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A Phase II, Randomized, Double-blind, Placebo-controlled Trial of Pluripotent Stem Cell-Derived Mesenchymal Stem Cells (CAStem Cells) Injection in the Treatment of Acute Exacerbation of Interstitial Lung Disease (AE-ILD)

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Background: Acute exacerbation of interstitial lung disease (AE-ILD) is a life-threatening condition. Current therapeutic options are limited.

Methods: This multicenter, randomized, double-blind, placebo-controlled phase II trial enrolled 45 patients with AE-ILD. Participants were randomly assigned in a 1:1:1 ratio to receive low-dose CAStem Cells (1×10^6 cells/kg), high-dose CAStem Cells (3×10^6 cells/kg), or placebo, with 15 patients in each group. All patients received standard care, and the study interventions (CAStem Cells or placebo) were administered via intravenous infusion on days 1, 8, and 15.

Results: CAStem Cell treatment demonstrated significant survival benefits. At 1 month, all-cause mortality and AE-ILD progression-related mortality were 0% in the combined CAStem Cell groups, significantly lower than in the placebo group (20%, P = 0.032). At 3 months, AE-ILD progression-related mortality remained 0% in the CAStem Cell groups, significantly lower than in the placebo group (20%, P = 0.032). The 3-month all-cause mortality was 10% in the combined CAStem Cell groups compared to 20% in the placebo group. Clinical assessment revealed a time-dependent decrease in mMRC dyspnea scores. CAStem Cell treatment promoted the resolution of acute inflammatory exudation and fibrotic lesions on HRCT. By day 90, improvement in the CAStem Cell groups was significantly greater than in the placebo group (P = 0.039). Safety analysis revealed no treatment-related serious adverse events or events leading to study discontinuation.

Conclusion: CAStem Cell injection showed promise in reducing early mortality risk, improving dyspnea symptoms, and promoting radiographic resolution in patients with AE-ILD, with a favorable safety and tolerability profile.