

This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Cardiovascular disease				
Heart failure	SMT3 suppressor of mitogen-activated protein kinase 1 (SUMO1); ATPase Ca ⁺⁺ transporting cardiac muscle slow twitch 2 (ATP2A2; SERCA2A)	<p>Pig studies suggest <i>SUMO1</i> gene therapy could help treat heart failure. Previous work in mouse models of heart failure showed that an adeno-associated viral (AAV) vector expressing mouse <i>Sumo1</i> increased cardiac function compared with an AAV vector expressing GFP. In pig models of heart failure, an AAV serotype 1 (AAV1) vector expressing human <i>SUMO1</i>, human <i>SERCA2A</i> or both all led to similar increases in cardiac function compared with saline control. Ongoing work includes selecting which AAV vector and promoter to advance to IND-enabling toxicology studies.</p> <p>Celladon Corp.'s Mydicar, a recombinant AAV vector bearing <i>SERCA2A</i>, is in Phase II testing to treat heart failure and preclinical testing to treat hypertension.</p> <p>SciBX 6(48); doi:10.1038/scibx.2013.1387 Published online Dec. 19, 2013</p>	Patented by the Icahn School of Medicine at Mount Sinai; unlicensed	<p>Tilemann, L. <i>et al. Sci. Transl. Med.</i>; published online Nov. 13, 2013; doi:10.1126/scitranslmed.3006487</p> <p>Contact: Roger J. Hajjar, Icahn School of Medicine at Mount Sinai, New York, N.Y. e-mail: roger.hajjar@mssm.edu</p> <p>Contact: Kiyotake Ishikawa, same affiliation as above e-mail: kiyotake.ishikawa@mssm.edu</p> <p>Contact: Ahyoung Lee, same affiliation as above e-mail: ahyoung.lee@mssm.edu</p> <p>Contact: Lisa Tilemann, same affiliation as above e-mail: lisa.tilemann@googlemail.com</p>