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First-in-disease therapies for patients with rare diseases



Q3 2026 Financial Results & Corporate Update
November 5, 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 with respect to the potential approval of the NDA for QTORIN™ rapamycin, the potential impact of Priority Review on the FDA's review timeline, any delays in the FDA's review of Palvella's accepted NDA, 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 commercial preparedness and the timing and execution of a potential launch following regulatory approval, 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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Industry and Market Data

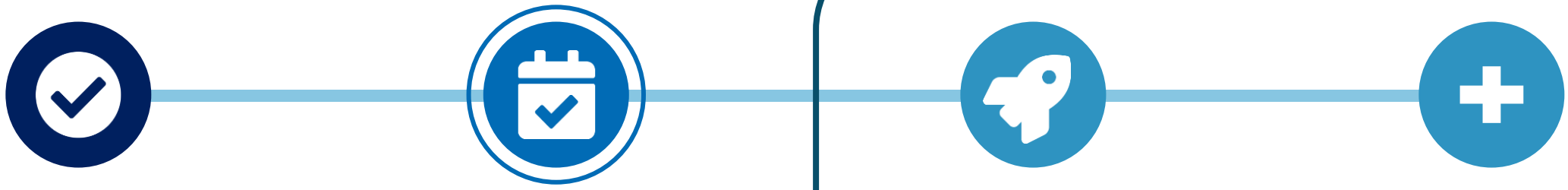
The Company may from time to time provide estimates, projections and other information concerning its industry, the general business environment, and the markets for certain conditions, including estimates regarding the potential size of those markets and the estimated incidence and prevalence of certain 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.

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QTORIN™ Rapamycin:
Microcystic LM Opens the Door to Many mTOR-driven Diseases



October 2026

NDA Accepted with Priority Review

February 28, 2027

PDUFA Target Action Date

Potentially first FDA approved mLM therapy

1H 2027

Standalone U.S. Launch if Approved

>30,000 estimated diagnosed U.S. patients[1]

2029+

Additional Potential Approvals in mTOR-Driven Diseases

>300,000 U.S. patients across diseases[1]

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

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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. **Zero to One: 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**

Shaping an Exceptional Launch for mLM Patients

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Proven Orphan Launch Leadership, Applied With Urgency

Significant progress across crucial keys to launch success



Ashley Kline
Chief Commercial Officer

- **Led U.S. launch of Oxervate®** a topical medicine for neurotrophic keratitis, a rare eye disease
- **Oxervate® reached >$1 billion** in U.S. sales seven years after launch
- **Joined Palvella ~18 months ago**; prior roles at Dompé, Genentech, and Bain Consulting

Keys to a Successful Orphan Launch

Strong Prescribing Intent
>70% plan to prescribe within 3 months of approval

Clear Path to Coverage
Payer research has confirmed pricing and access assumptions

Precision Patient-Finding
Multiple patient identification approaches deployed

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New Market Research Reinforces Potential for Strong Orphan Launch

Post-SELVA market research further substantiates previous launch positioning

Physician Survey

(~150 treating U.S. physicians)

>70%

of surveyed physicians expect to begin prescribing within 3 months of approval

>75%

would start several patients at once, then expand

Payer Survey

(10 U.S. payers)

- mLM is recognized as a serious, genetic vascular anomaly
- Product profile supported by compelling clinical evidence package
- Clinical diagnosis with no step-through anticipated
- Favorable coverage anticipated at orphan pricing corridor

Responses weighted by patient volume and adjusted for overstatement. Physicians evaluated a blinded product profile. Source: Palvella-sponsored market research (survey of 150 U.S. physicians; interviews with 14 physicians and 10 payers), April–June 2026.

