QTORIN™ Rapamycin 3.9% Anhydrous Gel: A Novel, Dermal-Targeted Topical mTOR Inhibitor in Phase 3 For Treatment of Microcystic Lymphatic Malformations

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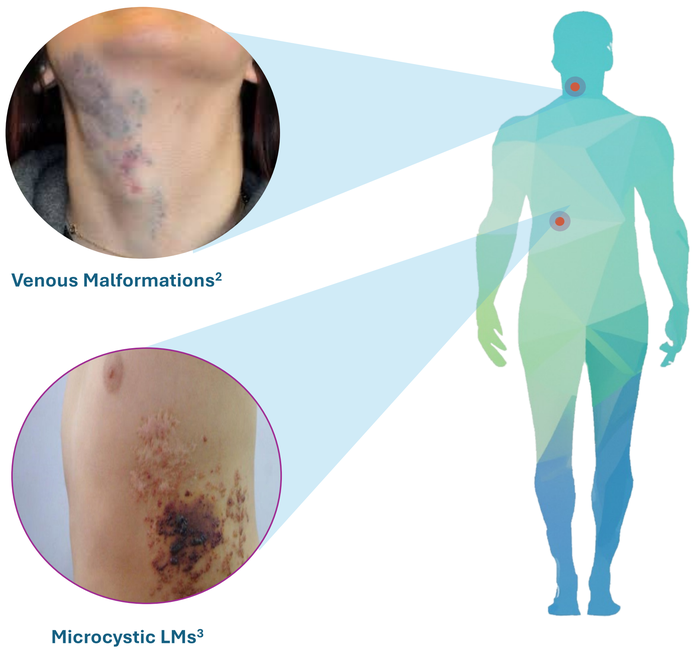
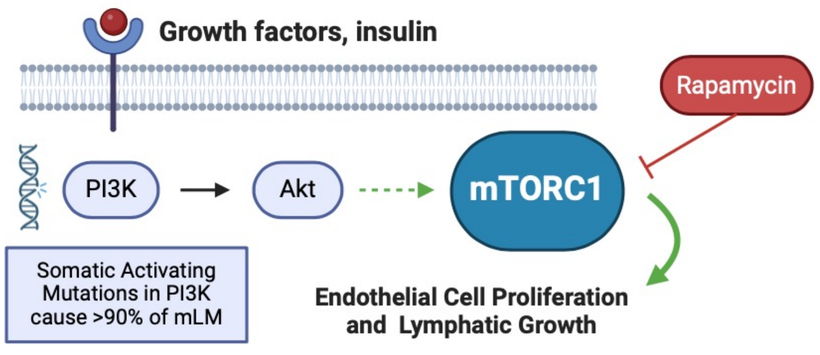
1. Rapamycin: a molecule with broad potential for treating many high unmet need dermatoses, but with significant barriers for use.





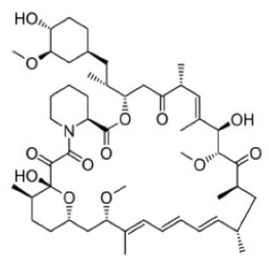
Systemic Limitations

- Strong Immunosuppressive Activity[1]
- Systemic Toxicities
- Poor Biodistribution to the Skin[2]

Cutaneous Delivery Challenges

- Limited Penetration due to High Molecular Weight
- Poor Solubility
- Poor Chemical Stability

