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THE DISTILLERY

This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Pulmonary disease				
Emphysema	α_1-Antitrypsin (SERPINA1; AAT; A_1AT)	A study in mice and in cell culture suggests that AAT gene therapy targeting alveolar macrophages could be useful for treating emphysema. In a mouse model of elastase-induced emphysema, intratracheal delivery of the human *AAT* gene to alveolar macrophages using a vesicular stomatitis virus (VSV)-based vector resulted in lower disease severity than delivery of a control vector. Next steps include studying the gene therapy in animal models of tobacco smoke–induced emphysema. Kamada Ltd. markets an i.v. formulation of human plasma-derived AAT to treat emphysema. Respriva AAT, a recombinant AAT from Arriva Pharmaceuticals Inc., is in a Phase II trial for the same indication. Applied Genetic Technologies Corp.'s AGTC-0106, a gene therapy using adeno-associated virus to deliver the *AAT* gene, is in Phase I to treat AAT deficiency.	Unpatented; licensing status not applicable	Wilson, A.A. *et al. J. Clin. Invest.*; published online Dec. 21, 2009; doi:10.1172/JCI36666 **Contact:** Darrell N. Kotton, Boston University School of Medicine, Boston, Mass. e-mail: dkotton@bu.edu

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