Effective hematopoietic stem cell-based gene therapy in a murine model of hereditary pulmonary alveolar proteinosis


Disclosures: The authors have nothing to disclose.

Contributions: M.H. designed and performed experiments, analyzed data, wrote and edited the manuscript. E.L.R. performed and analyzed lung function measurements, PAS staining, and immunohistochemistry. T.S. and K.S. performed BALF cytokine and cholesterol measurement. A.M., A.H.H.N., and T.B. assisted with organ preparation. A.M. performed VCN analysis. S.D., T.R. and J.P.B. performed and analyzed CT scans. P.M. provided the Lv.Csf2rb vector. A.S., G.H., B.C.T., N.L., and T.M. provided conceptual advice, discussed results and edited the manuscript. N.L. and T.M. conceived and supervised the project and provided financial funding.