2. QTORIN™: a versatile platform designed to deliver novel therapies to the dermis.

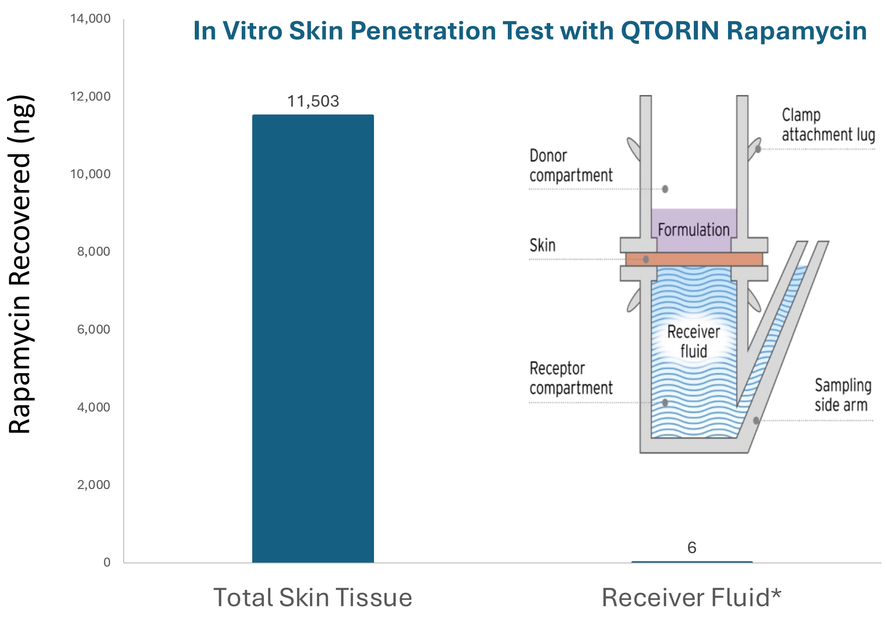


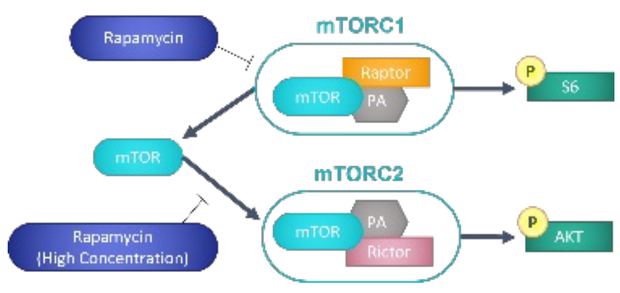
- > 20 Excipients, >80 Combinations
- Optimized Solubility and Systemic Absorption
- Final Formulation Selected

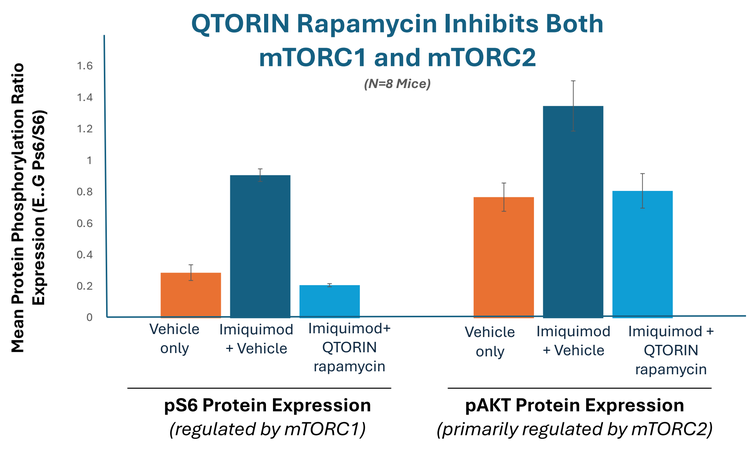
QTORIN Rapamycin 3.9% Anhydrous Gel

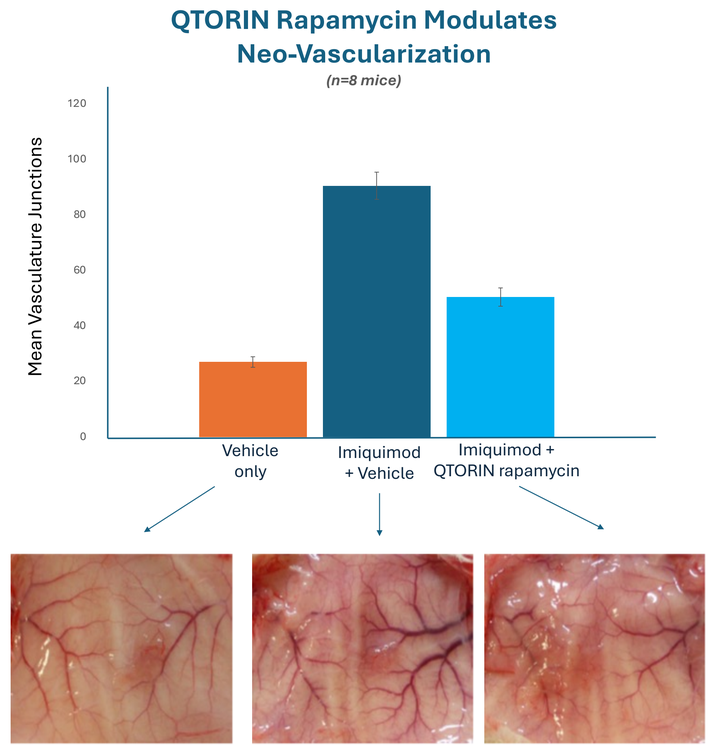
- Direct mechanistic engagement of causal mTOR pathway.
- Local delivery of 1000-fold more rapamycin than oral administration.
- Limited-to-undetectable systemic absorption seen.
- Enhanced chemical stability of rapamycin at room temperature for 36 months.

