Sep 18, 2025 08:00am

Lipella Pharmaceuticals Announces Positive Final Results from Phase 2a Study of LP-10 in Oral Lichen Planus

- *All 27 patients completed treatment with no serious adverse events, underscoring a favorable safety profile*
- *Statistically significant improvements achieved across all efficacy endpoints at the 4-week timepoint*
- *Preparing to initiate a pivotal Phase 2b study to advance LP-10 toward registration*

PITTSBURGH, Sept. 18, 2025 (GLOBE NEWSWIRE) -- Lipella Pharmaceuticals Inc. (Nasdaq : LIPO) ("Lipella," "we," "our," or the "Company"), a clinical-stage biotechnology company transforming care with innovative mucosal delivery solutions, today announced positive final results from its completed Phase 2a multicenter, dose-ranging study evaluating LP-10, a proprietary liposomal tacrolimus oral rinse, in patients with symptomatic oral lichen planus (OLP).

The study met its primary safety endpoint and achieved statistically significant improvements across all efficacy measures at the 4-week timepoint. These results support LP-10‘s potential to become the first FDA-approved therapy for OLP, a chronic inflammatory condition that affects an estimated 6 million Americans, with no approved treatment options.

"We believe these compelling results validate LP-10's potential to address a large, underserved patient population,” said Jonathan Kaufman, CEO and co-founder of Lipella. "The combination of excellent safety, minimal systemic absorption, and robust efficacy across multiple validated endpoints positions LP-10 as a first-in-class therapy. Based on these data, we are preparing to advance LP-10 into a pivotal Phase 2b study."

Key Study Results

Safety Profile

- All 27 patients completed the full 4-week treatment course
- No serious adverse events reported
- Minimal systemic exposure: 76% of tacrolimus blood measurements were below detection limits (<1.0 ng/mL)
- Well-tolerated, with only mild to moderate treatment-related adverse events; dry mouth being the most common, occurring in 18.5% of patients

Efficacy Outcomes

All three dose groups (0.25 mg, 0.5 mg, and 1.0 mg) demonstrated statistically significant improvements at Week 4 (all $p<0.05$) on the secondary efficacy endpoints:

Investigator Global Assessment (IGA) showed clear reductions in ulceration and erythema scores

- Pain and Sensitivity improved significantly, with patients reporting meaningful reductions on numerical rating scales
- Patient symptoms, as measured by the OLP Symptom Severity Measure (OLPSSM), demonstrated meaningful improvement in overall symptom burden
- Sustained efficacy was observed, with all patients maintaining clinical benefit and no evidence of worsening through the 2-week follow-up period

"Investigator examinations revealed significant healing, including reduction in inflammation and visible resolution of ulcerative lesions," said Dr. Michael Chancellor, Chief Medical Officer and Co-Founder of Lipella. "These observations were consistently supported by both investigator and patient-reported outcomes, demonstrating LP-10's meaningful impact on this debilitating condition. Importantly, the therapeutic benefits were generally maintained through the 2-week follow-up period."

Study Design

The Phase 2a study was a multicenter, dose-ranging trial conducted at five leading U.S. clinical sites. Twenty-seven adults with biopsy-confirmed symptomatic OLP were sequentially enrolled into three dose cohorts. Patients used a 3-minute LP-10 oral rinse twice daily for 4 weeks, followed by a 2-week safety follow-up.

The study population was representative of typical OLP patients: 81.5% were female, the median age was 62 years, and disease duration ranged from 1 to 28 years. All participants had previously failed standard therapies, including topical corticosteroids.

Next Steps

Based on these positive results, Lipella is advancing LP-10 into late-stage development:

- Preparation of Phase 2b protocol incorporating FDA feedback
- Scaling manufacturing capabilities to support larger clinical trials
- Exploring strategic partnerships and collaborations

"We believe LP-10 represents a paradigm shift in OLP treatment, if approved" added Kaufman. "By delivering tacrolimus through our proprietary liposomal formulation as an oral rinse, we've overcome the delivery challenges that have limited topical treatments while maintaining an exceptional safety profile. These results strengthen our conviction that LP-10 has the potential to transform the treatment landscape for millions of patients living with this chronic condition."

Full study results will be submitted for publication in a peer-reviewed journal and presented at upcoming medical conferences.

About Oral Lichen Planus

Oral lichen planus is a chronic inflammatory condition affecting the oral mucosa, characterized by white reticular lesions, erosions, and ulcerations that cause significant pain and functional impairment. Classified by the WHO as an oral potentially malignant disorder, OLP carries a malignant transformation risk of approximately 1.4%. Despite

affecting an estimated 6 million Americans, there are currently no FDA-approved therapies, leaving patients to rely on off-label treatments with limited efficacy and concerning side effect profiles.

About LP-10
LP-10 is a proprietary liposomal formulation of tacrolimus designed as an oral rinse for the treatment of oral lichen planus. The liposomal delivery system enables effective local administration while minimizing systemic absorption, addressing the key limitations of current topical treatments. LP-10 has previously demonstrated safety and preliminary efficacy in a Phase 2a study for hemorrhagic cystitis.

About Lipella Pharmaceuticals Inc.
Lipella Pharmaceuticals is a clinical-stage biotechnology company focused on reformulating and optimizing existing active pharmaceutical ingredients for new therapeutic applications in diseases with high unmet needs. The company completed its initial public offering in 2022. Learn more at lipella.com and follow us on X and LinkedIn.

Forward-Looking Statements
This press release includes certain "forward-looking statements." 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. Important factors that could cause actual results to differ materially from those in the forward-looking statements include, but are not limited to, risks related to clinical trial outcomes, regulatory approval processes, market acceptance, competitive products, intellectual property rights, availability of funding, and other factors described in our SEC filings. 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. 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.