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

Q2 2025 Financial Results & Corporate Update
August 14, 2025

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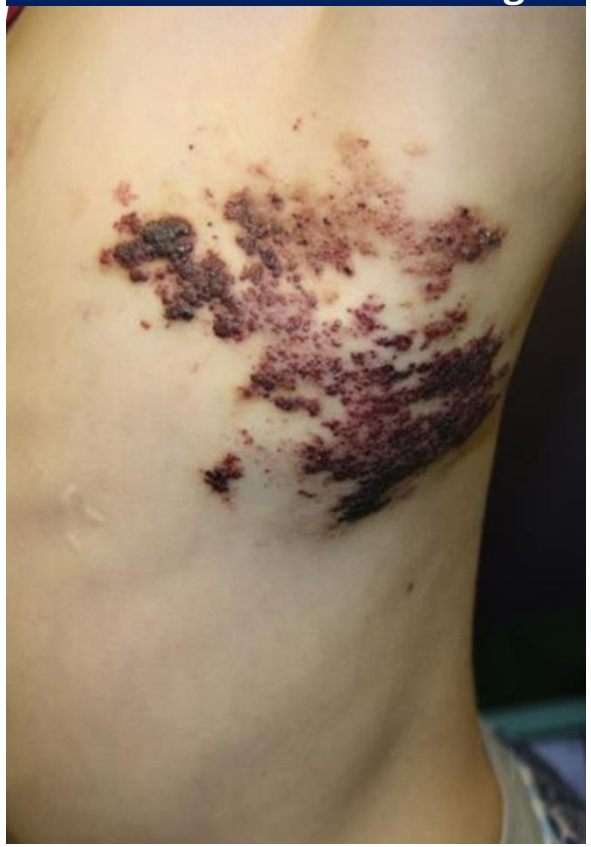
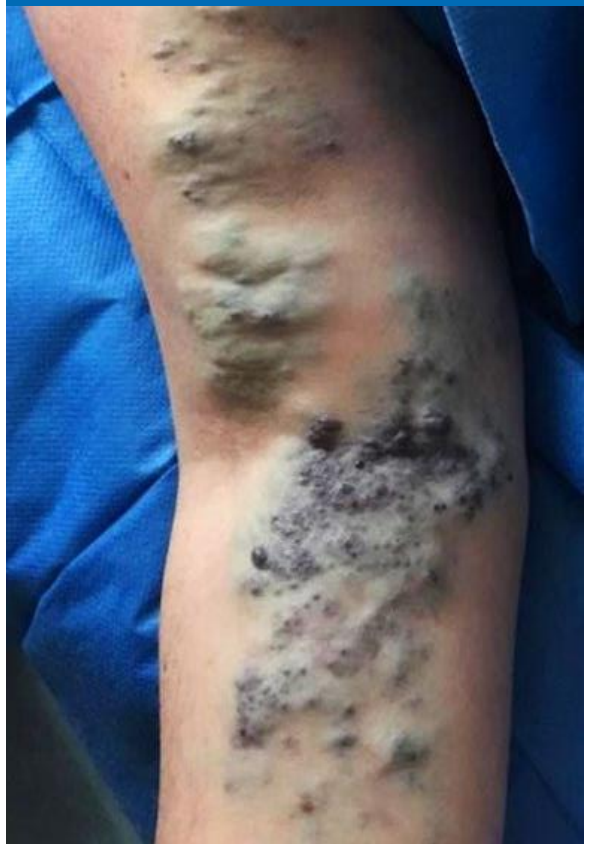
Multiple High-Impact Near-Term Milestones by Q1 2026

