

# FRONT RUNNERS



**Cover:** Magnetic resonance imaging (MRI) of a stroke in the territory of the middle cerebral artery (MCA) in a mouse (top) and human (bottom). Images are oriented from rostral (left image) to caudal (right image). The mouse images show typical stroke propagation at 24 hours after 1 hour of middle cerebral artery occlusion (MCAO). Cortical and sub-cortical lesions can be seen, corresponding to a severe human stroke. The lower panel shows the stroke of a 65-year-old male patient in the territory of the right MCA. Mouse MRI images are courtesy of Odilo Engel (Charité, Berlin). See Clinical Puzzle by Mergenthaler and Meisel on page 718.

## IN THIS ISSUE

707 **Summaries of selected research papers**

## RESEARCH HIGHLIGHTS

708 **Tissue regeneration in mammals: clues from *Acomys* mice**  
**CF-related diabetes: new insights from ferrets**  
**Immune system eliminates polyploid cancer cells**  
**Niemann-Pick disease and platelet dysfunction**

## JOURNAL CLUB

709 **Finding NECA: zebrafish screen identifies key signalling pathway in  $\beta$ -cell regeneration**

Asha Seth

711 **Lactate-starved neurons in ALS**

Bryan A. Martinez

## A MODEL FOR LIFE

713 **Synergy in science: an interview with Neal Copeland and Nancy Jenkins**

Sarah E. Allan

## CLINICAL PUZZLE

718 **Do stroke models model stroke?**

Philipp Mergenthaler and Andreas Meisel

## PRIMER

726 **Zebrafish models flex their muscles to shed light on muscular dystrophies**

Joachim Berger and Peter D. Currie

## SPECIAL ARTICLE

733 **Preclinical research in Rett syndrome: setting the foundation for translational success**

David M. Katz, Joanne E. Berger-Sweeney, James H. Eubanks, Monica J. Justice, Jeffrey L. Neul, Lucas Pozzo-Miller, Mary E. Blue, Diana Christian, Jacqueline N. Crawley, Maurizio Giustetto, Jacky Guy, C. James Howell, Miriam Kron, Sacha B. Nelson, Rodney C. Samaco, Laura R. Schaevitz, Coryse St. Hillaire-Clarke, Juan L. Young, Huda Y. Zoghbi and Laura A. Mamounas

## PERSPECTIVE

746 **Dysregulation of cholesterol balance in the brain: contribution to neurodegenerative diseases**

Jean E. Vance

## COMMENTARIES

756 **Fibrodysplasia ossificans progressiva: mechanisms and models of skeletal metamorphosis**

Frederick S. Kaplan, Salin A. Chakkalakal and Eileen M. Shore

763 **Understanding the hypoxic niche of multiple myeloma: therapeutic implications and contributions of mouse models**

Jinsong Hu, Els Van Valckenborgh, Eline Menu, Elke De Bruyne and Karin Vanderkerken

## ORIGINAL RESEARCH

## RESEARCH ARTICLES

- 773 Identification of compounds with anti-convulsant properties in a zebrafish model of epileptic seizures**  
Sarah Baxendale, Celia J. Holdsworth, Paola L. Meza Santoscoy, Michael R. M. Harrison, James Fox, Caroline A. Parkin, Philip W. Ingham and Vincent T. Cunliffe
- 785 Zebrafish larvae exposed to ginkgotoxin exhibit seizure-like behavior that is relieved by pyridoxal-5'-phosphate, GABA and anti-epileptic drugs**  
Gang-Hui Lee, Shian-Ying Sung, Wen-Ni Chang, Tseng-Ting Kao, Hung-Chi Du, Tsun-Hsien Hsiao, Martin K. Safo and Tzu-Fun Fu
- 796 Mediators of a long-term movement abnormality in a *Drosophila melanogaster* model of classic galactosemia**  
Emily L. Ryan, Brian DuBoff, Mel B. Feany and Judith L. Fridovich-Keil
- 804 Characterization of a canine model of glycogen storage disease type IIIa**  
Haiqing Yi, Beth L. Thurberg, Sarah Curtis, Stephanie Austin, John Fyfe, Dwight D. Koeberl, Priya S. Kishnani and Baodong Sun
- 812 Deletion of a conserved regulatory element required for Hmx1 expression in craniofacial mesenchyme in the dumbo rat: a newly identified cause of congenital ear malformation**  
Lely A. Quina, Takashi Kuramoto, Daniela V. Luquetti, Timothy C. Cox, Tadao Serikawa and Eric E. Turner
- 823 Interleukin-1 receptor antagonist is beneficial after subarachnoid haemorrhage in rat by blocking haem-driven inflammatory pathology**  
Andrew D. Greenhalgh, David Brough, Emily M. Robinson, Sylvie Girard, Nancy J. Rothwell and Stuart M. Allan
- 834 Zebrafish homologs of genes within 16p11.2, a genomic region associated with brain disorders, are active during brain development, and include two deletion dosage sensor genes**  
Alicia Blaker-Lee, Sunny Gupta, Jasmine M. McCammon, Gianluca De Rienzo and Hazel Sive
- 852 Myotubular myopathy and the neuromuscular junction: a novel therapeutic approach from mouse models**  
James J. Dowling, Romain Joubert, Sean E. Low, Ashley N. Durban, Nadia Messaddeq, Xingli Li, Ashley N. Dulin-Smith, Andrew D. Snyder, Morgan L. Marshall, Jordan T. Marshall, Alan H. Beggs, Anna Buj-Bello and Christopher R. Pierson
- 860 Clinical data and characterization of the liver conditional mouse model exclude neoplasia as a non-neurological manifestation associated with Friedreich's ataxia**  
Alain Martelli, Lisa S. Friedman, Laurence Reutenauer, Nadia Messaddeq, Susan L. Perlman, David R. Lynch, Kathrin Fedosov, Jörg B. Schulz, Massimo Pandolfo and H  l  ne Puccio
- 870 Neonatal diethylstilbestrol exposure alters the metabolic profile of uterine epithelial cells**  
Yan Yin, Congxing Lin, G. Michael Veith, Hong Chen, Maulik Dhandha and Liang Ma