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Proven Launch Leaders Recruited to Serve the mLM Community

Anticipated launch in 1H 2027

Leadership team brings deep expertise in both **rare disease** and **dermatology**

>70

field-based team members at launch

Sales

Focused on winners from first-in-indication launches

Field Marketing

HCP education beyond sales call points

In-House Patient Team

Personalized support for patients

Medical Affairs

Deep discussions with providers on our science

Precision Digital Effort

Physician National Prescriber Identification (NPI)-targeted strategy informed by claims data

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QTORIN™ Rapamycin: Opportunity to be the First FDA-approved Therapy for Estimated >30K Microcystic LM Patients in the U.S.

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.

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Rare Disease Pipeline Updates

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FDA Rare Disease Framework Public Meeting (October 2026)

REAGAN-UDALL FOUNDATION for the Food and Drug Administration

FDA PUBLIC MEETING

Rare Disease Framework Initiative Public Meeting

October 28, 2026

FDA White Oak Campus, Silver Spring, MD
Hosted by the Reagan-Udall Foundation for the FDA

> ***"Dermatology has more rare diseases than any other specialty."***

Dr. Matthew Zirwas, Board-certified dermatologist

Public comment, October 28, 2026

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Deep Pipeline in Serious, Rare Skin Diseases and Vascular Anomalies

PRODUCT CANDIDATE	DISEASE	ESTIMATED U.S. PREVALENCE[1]	CLINICAL STATUS	FDA DESIGNATIONS	UPCOMING MILESTONES
QTORIN™ Rapamycin *3.9% anhydrous gel*	Microcystic Lymphatic Malformations	>30,000	PDUFA: Feb 28, 2027	• Breakthrough Therapy • Fast Track • Orphan	PDUFA: Feb 28, 2027
	Cutaneous Venous Malformations	>75,000	Phase 2 Completed	Fast Track	Phase 3 trial initiation expected Q4 2026
	Clinically Significant Angiokeratomas	>50,000	Phase 2 Ongoing	Fast Track	Phase 2 data expected 2H 2027
	Fourth QTORIN™ rapamycin indication	*TBD*	Phase 2 Ready	Planned	Announcement expected Q4 2026
QTORIN™ Pitavastatin	Disseminated Superficial Actinic Porokeratosis (DSAP)	>50,000	Phase 2 Ready	Planned	Phase 2 trial initiation expected Q4 2026
Third QTORIN™ Program	*Serious, rare disease*	*TBD*		Planned	Announcement expected Q4 2026
Additional New QTORIN™ Programs	*Serious, rare diseases*	*TBD*		Planned	Announcement expected 2027+

Preclinical | Phase 1 | Phase 2 | Phase 3

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

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Why the Microcystic LM Field is Moving Toward Early Intervention

THE BIOLOGY

Somatic PIK3CA mutations keep the growth pathway always "on"[1]

LM is proliferative and progressive, not a static lesion

Luks et al., J Pediatr 2015 (Boston Children's)[1]

Boston Children's[2]

85% **of childhood LMs progressed by adulthood**

CLINICAL STUDY

Lymphatic Malformation: Risk of Progression During Childhood and Adolescence

Aladdin H. Hassanein, MD, MMSc, John B. Mulliken, MD,* Steven J. Fishman, MD,† Nicola A. Quatrano, BS,* David Zurakowski, PhD,† and Arin K. Greene, MD, MMSc**

Stanford[3]

2.8x **higher odds of progression without treatment**

Journal of Clinical & Experimental Dermatology Research

Patel et al., J Clin Exp Dermatol Res 2017, 8:6
DOI: 10.4172/2155-9554.1000424

Research Article | Open Access

Natural History of Lymphatic Malformation Progression in Children: A Retrospective Study

Viraat Patel[1], Joanna H. Tu[2,3], and Joyce M. C. Teng[3*]

"Early intervention should be considered...because of their tendency to progress."[2]

1. Luks VL et al. J Pediatr. 2015;166:1048–54. 2. Hassanein AH et al. J Craniofac Surg. 2012;23(1):149–52. 3. Patel V et al. J Clin Exp Dermatol Res. 2017;8(6):424.