1 **Phase 3 data in microcystic LMs** (Q1:26) – ***exceeded enrollment target***



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



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

4 **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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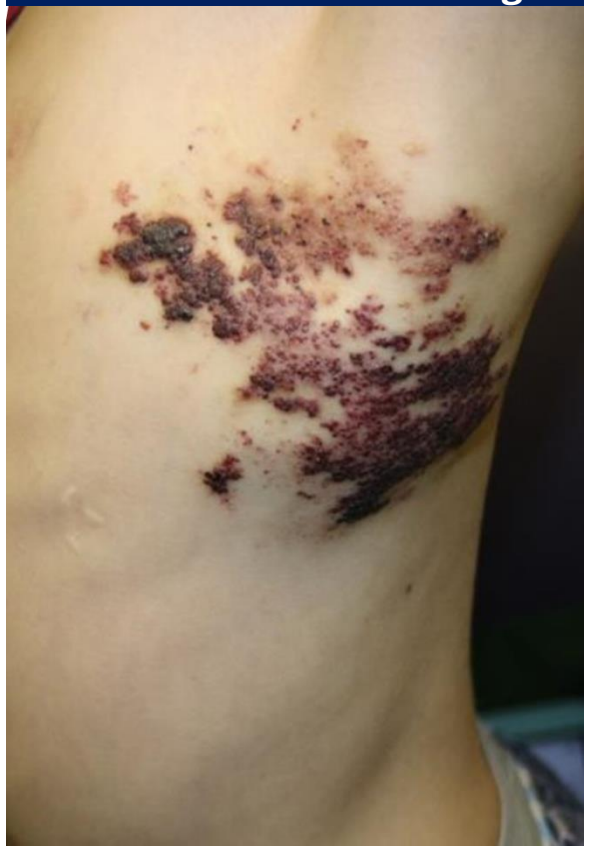
QTORIN™ Rapamycin: Potential To Be First FDA-Approved Therapy for Serious, Rare, Lifelong Disease

Phase 3 SELVA trial **over-enrolled** and on track for Q1 2026 readout

1

Phase 3 data in microcystic LMs (Q1:26) – ***exceeded enrollment target***



Amy Paller, MD

Northwestern Medicine®
Feinberg School of Medicine

Ashley Kline

Chief Commercial Officer

- **Key presentations and posters at major scientific meetings**
  - SID poster supports > 30,000 estimated diagnosed prevalence and > 1,500 annual incidence of microcystic lymphatic malformations in the U.S.
- **NDA planning accelerating**
- **Chief Commercial Officer hired; U.S. launch planning underway**

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QTORIN™ Rapamycin: Potential To Be First FDA-Approved Therapy for Serious, Rare, Chronically Debilitating Disease

Phase 2 TOIVA trial on track for Q4 2025 readout

Megha Tollefson, MD
Principal Investigator
MAYO CLINIC

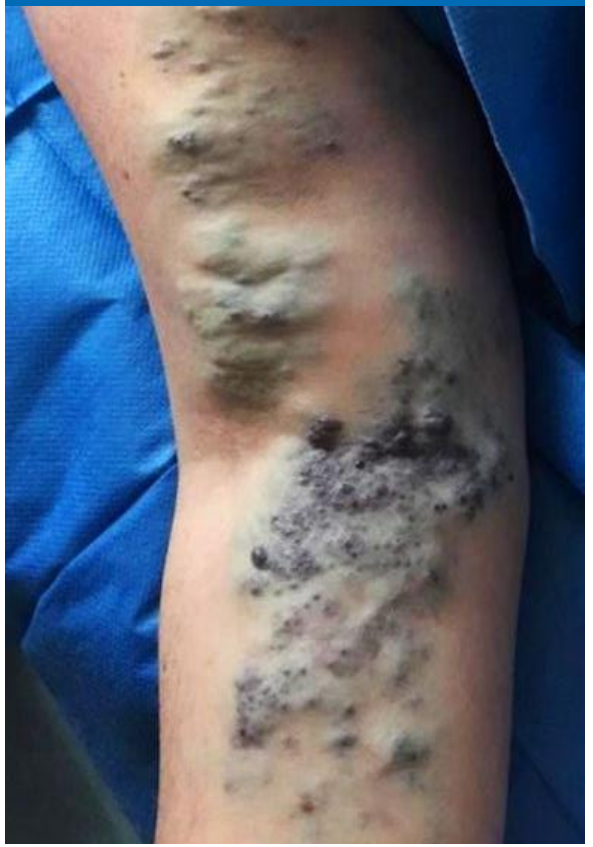
- **Phase 2 TOIVA trial enrollment ongoing**
  - PoC study to enroll ~15 patients
  - No statistical hierarchy of endpoints
- **Estimated U.S. diagnosed prevalence: > 75,000**
  - Epi study completed to determine cutaneous-only and mixed population
- **FDA Fast Track Designation granted (2024)**

