

This week in therapeutics

| Indication | Target/marker/pathway | Summary | Licensing status | Publication and contact information |
|--------------|---|---|---|--|
| Other | | | | |
| Progeria | Mammalian target of rapamycin (mTOR; FRAP; RAFT1) | <p>Cell culture studies suggest rapamycin or another mTOR inhibitor could help treat Hutchinson-Gilford progeria syndrome (HGPS), which causes premature aging and can lead to death from cardiovascular complications. In fibroblasts from HGPS patients, rapamycin corrected nuclear and abnormal protein expression defects and prolonged cellular lifespan compared with vehicle. Next steps include testing the effects of rapamycin in mouse models of progeria. Afinitor everolimus, an mTOR inhibitor from Novartis AG, is marketed to treat renal cell carcinoma (RCC) and prevent transplant rejection.</p> <p>At least eight other companies have mTOR inhibitors in development stages ranging from preclinical to marketed to treat cancer.</p> <p>SciBX 4(28); doi:10.1038/scibx.2011.804 Published online July 21, 2011</p> | Unpatented; licensing status not applicable | <p>Cao, K. <i>et al. Sci. Transl. Med.</i>; published online June 29, 2011; doi:10.1126/scitranslmed.3002346</p> <p>Contact: Dimitri Krainc, Harvard Medical School, Charlestown, Mass. e-mail: krainc@helix.mgh.harvard.edu</p> <p>Contact: Francis S. Collins, National Institutes of Health, Bethesda, Md. e-mail: Francis.Collins@nih.gov</p> |