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Cutaneous Venous Malformations

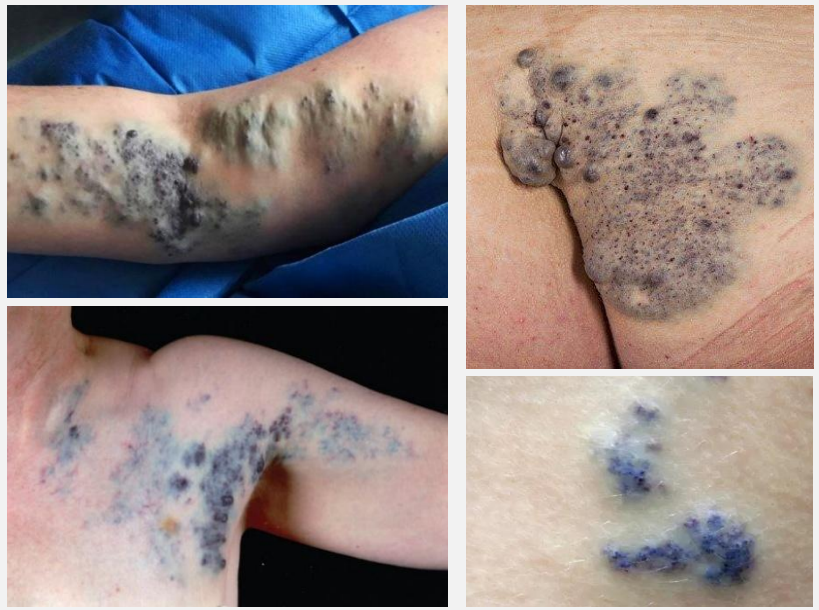
Near-term Phase 3 initiation on track

QTORIN™ RAPAMYCIN HAS POTENTIAL TO BE FIRST APPROVED THERAPY

- **Phase 3 initiation on track for Q4 2026**
- **Significant treater overlap with microcystic lymphatic malformations**
- **Positive Phase 2 TOIVA data with 73% of patients improved on cVM-IGA at Week 12**
  - 24-week data presented at PeDRA October 2026
    - Additional improvement between Week 12 and Week 24 on multiple endpoints
    - Data demonstrated reduction in disease severity and bleeding in pediatric patients after treatment

> 75k patients

ESTIMATED DIAGNOSED IN THE U.S.



No FDA-approved therapies

Fast Track Designation Granted

Pipeline-in-a-product:
sNDA planned

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Clinically Significant Angiokeratomas

Superficial lymphatic malformations

UNDER-RECOGNIZED MARKET OPPORTUNITY

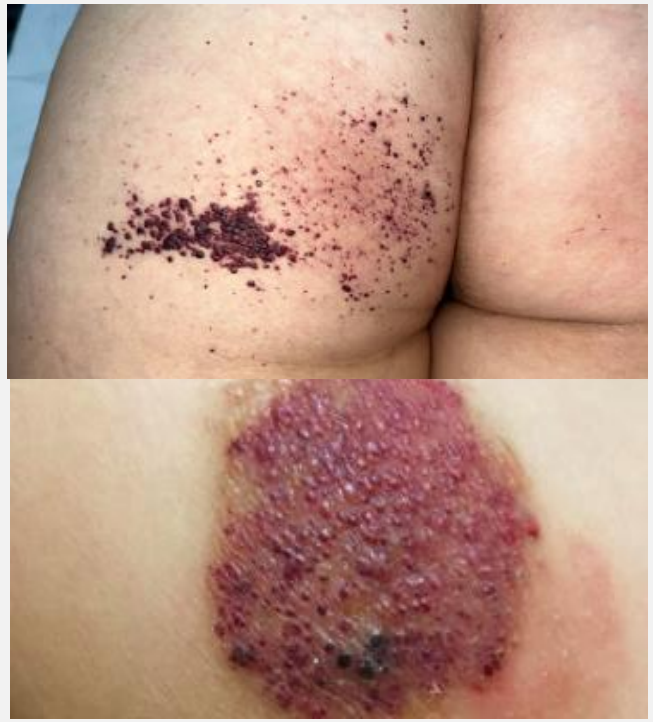
- **Topline results (n=~15) expected in 2H 2027**
  - Phase 2 LOTU study is a 12-week, single-arm, baseline-controlled clinical trial evaluating QTORIN™ rapamycin topically once daily
  - Strong enrollment momentum and site engagement
- **Highly symptomatic with bleeding and painful clots; sensitive location limits destructive interventional treatments**