Significant unmet need for targeted, localized therapy for cutaneous venous malformations

Large growing base of real-world evidence supporting off-label systemic rapamycin as targeted therapy for internal venous malformations

2

Phase 2 data in cutaneous VMs (Q4:25)



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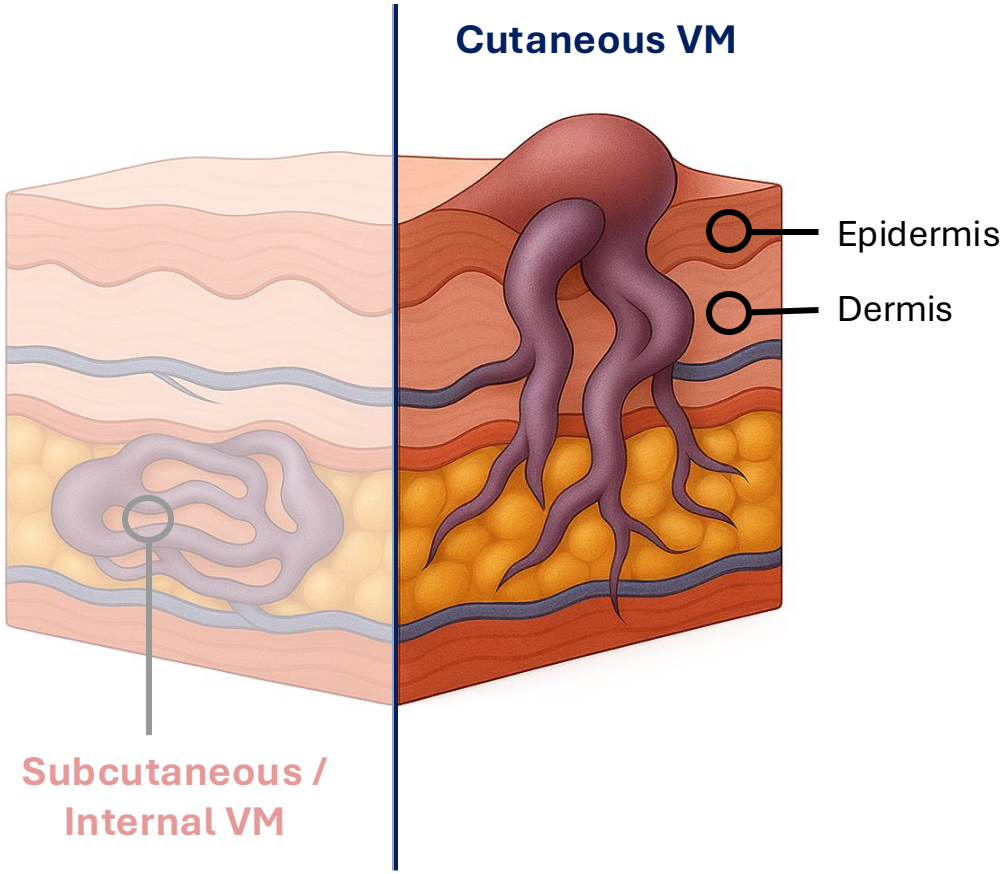
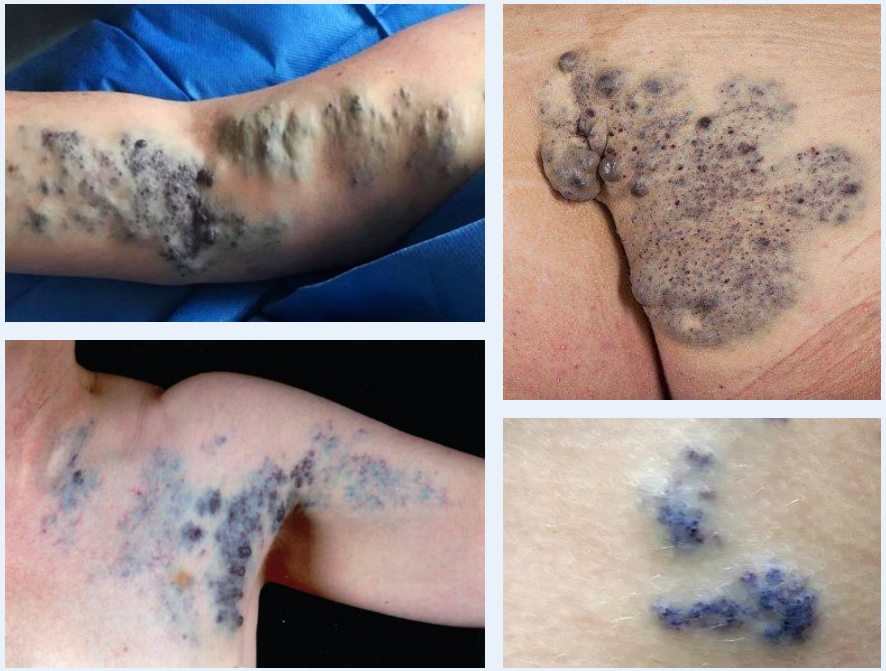
QTORIN™ Rapamycin Targets Cutaneous Venous Malformations:
On Target, In Tissue





