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The safety, tolerability and efficacy of LP-10 in subjects with refractory moderate to severe hemorrhagic cystitis: Phase 2a multicenter dose escalation clinical trial.

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Background: Hemorrhagic Cystitis (HC), a rare but highly morbid disease for which there are currently no FDA approved treatments, can occur in cancer survivors including patients with prostate cancer, cervical/uterine cancer and colon cancer. Lipella Pharmaceuticals received orphan disease designation for HC, and recently completed a phase 2a clinical trial of LP-10 (intravesical tacrolimus) for the treatment of HC. **Methods:** The LP-10 Phase 2a clinical trial was a multi-center, dose-escalation study (clinicaltrials.gov: NCT01393223). The study recruited subjects with moderate to severe refractory HC. These subjects were treated with up to two courses of LP-10 intravesical bladder instillations (liposomal formulation of tacrolimus at 2 mg, 4 mg and 8 mg). **Results:** Fifteen subjects were screened (14 male and 1 female) and 13 enrolled. All enrolled subjects were male with mean age 67 (range 25-89 years old) with a history of prostate cancer (n = 9), bladder cancer (n = 2) and lymphoma (n = 2). Mean duration of HC was 4 years and ranged from 1-14 years. No subject discontinued treatment or were lost to follow-up. All subjects tolerated LP-10 instillations and completed the study without report of product related serious adverse events. 12 AEs were reported in 6 subjects. Pharmacokinetic analysis demonstrated short duration of low systemic uptake of tacrolimus. A dose response was noted with higher efficacy at both the 4mg and 8 mg dose groups. After LP-10 treatment, the number of cystoscopic bleeding sites and bladder ulcerations decreased and patients' urinary symptoms improved. **Conclusions:** This first phase 2a study demonstrated safety and a signal of efficacy at intravesical tacrolimus (LP-10) doses of 4 mg and 8 mg for the treatment for HC, a rare and serious disease. Clinical trial information: NCT01393223. Research Sponsor: Lipella Pharmaceuticals, Inc.

	Mean	Comment
Age, years	67	Range 25-89
Race	White 9; Non-White 4	
Radiation induced HC	11	
Chemotherapy induced HC	2	
Cancer Prostate cancer	9	
Bladder cancer	2	
Lymphoma	2	
Duration of HC years	4	Range 1 -14 years
Prior HC treatment	13	medication, Hyperbaric oxygen, catheters, surgical procedures