Market research (n=50 physicians):

***96%** would incorporate QTORIN™ rapamycin into their practice*

> 50k patients

ESTIMATED DIAGNOSED IN THE U.S.



No FDA-approved therapies

Fast Track Designation Granted

Pipeline-in-a-product: sNDA planned

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Disseminated Superficial Actinic Porokeratosis

Chronic, pre-cancerous, and progressive

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

- **QTORIN™ pitavastatin: First pathogenesis-directed therapy**
- **Milestones:**
  - ☑ Yale collaboration: pharmacodynamic proof of principle with pitavastatin reversing hyperproliferation in a human porokeratosis cell model
  - ☑ Formulation development completed
  - ☑ Licensed Yale U.S. patent and filed IP
  - ☑ Multiple IND-enabling tox studies completed
  - ☐ **Q4 2026:** Phase 2 initiation anticipated

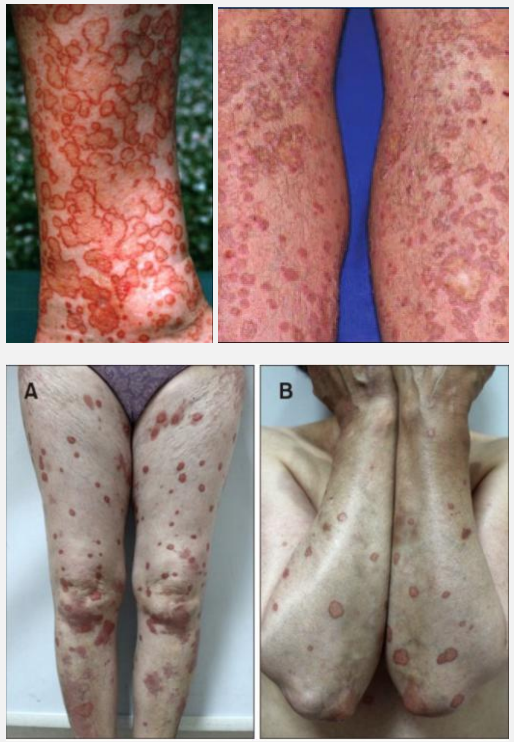
FALL CLINICAL DERMATOLOGY CONFERENCE®

New claims analysis October 2026

~155K

diagnosed U.S. DSAP patients

First real-world claims analysis-based characterization of the diagnosed U.S. DSAP population



No FDA-approved therapies

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

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Finance & Cash Update

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U.S. Launch and Rare Disease Pipeline are Well-Funded with $236.7 Million on Balance Sheet as of 9/30/26

Key strategic priorities accelerating:

- NDA accepted with Priority Review; PDUFA date of February 28, 2027
- Commercial and medical affairs expansion
- Major QTORIN™ pipeline and platform value drivers

