1 2026**

**Joyce Teng, MD, PhD**
Principal Investigator
Stanford MEDICINE

Children's Hospital of Philadelphia (Sally Cohen-Cutler, MD)

Cincinnati Children's (Kiersten Ricci, MD)

VANDERBILT HEALTH (Neeraja Swaminathan, MD)

UNC HEALTH (Elizabeth Nieman, MD)

Cleveland Clinic (Mike Kelly, MD, PhD)

Children's Healthcare of Atlanta (Michael White, MD)

MAYO CLINIC (Megha Tollefson, MD)

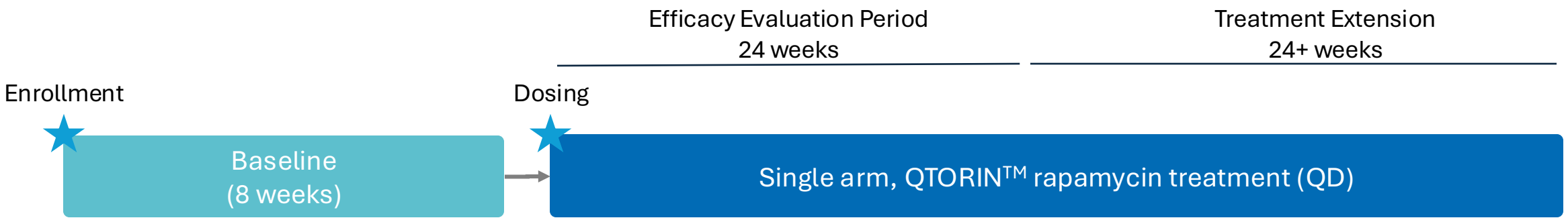
MINNESOTA CLINICAL STUDY CENTER (Steve Kempers, MD)

UCI (Maria Buethe, MD, PhD)

Texas Children's Hospital (Ionela Iacobas, MD)

PennState Health (Andrea Zaenglein, MD)

HEALTH UNIVERSITY OF UTAH (Amy Theos, MD)



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# QTORIN™ Rapamycin for Treatment of mLMs: Regulatory Overview

## Consistent and productive engagement with FDA on development program

- **FDA Overview:**
  - **Center:** Center for Drug Evaluation Research (CDER)
  - **Division:** Dermatology and Dentistry
  - **Division Leadership:** Dr. Jill Lindstrom remains in Director role
  - **NDA Review and Signature:** Due to planned 505(b)(2) pathway, division leadership is responsible for NDA decision

- **Palvella is anticipating expedited pathway to submission given Breakthrough, Fast Track and Orphan Drug Designations and 505(b)(2) pathway**


Hyman, Phelps & McNamara PC

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

By Sarah Wicks & James E. Valentine

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

- **Other FDA dynamics for pipeline programs**
  - New potential accelerated pathway for rare and ultra rare disease drugs based on a "plausible mechanism" announced by Commissioner Makary



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# QTORIN™ Rapamycin for Treatment of mLMs: Commercial Opportunity


**Incidence, prevalence, and care for patients with lymphatic malformations (LMs) in the U.S.: A claims-based analysis**

Ashley Kline,[1,*] David Lapidus,[1] Katherine Tsai,[1,2] Ionela Iacobas[3]

[1]Consultant to Palvella Therapeutics, Wayne, PA 19087; [2]Employee of ZS Associates, Evanston, IL 60201; [3]Cancer and Hematology Center, Texas Children's Hospital, Baylor College of Medicine, Houston, TX 77030
*corresponding author e-mail: ashley.kline@palvellatx.com

| Est. Diagnosed U.S. Prevalence | Estimated U.S. Incidence | Concentration |
|---|---|---|
| > 44k | ~1,500 annually or more | *~1/3 of patients treated at institutions with VACs (~150 centers)* |

**Orphan pricing anticipated**

*Prior first-in-disease launches and recent topical orphan launches both support orphan drug pricing*

**Multi-Billion Dollar Total Addressable Market (TAM) Currently With No FDA-Approved Therapies**



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# ISSVA Conference 2025: April 23rd-25th in Paris, France


- Treatment paradigm rapidly evolving to **targeted pharmacotherapy** approaches, replacing surgery and sclerotherapy
- **Off-label systemic agents, incl. oral PI3K inhibitors, introduce unacceptable side effects** (e.g., growth retardation) for diseases that locally present on the skin – significant unmet needs exist for targeted, topical therapies

***Strong KOL support for QTORIN™ Rapamycin and other potential QTORIN™ programs***

- **Identification of additional high unmet need clinical indications** that could be future disease targets for Palvella



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# Cutaneous Venous Malformations

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# Phase 2 TOIVA Study in cVMs: Enrollment Ongoing

## Single-arm, baseline-controlled, QD dose, age 6+, 12 weeks, n=~15

**6 sites open and enrolling, including 5 sites opened in last two months**

**Megha Tollefson, MD**
Principal Investigator

Stanford MEDICINE
(Joyce Teng, MD, PhD)

UNC HEALTH
(Elizabeth Nieman, MD)

UCI
(Maria Buethe, MD, PhD)

HEALTH UNIVERSITY OF UTAH
(Amy Theos, MD)

MINNESOTA CLINICAL STUDY CENTER
(Steve Kempers, MD)

Children's Hospital Colorado
(Taizo Nakano, MD)

**Safety**

- Safety and tolerability

**Efficacy**

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

**Topline data anticipated Q4 2025**



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# Key Value Drivers from Pipeline Programs in Second Half of 2025

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


## QTORIN™ rapamycin next indication

- Serious, rare, no FDA-approved therapies
- mTOR drives disease pathology
- Commercially attractive

## New QTORIN™ program

- Serious, rare, no FDA-approved therapies
- Well-defined genetics
- Clear biology
- Commercially attractive
- Targeting <$10mm and <2.5 years to Phase 2 POC data

**New QTORIN™ Program** (2H:25)




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# Financial Update

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# Q1 2025 Financial Highlights and 2025 Outlook

## Strong Cash Position

**2+ years**

Runway into 2H 2027

**$75.6 million**

Cash at 3/31/2025

**$7.9 million**

R&D + G&A spend in Q1 2025

**>$55 million**

Projected cash at year end

## Oversubscribed PIPE Financing (Dec. 2024)

BVF PARTNERS L.P.

FRAZIER LIFE SCIENCES

BLUE OWL

LIGAND

CAMCapital
CAXTON ALTERNATIVE MANAGEMENT

PETRICHOR

SAMSARA BIOCAPITAL



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# Q&A

*Striving to be first for rare disease patients*

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