



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

Q3 2025 Financial Results & Corporate Update
November 11, 2025



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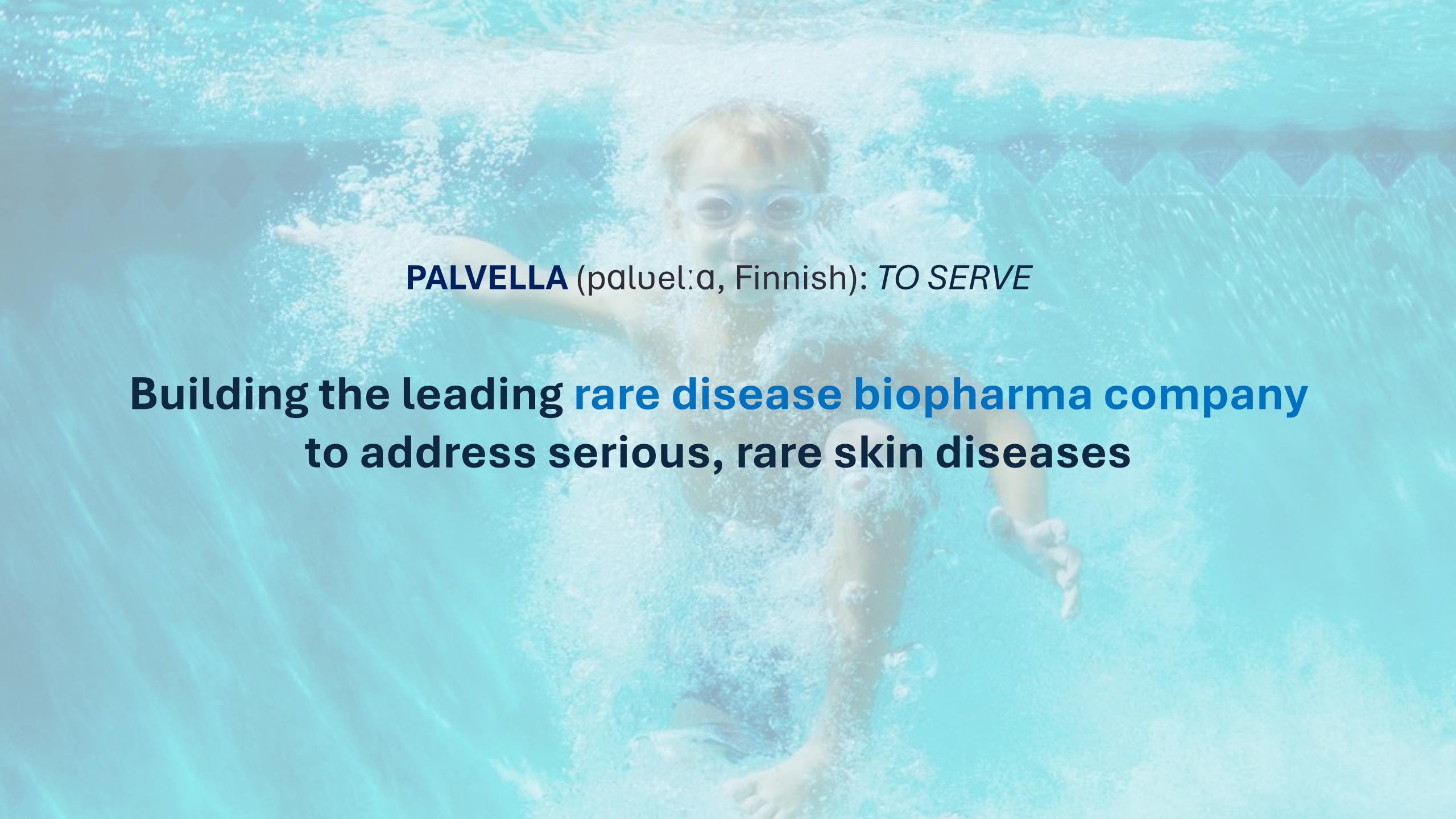
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A young child is swimming underwater in a pool. The water is a clear, light blue, and sunlight is filtering down from the surface, creating bright rays and bubbles. The child's face is visible above the water, looking towards the camera. The overall atmosphere is peaceful and suggests a sense of freedom and exploration.