$236.7 million

9/30/26 cash

Cash, cash equivalents and short-term investments

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Closing Remarks

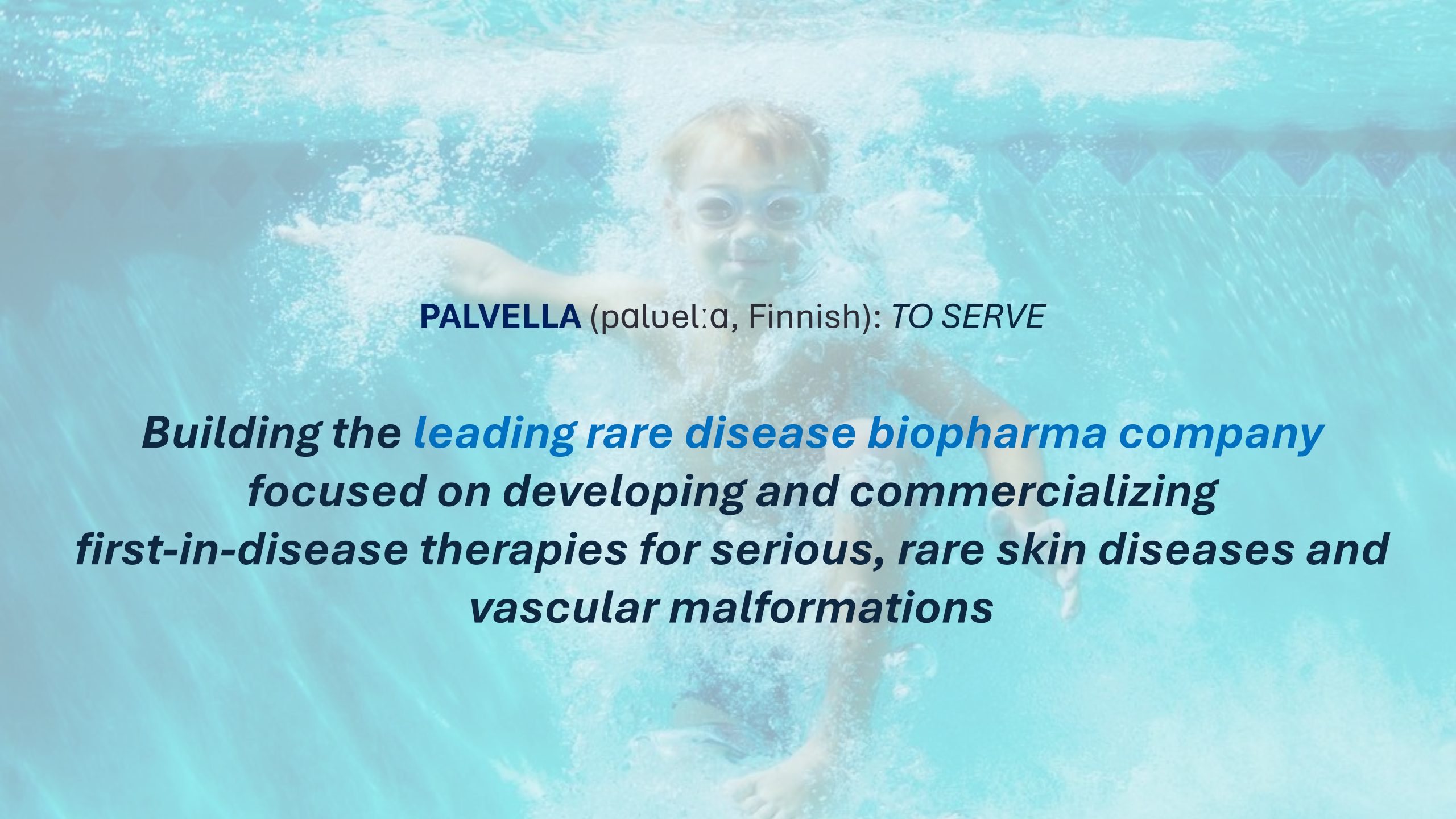
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Palvella: Driving the Next Wave of Innovation in Under-Appreciated Rare Skin Diseases and Vascular Malformations

	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™ Rapamycin / Pitavastatin as **First-line Therapy**[2]	> 80%			

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

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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 malformations

Thank You

Striving to be first for rare disease patients

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