Bleeding, thrombosis, ulceration, disfigurement, and proliferation can significantly impact patient QoL

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QTORIN™ Pipeline Development

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QTORIN™ Rapamycin: "Pipeline-in-a-Product" to Address Broad Range of mTOR-Driven Skin Diseases

2015

Advances in the therapeutic use of mammalian target of rapamycin (mTOR) inhibitors in dermatology

Alexander L. Fogel, BS,[d] Sharleen Hill, MD,[e] and Joyce M. C. Teng, MD, PhD[a,b,c,d]
Palo Alto, California, and London, United Kingdom

Australasian Journal of Dermatology

2021

Australasian Journal of Dermatology (2021) ••, ••–••

doi: 10.1111/ajd.13671

REVIEW ARTICLE

Systematic review of sirolimus in dermatological conditions

Andrew W Swarbrick[1] | Aaron J Frederiks[2,3] | Rachael S Foster[1,4]

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Review Article

2025

Off-Label Topical Application of Sirolimus (Rapamycin) for Dermatological Conditions

Tatiana Lapa, MD, MSc[1] and Matt Sandre, MD, FRCPC[2]

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Canadian Dermatology Association / Association canadienne de dermatologie

>25 mTOR-driven skin diseases

Vascular anomalies, genodermatoses, non-vascular neoplasms, and others

Need for safe and effective FDA-approved therapy for these indications

> ***Larger, controlled studies are needed to define optimal concentrations, formulations, and dosing schedules.***

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Key Value Drivers from Pipeline Programs in Second Half of 2025

3 **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

4 **New QTORIN™ Program** (2H:25)

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Financial Update

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Q2 2025 Financial Highlights and 2025 Outlook

Strong Cash Position

~2 years

Runway into 2H 2027

$70.4 million

Cash at 6/30/2025

$9.3 million

R&D + G&A expenses in Q2 2025

~$55 million

Projected cash at year end

June 2025 Update

Palvella added to

Russell 2000®

&

Russell 3000®

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Q&A

Striving to be first for rare disease patients

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