3. QTORIN™ rapamycin: high concentration and targeted inhibition of mTOR in the dermis.

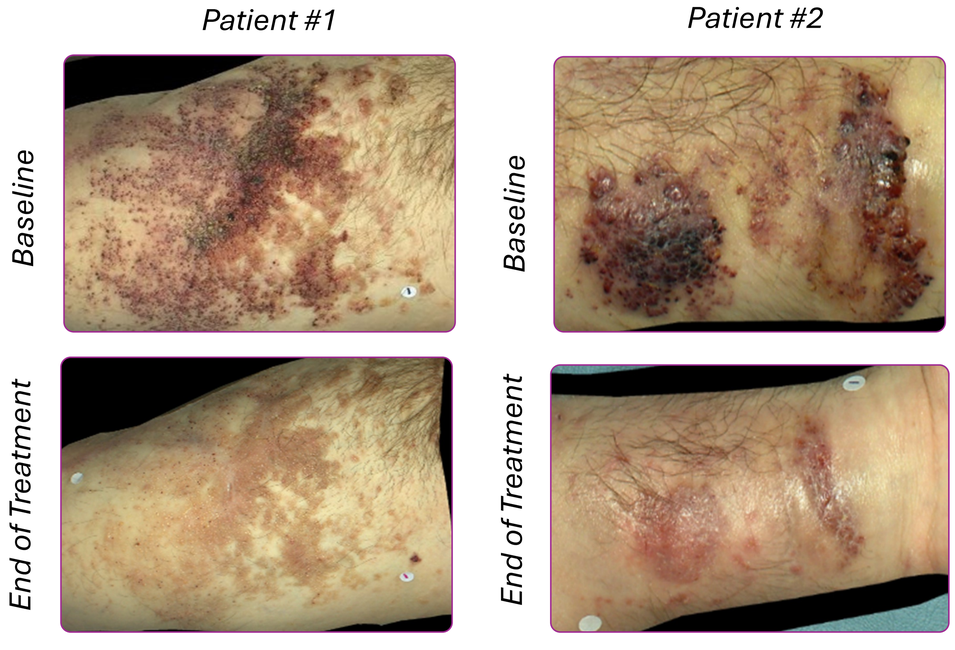


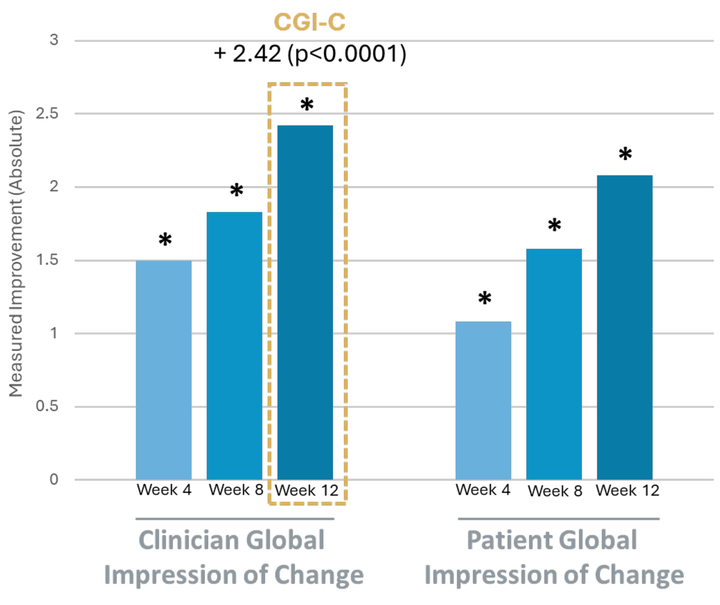






4. Phase 2 clinical trial shows efficacy of QTORIN™ rapamycin for microcystic lymphatic malformation.





5. Status of the QTORIN™ platform.

- Phase 3 trial in microcystic LM ongoing.
- Phase 2 trial in cutaneous VM ongoing.
- FDA designations & awards:
  - Fast Track, Orphan Drug Designation, Breakthrough Designation, Orphan Products Development Grant*.
- Ongoing evaluation of mTOR driven indications.
- Ongoing evaluation of QTORIN enabled products.

* Palvella Therapeutics Awarded Up to $2.6 million Grant from the FDA Office of Orphan Products Development to Support Phase 3 Single-Arm, Baseline-Controlled Trial in Microcystic LM

1. Rapamune package insert. 2)Kitayama et al., Journal of Derm. Science (2019)

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