PALVELLA (paluelä, Finnish): *TO SERVE*

**Building the leading rare disease biopharma company
to address serious, rare skin diseases**

Multiple High-Impact Milestones By End of Q1 2026



Advancing QTORIN™ Programs for Four Serious, Rare Skin Diseases and Beyond

Key 2025 Milestones Achieved

Continued Strong Momentum With Steady Cadence Of Anticipated Milestones Over Next 18 Months

QTORIN™ Rapamycin Microcystic Lymphatic Malformations	 Phase 3 SELVA trial exceeded enrollment target with 51 subjects	<input type="checkbox"/> Phase 3 SELVA trial top-line data (Q1 2026) <input type="checkbox"/> Planned medical / scientific presentations (ongoing) <input type="checkbox"/> NDA submission (2H 2026) <input type="checkbox"/> Potential FDA approval (1H 2027)
QTORIN™ Rapamycin Cutaneous Venous Malformations	 Phase 2 TOIVA trial fully enrolled with 16 subjects	<input type="checkbox"/> Phase 2 TOIVA trial top-line data (Mid-Dec '25) <input type="checkbox"/> Apply for Breakthrough Therapy Designation <input type="checkbox"/> Phase 3 trial initiation
QTORIN™ Rapamycin Clinically Significant Angiokeratomas	 Program announced Sept. 2025	<input type="checkbox"/> FDA interactions and potential for regulatory designations (1H 2026) <input type="checkbox"/> Phase 2 trial initiation (2H 2026)
QTORIN™ Pitavastatin Disseminated Superficial Actinic Porokeratosis	 Program announced Nov. 2025	<input type="checkbox"/> FDA interactions and potential for regulatory designations (1H 2026) <input type="checkbox"/> IND submission <input type="checkbox"/> Phase 2 trial initiation (2H 2026)
Additional Pipeline Programs	<i>Additional future QTORIN™ pipeline programs and indications</i>	



QTORIN™ 3.9% RAPAMYCIN

Clinical Programs

Phase 2 TOIVA Study in cVMs: Full Enrollment Announced Sept '25

Single-arm, baseline-controlled, QD dose, age 6+, 12 weeks, n=16

Enrollment closed in Sept '25 with n=16 patients



Megha Tollefson, MD

Principal Investigator



No Statistical Hierarchy of Endpoints

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



toiva
Top-line data
anticipated
Mid-December 2025

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QTORIN™ Rapamycin for Cutaneous Venous Malformations: Phase 2 TOIVA Study Objectives



No FDA-approved therapies: Current options include laser, sclerotherapy, off-label systemic pharmacotherapies limited by toxicities

Progressive disease; no spontaneous regression

- **Safety**
 - Evaluating safety and tolerability compared to interventional and destructive approaches
- **Efficacy**
 - Proof-of-concept study to detect one or more endpoints that could serve as the primary endpoint for Phase 3 study
 - Dynamic change scales and static scales
 - Evidence of clinical improvements or slowing disease progression
 - Improvement in one or more endpoints in ~30% of patients
 - Evidence of any time-dependent pharmacologic effect

QTORIN™ Rapamycin for Microcystic Lymphatic Malformations

Q1 2026

Phase 3 Top-line Data in
Microcystic LMs

EXCEEDED ENROLLMENT TARGET

✓ 51 subjects



- **Potential to be first FDA-approved therapy**
- **Phase 3 SELVA trial top-line data on track for Q1 2026**
- **Received year two of FDA Orphan Drug Grant**
 - Proceeds granted following FDA review of annual performance progress report on Phase 3 SELVA trial
- **On track for planned 2H 2026 NDA submission**
 - Strengthened regulatory affairs leadership team
- **Estimated >30,000 diagnosed U.S. patients support multi-billion dollar TAM**
- **Building commercial and medical affairs teams in anticipation of standalone U.S. commercialization in 2027**

