

This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Other				
Progeria	N-acetyltransferase 10 (NAT10)	<i>In vitro</i> studies suggest inhibiting NAT10 could help treat Hutchinson-Gilford progeria syndrome (HGPS) and other laminopathies. In a cell-based model of HGPS, a lysine acetyltransferase-inhibiting compound normalized the aberrant nuclear morphology that is a hallmark of progeria. A chemical derivative of the compound called Remodelin was generated and shown to inhibit NAT10. In cells from patients with HGPS, Remodelin decreased multiple disease markers including misshapen nuclei, DNA damage and excess microtubule anchorage compared with no treatment. Next steps include evaluating the potential of Remodelin or other NAT10 inhibitors in <i>in vivo</i> models of HGPS.	Patent application filed covering Remodelin and structural analogs for the treatment of progeria; licensing discussions under way with undisclosed companies	Larrieu, D. <i>et al. Science</i> ; published online May 2, 2014; doi:10.1126/science.1252651 Contact: Stephen P. Jackson, The Wellcome Trust/Cancer Research UK Gurdon Institute, Cambridge, U.K. e-mail: s.jackson@gurdon.cam.ac.uk Contact: Raphaël Rodriguez, Centre National de la Recherche Scientifique (CNRS), Gif-sur-Yvette, France e-mail: raphael.rodriguez@cns.fr
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