## ORIGINAL RESEARCH

- 881 Zebrafish neurofibromatosis type 1 genes have redundant functions in tumorigenesis and embryonic development**  
Jimann Shin, Arun Padmanabhan, Eric D. de Groh, Jeong-Soo Lee, Sam Haidar, Suzanne Dahlberg, Feng Guo, Shuning He, Marc A. Wolman, Michael Granato, Nathan D. Lawson, Scot A. Wolfe, Seok-Hyung Kim, Lilianna Solnica-Krezel, John P. Kanki, Keith L. Ligon, Jonathan A. Epstein and A. Thomas Look
- 895 Sertoli-cell-specific knockout of connexin 43 leads to multiple alterations in testicular gene expression in prepubertal mice**  
Sarah Giese, Hamid Hossain, Melanie Markmann, Trinad Chakraborty, Svetlin Tchatalbachev, Florian Guillou, Martin Bergmann, Klaus Failing, Karola Weider and Ralph Brehm
- 914 Initiation of prostate cancer in mice by *Tp53*<sup>R270H</sup>: evidence for an alternative molecular progression**  
Ruth L. Vinall, Jane Q. Chen, Neil E. Hubbard, Shola S. Sulaimon, Michael M. Shen, Ralph W. DeVere White and Alexander D. Borowsky
- 921 Spinocerebellar ataxia type 13 mutation that is associated with disease onset in infancy disrupts axonal pathfinding during neuronal development**  
Fadi A. Issa, Allan F. Mock, Alvaro Sagasti and Diane M. Papazian
- RESEARCH REPORTS**
- 930 The cystic-fibrosis-associated  $\Delta F508$  mutation confers post-transcriptional destabilization on the *C. elegans* ABC transporter PGP-3**  
Liping He, Jennifer Skirkanich, Lorenza Moronetti, Rosemary Lewis and Todd Lamitina
- 940 Tumorigenic fragments of APC cause dominant defects in directional cell migration in multiple model systems**  
Scott A. Nelson, Zhouyu Li, Ian P. Newton, David Fraser, Rachel E. Milne, David M. A. Martin, David Schiffmann, Xuesong Yang, Dirk Dormann, Cornelis J. Weijer, Paul L. Appleton and Inke S. Näthke
- 948 Reduced bone morphogenetic protein receptor type 1A signaling in neural-crest-derived cells causes facial dysmorphism**  
Hiromitsu Saito, Ken-ichi Yamamura and Noboru Suzuki
- RESOURCE ARTICLE**
- 956 Partial promoter substitutions generating transcriptional sentinels of diverse signaling pathways in embryonic stem cells and mice**  
Palle Serup, Carsten Gustavsén, Tino Klein, Leah A. Potter, Robert Lin, Nandita Mullapudi, Ewa Wandzioch, Angela Hines, Ashley Davis, Christine Bruun, Nina Engberg, Dorthe R. Petersen, Janny M. L. Peterslund, Raymond J. MacDonald, Anne Grapin-Botton, Mark A. Magnuson and Kenneth S. Zaret