Breakthrough
Therapy
Designation

Fast
Track
Designation

Orphan
Drug
Designation

Growing Commercial Opportunity for QTORIN™ Rapamycin: Pool of Addressable Patients in the U.S. Projected to Expand by >10x with Pipeline-in-a-Product Strategy

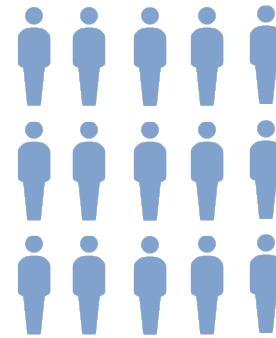
1 person = 5k

30k+



Microcystic LMs

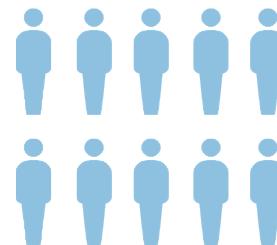
75k+



Cutaneous VMs

Announced
September 2025

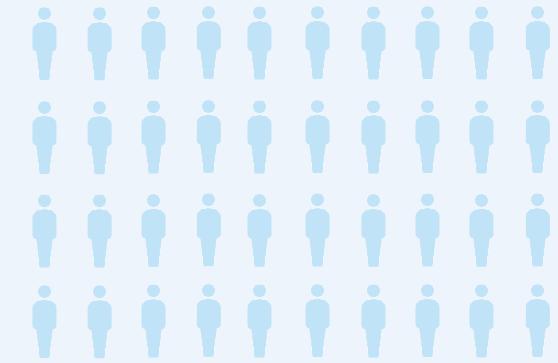
50k+



Clinically Significant
Angiokeratomas

Targeting announcement
in 2026 and beyond

200k+



Additional Potential Future
Indications¹

TODAY

FUTURE

Estimated timeline for potential regulatory approval

2027

2029

2031+

2032+



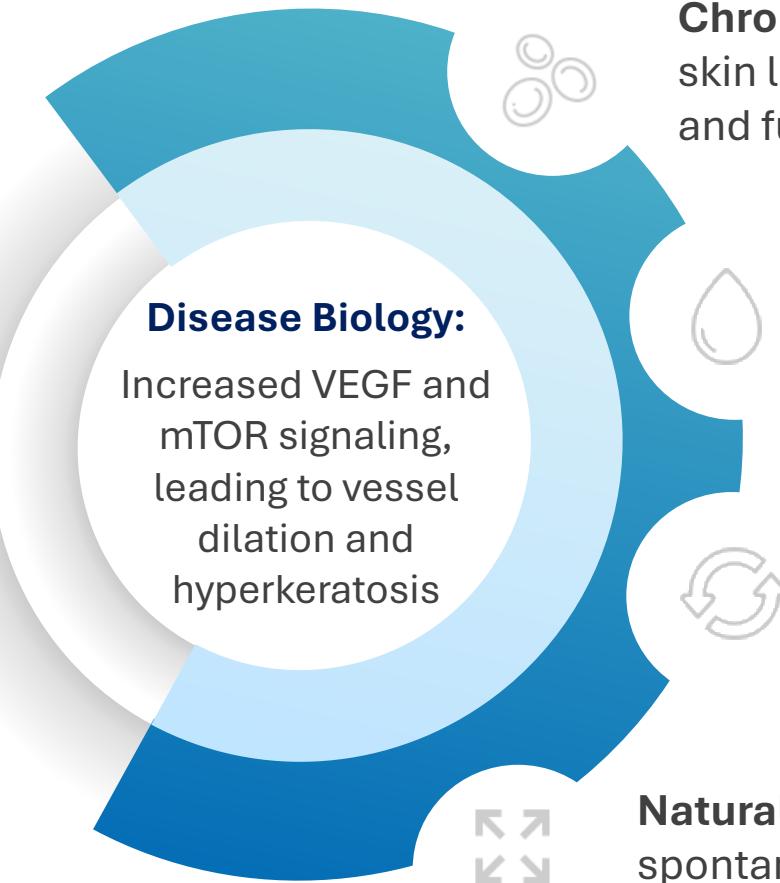
QTORIN™

Pipeline Programs

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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



No FDA-approved therapies

Current options:
laser therapy, electrosurgery, cryotherapy, and surgical excision

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.

