Lipella Reports Positive Phase 2a Results from Second LP-310 Cohort in Oral Lichen Planus; Final Data Expected Q2 2025

April 22, 2025 05:30 ET

Second Cohort Results Highlight Efficacy Across All Key Measures and Reinforce Safety of Twice-Daily Oral Rinse

Phase 2a Study Now Fully Enrolled Across All Three Dose Cohorts

Advancing Toward Phase 2b IND Submission and Broader Regulatory Engagement

PITTSBURGH, April 22, 2025 (GLOBE NEWSWIRE) -- Lipella Pharmaceuticals Inc. (Nasdaq: LIPO) ("Lipella," "our," "us," or the "Company"), a clinical-stage biotechnology company addressing serious diseases with significant unmet needs, today announced positive topline results from the second cohort (0.50 mg) of its Phase 2a multicenter, dose-ranging trial evaluating LP-310, a liposomal-tacrolimus oral rinse formulation of LP-10, for the treatment of oral lichen planus (OLP). The Company also recently reported full enrollment across all three planned dose cohorts.

Treatment with LP-310 at the 0.50 mg dose demonstrated statistically significant improvements in multiple patient-reported and investigator-measured efficacy endpoints, reinforcing LP-310's potential as a non-steroidal, locally delivered therapy for OLP. The trial, currently active across seven U.S. study sites, has now progressed to the third and highest dose cohort (1.0 mg/10 mL), with topline results expected in the second quarter of 2025.

"These results mark meaningful progress for LP-310 and further strengthen our confidence in its clinical and commercial potential," said Jonathan Kaufman, Co-Founder and Chief Executive Officer of Lipella Pharmaceuticals. "With the Phase 2a trial now fully enrolled and final data expected this quarter, we are advancing toward key value-driving milestones, including a Phase 2b IND submission and broader regulatory engagement to support the program's continued development."

Topline Findings from the 0.50 mg Cohort:

- Investigator Global Assessment (IGA): Improved from 3.42 ± 0.21 at baseline to 2.71 ± 0.30 at week 1 (p=0.029), 1.71 ± 0.43 at week 4 (p=0.007) and 2.75 ± 0.34 at week 6 (p=0.112).
- Reticulation, Erythema and Ulceration (REU) Score: Reduced from 26.91 ± 2.54 at baseline to 17.02 ± 2.36 at week 1 (p=0.003), 11.88 ± 2.91 at week 4 (p=0.003) and 18.47 ± 4.12 at week 6 (p=0.028).
- Oral Lichen Planus Symptom Severity Measure (OLPSSM): Decreased from 14.92 ± 2.10 at baseline to 9.87 ± 2.27 at week 1 (p=0.032), 4.88 ± 2.15 at week 4 (p=0.003) and 8.42 ± 3.98 at week 6 (p=0.028).
- Pain Numerical Rating Scale (NRS): Improved from 6.42 ± 0.75 at baseline to 4.25 ± 0.89 at week 1 (p=0.003), 2.25 ± 1.09 at week 4 (p=0.003) and 3.52 ± 1.51 at week 6 (p=0.028).

- Global Response Assessment (GRA): Significant improvement was observed at week 4 (p=0.028).

Safety and Tolerability

LP-310 continues to be well tolerated, with no treatment-related SAEs and no patient dropouts. All participants successfully adhered to the twice-daily 10-milliliter rinse regimen. Pharmacokinetic analysis confirmed that tacrolimus levels remained undetectable or minimal in all patients, reinforcing LP-310's ability to deliver localized benefits without systemic toxicity.

"We're very encouraged by the positive data from the 0.50 mg cohort, which demonstrated statistically significant efficacy across multiple clinical measures while maintaining a strong safety profile," said Dr. Michael Chancellor, Co-Founder and Chief Medical Officer of Lipella Pharmaceuticals. "Oral lichen planus is a painful, chronic condition with no FDA-approved treatment and limited therapeutic options. The symptom relief reported by patients, along with reductions in inflammation and ulceration, highlights LP-310's potential to be a transformative, steroid-free, and easy-to-use therapy that addresses a longstanding clinical unmet need and improves quality of life."

