

This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Endocrine/metabolic disease				
Glycosphingolipid storage disorders	Adenosine A _{2A} receptor (ADORA _{2A})	<p><i>In vitro</i> studies suggest ADORA_{2A} agonists could help treat Niemann-Pick disease type C1 (NPC1). ADORA_{2A} plays a role in regulating lysosomal pH and calcium ion (Ca²⁺) levels, which are downregulated in NPC1. In primary fibroblasts from patients with NPC1, the ADORA_{2A} agonist CGS21680 increased lysosomal Ca²⁺ levels and decreased cholesterol accumulation to levels comparable to those of fibroblasts from healthy controls.</p> <p>Next steps could include testing ADORA_{2A} agonists in animal models of NPC1. Adenosine Therapeutics LLC has the ADORA_{2A} agonist Stedivaze apadenoson in Phase III testing for use as a pharmacological stress agent in myocardial perfusion imaging (MPI). Swedish Orphan Biovitrum AB, CBT Development Ltd. and Ergomed Clinical Research Ltd. have the ADORA_{2A} agonist BVT.115959 in Phase II testing to treat pain. Forest Laboratories Inc. and Zalicus Inc. have the ADORA_{2A} agonist ALT313 in preclinical testing to treat cancer. Forest also has ALT313 in preclinical testing to treat pain. CGS21680 is a research reagent.</p> <p>SciBX 6(40); doi:10.1038/scibx.2013.1129 Published online Oct. 17, 2013</p>	Patent and licensing status unavailable	<p>Visentin, S. <i>et al. J. Neurosci.</i>; published online Sept. 25, 2013; doi:10.1523/JNEUROSCI.0558-13.2013 Contact: Patrizia Popoli, National Institute of Health, Rome, Italy e-mail: patrizia.popoli@iss.it</p>