QTORIN™ Rapamycin as a “Pipeline-in-a-Product”: Advancing Program to Angiokeratoma Patients

Leveraging established aspects of QTORIN™ rapamycin program

- QTORIN™ 3.9% rapamycin formulation
- Drug supply ready to deploy to clinic
- Open IND with FDA Division of Dermatology and Dentistry
- Existing intellectual property coverage

FDA meeting planned 1H 2026

- Discuss proposed Phase 2 study design
- Longer-term, supplemental NDA (sNDA) submission planned (if approval achieved) in microcystic LMs and/or cutaneous VMs
- Discuss eligibility for expedited programs (Fast Track Designation)

Planned Phase 2 study initiating in 2H 2026

- Single arm, baseline-controlled study with $n \sim 10-20$ patients
- Microcystic LM efficacy endpoints potentially applicable based on clinical overlap

GOAL: Initiate Phase 2 clinical development in 2H 2026

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

> 50k patients

ESTIMATED DIAGNOSED IN THE U.S.¹



Genetics & Disease Biology:

Autosomal dominant (primary) or de novo germline mutation leads to accumulation of toxic intermediates

Risk of malignant transformation:

Premalignant disease with transformation to non-melanoma skin cancers²

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 rare²

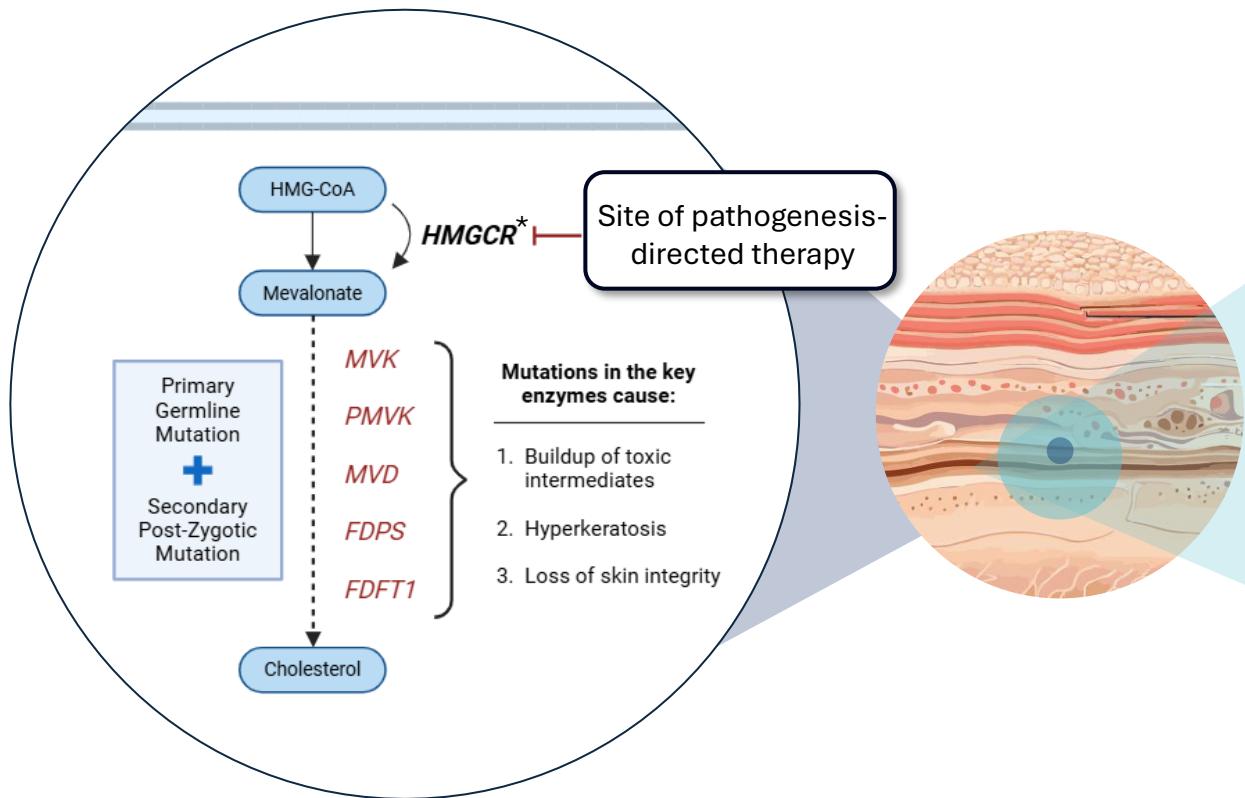
No FDA-approved therapies

Current options:

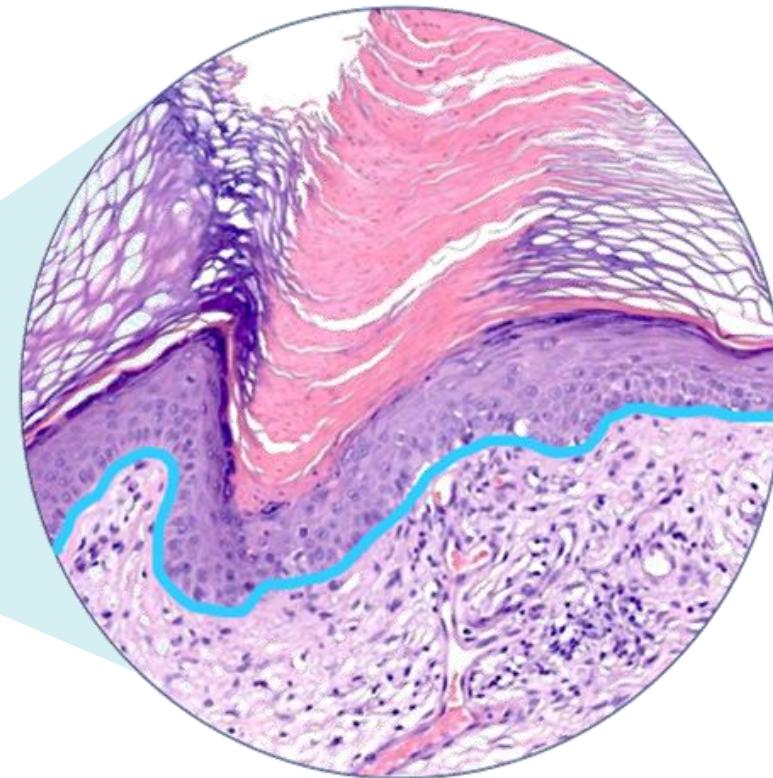
Laser, surgery, and off-label topical chemo agents & mevalonate pathway inhibitors

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

QTORIN™ Pitavastatin Clinical Pathway:

Planned Initiation of Phase 2 in 2H 2026

QTORIN™ Pitavastatin: From Concept to Clinic

- QTORIN™ pitavastatin optimized for stability and drug delivery
- Working with FDA Division of Dermatology and Dentistry
- Filed intellectual property

FDA meeting planned 1H 2026

- Discuss proposed Phase 2 study design
- Discuss eligibility for expedited programs (Fast Track Designation)

Initiation of Proposed Phase 2 study anticipated in 2H 2026

- Phase 2 protocol drafted
- Endpoint development nearing completion with extensive input from key opinion leaders and patients

GOAL: Initiate Phase 2 clinical development in 2H 2026



Finance

Q3 2025 Financial Highlights and 2025 Outlook

\$63.6 million

Cash at 9/30/2025

Runway into
2H 2027

\$10.2 million

R&D + G&A spend in Q3 2025

~\$55 million

Projected cash at year end



Striving to be first for rare disease patients

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THERAPEUTICS

Q&A