

- 2) Eriksson M, Hedberg B, Carey N, Ansved T: Decreased DMPK transcript levels in myotonic dystrophy 1 type IIA muscle fibers. *Biochem Biophys Res Commun* **286**: 1177-1182, 2001
- 3) Zu T, Gibbens B, Doty NS, Gomes-Pereira M, Huguet A, et al: Non-ATG-initiated translation directed by microsatellite expansions. *Proc Natl Acad Sci U S A* **108**: 260-265, 2011
- 4) Taneja KL, McCurranch M, Schalling M, Housman D, Singer RH: Foci of trinucleotide repeat transcripts in nuclei of myotonic dystrophy cells and tissues. *J Cell Biol* **128**: 995-1002, 1995
- 5) Michalowski S, Miller JW, Urbinati CR, Paliouras M, Swanson MS, et al: Visualization of double-stranded RNAs from the myotonic dystrophy protein kinase gene and interactions with CUG-binding protein. *Nucleic Acids Res* **27**: 3534-3542, 1999
- 6) Ho TH, Savkur RS, Poulos MG, Mancini MA, Swanson MS, et al: Colocalization of muscleblind with RNA foci is separable from mis-regulation of alternative splicing in myotonic dystrophy. *J Cell Sci* **118**: 2923-2933, 2005
- 7) Savkur RS, Phillips AV, Cooper TA: Aberrant regulation of insulin receptor alternative splicing is associated with insulin resistance in myotonic dystrophy. *Nat Genet* **29**: 40-47, 2001
- 8) Salisbury E, Sakai K, Schoser B, Huichalaf C, Schneider-Gold C, et al: Ectopic expression of cyclin D3 corrects differentiation of DM1 myoblasts through activation of RNA CUG-binding protein, CUGBP1. *Exp Cell Res* **314**: 2266-2278, 2008
- 9) Kuyumcu-Martinez NM, Wang GS, Cooper TA: Increased steady-state levels of CUGBP1 in myotonic dystrophy 1 are due to PKC-mediated hyperphosphorylation. *Mol Cell* **28**: 68-78, 2007
- 10) Jin J, Wang GL, Salisbury E, Timchenko L, Timchenko NA: GSK3beta-cyclin D3-CUGBP1-eIF2 pathway in aging and in myotonic dystrophy. *Cell Cycle* **8**: 2356-2359, 2009
- 11) Kimura T, Nakamori M, Lueck JD, Pouliquin P, Aoike F, et al: Altered mRNA splicing of the skeletal muscle ryanodine receptor and sarcoplasmic/endoplasmic reticulum Ca²⁺-ATPase in myotonic dystrophy type 1. *Hum Mol Genet* **14**: 2189-2200, 2005
- 12) Tang ZZ, Yarotskyy V, Wei L, Sobvzak K, Nakamori E, et al: Muscle weakness in myotonic dystrophy associated with misregulated splicing and altered gating of Ca_v 1.1 calcium channel. *Hum Mol Genet* **21**: 1312-1324, 2012
- 13) Fugier C, Klein AF, Hammer C, Vassilopoulos S, Ivarsson Y, et al: Mis-regulated alternative splicing of BIN1 is associated with T tubule alterations and muscle weakness in myotonic dystrophy. *Nat Med* **17**: 720-725, 2011
- 14) Kanadia RN, Shin J, Yuan Y, Beattie SG, Wheeler TM, et al: Reversal of RNA missplicing and myotonia after muscleblind overexpression in a mouse poly (CUG) model for myotonic dystrophy. *Proc Natl Acad Sci U S A* **103**: 111748-111753, 2006
- 15) Mankodi A, Takahashi MP, Jiang H, Beck CL, Bowers WJ, et al: Expanded CUG repeats trigger aberrant splicing of CIC-1 chloride channel pre-mRNA and hyperexcitability of skeletal muscle in myotonic dystrophy. *Mol Cell* **10**: 35-44, 2002
- 16) Jacobs AE, Benders AA, Oosterhof A, Veerkamp JH, van Mier P, et al: The calcium homeostasis and the membrane potential of cultured muscle cells from patients with myotonic dystrophy. *Biochim Biophys Acta* **1096**: 14-19, 1990
- 17) Tang W, Sencer S, Hamilton SL: Calmodulin modulation of proteins involved in excitation-contraction coupling. *Front Biosci* **7**: d1583-d1589, 2002
- 18) Klesert TR, Cho DH, Clark JI, Maylie J, Adelman J, et al: Mice deficient in Six5 develop cataracts: implications for myotonic dystrophy. *Nat Genet* **25**: 105-109, 2000
- 19) Wheeler TM, Sobczak K, Lueck JD, Osborne RJ, Lin X, et al: Reversal of RNA dominance by displacement of protein sequestered on triplet repeat RNA. *Science* **325**: 336-339, 2009
- 20) Wheeler TM, Leger AJ, Pandey SK, Macleod AR, Nakamori M, et al: Targeting nuclear RNA for in vivo correction of myotonic dystrophy. *Nature* **488**: 111-115, 2012
- 21) Gonzalez-Barriga A, Mulders SA, van de Giessen J, Hooijer JD, Bijl S, et al: Design and analysis of effects of triplet repeat oligonucleotides in cell models for myotonic dystrophy. *Mol Ther Nucleic Acids*, 2013 Mar 19; **2**: e81. doi: 10.1038/mtna.2013.9
- 22) Warf MB, Nakamori M, Matthys CM, Thornton CA, Berglund JA: Pentamidine reverses the splicing defects associated with myotonic dystrophy. *Proc Natl Acad Sci U S A* **106**: 18551-18556, 2009
- 23) Huichalaf C, Sakai K, Jin B, Jones K, Wang GL, et al: Expansion of CUG RNA repeats causes stress and inhibition of translation in myotonic dystrophy 1 (DM1) cells. *FASEB J* **24**: 3705-3719, 2010
- 24) Jones K, Wei C, Iakova P, Bugiardini E, Schneider-Gold C, et al: GSK3beta mediates muscle pathology in myotonic dystrophy. *J Clin Invest* **122**: 4461-4472, 2012
- 25) Wang GS, Kuyumcu-Martinez MN, Sarma S, Mathur N, Wehrens XH, et al: PKC inhibition ameliorates the cardiac phenotype in a mouse model of myotonic dystrophy type 1. *J Clin Invest* **119**: 3797-3806, 2009