Next Steps in Development

With enrollment now complete across all three dose cohorts, Lipella is positioned to deliver final topline data from the 1.0 mg cohort in Q2 2025. These data are expected to form the foundation for upcoming regulatory interactions and next-phase clinical advancement. Key development milestones include:

- Reporting final phase 2a topline results in Q2 2025
- Advancing discussions with regulatory agencies including submission of an Investigational New Drug (IND) application for a Phase 2b trial in late 2025
- Preparing to pursue FDA Breakthrough Therapy designation request

About LP-310

LP-310 is an innovative oral rinse formulation of LP-10 (tacrolimus), developed to address OLP. Designed to provide localized therapeutic effects while minimizing systemic exposure, LP-310 offers a promising new approach to managing this painful and often debilitating condition.

A Phase 2a multicenter, dose-ranging clinical trial is currently underway to evaluate the safety, tolerability and efficacy of LP-310 in adult participants with symptomatic OLP. The trial includes three dose levels (0.25 mg, 0.5 mg and 1.0 mg of tacrolimus) and is being conducted across seven active U.S. sites, which are now recruiting participants. Lipella has reported topline data from the first two cohorts (0.25 mg and 0.50 mg), with the final cohort (1.0 mg) currently enrolling. Topline results from the 1.0 mg cohort are expected in the first half of 2025.

For more information about the study or to participate,
visit https://clinicaltrials.gov/study/NCT06233591.

About Lipella Pharmaceuticals Inc.

Lipella Pharmaceuticals is a clinical-stage biotechnology company focused on developing new drugs by reformulating active agents in existing generic drugs and optimizing these reformulations for new applications. Lipella targets diseases with significant unmet needs, where no approved drug therapies currently exist. The company completed its initial public offering in 2022.

Forward-Looking Statements

This press release includes certain "forward-looking statements" which are not historical facts, within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. All statements, other than statements of historical fact, included in this press release regarding, among other things, our strategy, future operations, financial position, prospects, clinical trials, regulatory approvals, pipeline and opportunities, sources of growth, successful implementation of our proprietary technology, plans and objectives are forward-looking statements. Forward-looking statements can be identified by words such as "may," "will," "could," "continue," "would," "should," "potential," "target," "goal," "anticipates," "intends," "plans," "seeks," "believes," "estimates," "predicts," "expects," "projects" and similar references to future periods. Forward-looking statements are based on our current expectations and assumptions regarding future events and financial trends that we believe may affect among other things, market and other conditions, our financial condition, results of operations, business strategy, short- and long-term business operations and objectives, and financial needs. Because forward-looking statements relate to the future, they are subject to inherent uncertainties, risks and changes in circumstances that are difficult to predict. Our actual results may differ materially from those contemplated by the forward-looking statements. We caution you, therefore, against relying on any of these forward-looking statements. They are neither statements of historical fact nor guarantees or assurances of future performance. There are risks, uncertainties and other factors, both known and unknown, that could cause actual results to differ materially from those in the forward-looking statements which include, but are not limited to, the current clinical trial results for LP-310 and our other products general capital market risks, our ability to regain and maintain compliance with the listing standards of The Nasdaq Stock Market LLC, regional, national or global political, economic, business, competitive, market and regulatory conditions, our current liquidity position and the need to obtain additional financing to support ongoing operations, and other risks as more fully described in our filings with the U.S. Securities and Exchange Commission. Any forward-looking statement made by us is based upon the reasonable judgment of our management at the time such statement is made and speaks only as of the date on which it is made. Factors or events that could cause our actual results to differ may emerge from time to time, and it is not possible for us to predict all of them. We undertake no obligation to update any forward-looking statement, whether as a result of new information, future developments or otherwise, except as may be required by applicable law. Nothing contained herein is, or shall be relied upon as, a promise or representation as to the past or future. In addition, the information contained in this press release is as of the date hereof, and the Company has

no obligation to update such information, including in the event that such information becomes inaccurate. You should not construe the contents of this press release as legal, tax or investment advice and should consult with your own advisors as to the matters described herein, as applicable.