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This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Musculoskeletal disease	Calcium release–activated calcium channel (CRAC); stromal interaction molecule 1 (STIM1); transmembrane protein 142A (ORAI1; TMEM142A; CRACM1)	*In vitro* and genetic studies suggest decreasing calcium influx through CRAC could help treat Stormorken syndrome and related diseases. Stormorken syndrome is a tubular myopathy with symptoms that include abnormal bleeding. Sequencing studies identified the p.R304M mutation in *STIM1* in two patients with Stormorken syndrome and the p.P2452 mutation in *ORAI1* in two patients with a Stormorken-like syndrome that lacked bleeding symptoms. In human embryonic kidney cells, expression of the *STIM1* mutation caused constitutive activation of CRAC and expression of the *ORAI1* mutation also increased calcium influx by suppressing channel inactivation. Next steps could include developing specific CRAC inhibitors. CalciMedica Inc.'s CRAC inhibitor CM2489 is in Phase I testing to treat psoriasis. Rhizen Pharmaceuticals S.A. and Synta Pharmaceuticals Corp. have CRAC inhibitors in preclinical development for autoimmune and inflammatory indications. ***SciBX*** **7(13); doi:10.1038/scibx.2014.377** **Published online April 3, 2014**	Patent status not applicable; unavailable for licensing	Nesin, V. *et al. Proc. Natl. Acad. Sci. USA*; published online March 3, 2014; doi:10.1073/pnas.1312520111 **Contact:** Leonidas Tsiokas, University of Oklahoma Health Sciences Center, Oklahoma City, Okla. e-mail: leonidas-tsiokas@ouhsc.edu **Contact:** Klaas J. Wierenga, same affiliation as above e-mail: klaas-wierenga@ouhsc.edu