RESEARCH HIGHLIGHTS

IN BRIEF

GENE THERAPY

Intravascular AAV9 preferentially targets neonatal neurons and adult astrocytes

Goust, K. D. et al. Nature Biotech. 21 Dec 2008 (doi:10.1038/nbt.1515)

Gene delivery across the blood-brain barrier poses a major challenge. A new study uses adeno-associated virus 9 (AAV9) as a delivery vector to effectively target the central nervous system. Intravenous injection of AAV9 led to widespread targeting of neurons in neonatal mice and to transduction of astrocytes in adult mice. These differences in AAV9 tropism might reflect restriction of the virus to particular cell populations depending on developmental stage. This approach may be used to develop gene therapy to treat neurodegenerative diseases.

STEM CELLS

Induced pluripotent stem cells from a spinal muscular atrophy patient

Ebert, A. D. et al. Nature 21 Dec 2008 (doi:10.1038/nature07677)

Until now, no patient-specific induced pluripotent stem (iPS) cells have exhibited disease-associated phenotypes. Now, the first iPS cells that model a specific pathology have been generated. iPS cells isolated from a patient with spinal muscular atrophy (SMA) differentiated into neurons that, as occurs in patients with SMA, lack the expression of the survival motor neuron (SMN) protein and show selective neuronal death. The iPS cells also responded to compounds that increase SMN expression, indicating that this model can be used for screening drugs to treat SMA.

THEORETICAL GENETICS

The population genetics of dN/dS

Kryazhimskiy, S. & Plotkin, J. B. PLoS Genet. 4, e1000304 (2008)

The ratio of the substitution rate at non-synonymous and synonymous sites (dN/dS) was originally introduced to assess the evolutionary pressure on proteins in divergent lineages; however, the test is routinely used on sequences drawn from the same population. By comparing theoretical calculations with a computational simulation, the authors showed that, within a population, dN/dS is insensitive to selective pressure and therefore it cannot accurately assess the degree of protein adaptation. In practice, this means that some species, particularly bacterial ones, might be under more selective pressure than suspected.

GENE REGULATION

Temporal and spatial control of germ plasm RNAs

Rangan, P. et al. Curr. Biol. 24 Dec 2008 (doi:10.1016/j.cub.2008.11.066)

The authors investigated the relationship between the localization of maternal RNAs in the *Drosophila melanogaster* germplasm and their temporal pattern of translation. Localized RNAs were divided into five classes according to their timing of translation. Exchanges between the 3' UTRs of genes in different classes show that this region instructs both localization and the specific timing of expression of a localized RNA, a conclusion that contrasts with the current model that localization merely relieves translational repression. The results might also apply to other cell types, such as migrating fibroblasts, that have localized RNAs.