

Stasimon Contributes to the Loss of Sensory Synapses and Motor Neuron Death in a Mouse Model of Spinal Muscular Atrophy. Simon CM, Van Alstyne M, Lotti F, Bianchetti E, Tisdale S, Watterson DM, Mentis GZ, Pellizzoni L. Cell Rep. 2019 Dec 17;29(12):3885-3901.e5. doi: 10.1016/j.celrep.2019.11.058.

Splicing Defects of the Profilin Gene Alter Actin Dynamics in an *S. pombe* SMN Mutant. Antoine M, Patrick KL, Soret J, Duc P, Rage F, Cacciottolo R, Nissen KE, Cauchi RJ, Krogan NJ, Guthrie C, Gachet Y, Bordonné R. iScience. 2019 Dec 28;23(1):100809. doi: 10.1016/j.isci.2019.100809. [Epub ahead of print]

Serum creatinine is a biomarker of progressive denervation in spinal muscular atrophy. Alves CRR, Zhang R, Johnstone AJ, Garner R, Nwe PH, Siranosian JJ, Swoboda KJ. Neurology. 2019 Dec 27. pii: 10.1212/WNL.0000000000008762. doi: 10.1212/WNL.0000000000008762. [Epub ahead of print]

R-Roscovitine Improves Motoneuron Function in Mouse Models For Spinal Muscular Atrophy. Tejero R, Balk S, Franco-Espin J, Ojeda J, Hennlein L, Drexel H, Dombert B, Clausen JD, Torres-Benito L, Saal-Bauernschubert L, Blum R, Briese M, Appenzeller S, Tabares L, Jablonka S.

Muscle-specific SMN reduction reveals motor neuron-independent disease in spinal muscular atrophy models. Kim JK, Jha NN, Feng Z, Faleiro MR, Chiriboga CA, Wei-Lapierre L, Dirksen RT, Ko CP, Monani UR. J Clin Invest. 2020 Feb 10. pii: 131989. doi: 10.1172/JCI131989. [Epub ahead of print]

Comparison of the efficacy of MOE and PMO modifications of systemic antisense oligonucleotides in a severe SMA mouse model. Sheng L, Rigo F, Bennett CF, Krainer AR, Hua Y. Nucleic Acids Res. 2020 Feb 27. pii: gkaa126. doi: 10.1093/nar/gkaa126. [Epub ahead of print]

Motor transmission defects with sex differences in a new mouse model of mild spinal muscular atrophy. Deguise MO, De Repentigny Y, Tierney A, Beauvais A, Michaud J, Chehade L, Thabet M, Paul B, Reilly A, Gagnon S, Renaud JM, Kothary R. EBioMedicine. 2020 Apr 24;55:102750. doi: 10.1016/j.ebiom.2020.102750. [Epub ahead of print]

Blood Flow to the Spleen is Altered in a Mouse Model of Spinal Muscular Atrophy. Deguise MO, Beauvais A, Schneider BL, Kothary R. J Neuromuscul Dis. 2020 Apr 24. doi: 10.3233/JND-200493. [Epub ahead of print]

SMN-deficiency disrupts SERCA2 expression and intracellular Ca<sub>2+</sub> signaling in cardiomyocytes from SMA mice and patient-derived iPSCs. Khayrullina G, Moritz KE, Schooley JF, Fatima N, Viollet C, McCormack NM, Smyth JT, Doughty ML, Dalgard CL, Flagg TP, Burnett BG. Skelet Muscle. 2020 May 8;10(1):16. doi: 10.1186/s13395-020-00232-7.

Whole Blood SMN Protein Levels Correlate with Severity of Denervation in Spinal Muscular Atrophy. Alves CRR, Zhang R, Johnstone AJ, Garner R, Eichelberger EJ, Da

Silva Duarte Lepez S, Yi V, Stevens V, Poxson R, Schwartz R, Zaworski P, Swoboda KJ. Muscle Nerve. 2020 Jun 8. doi: 10.1002/mus.26995. Online ahead of print. PMID: 32511765

SMN-deficiency disrupts SERCA2 expression and intracellular Ca(2+) signaling in cardiomyocytes from SMA mice and patient-derived iPSCs. Khayrullina G, Moritz KE, Schooley JF, Fatima N, Viollet C, McCormack NM, Smyth JT, Doughty ML, Dalgard CL, Flagg TP, Burnett BG. Skelet Muscle. 2020 May 8;10(1):16. doi: 10.1186/s13395-020-00232-

Stander Use in Spinal Muscular Atrophy: Results From a Large Natural History Database. Townsend EL, Simeone SD, Krosschell KJ, Zhang RZ, Swoboda KJ; Project Cure SMA Investigator's Network. Pediatr Phys Ther. 2020 Jun 16. doi: 10.1097/PEP.0000000000000713. Online ahead of print. PMID: 32569095

Whole-blood dysregulation of actin-cytoskeleton pathway in adult spinal muscular atrophy patients. Siranosian JJ, Nery FC, Alves CRR, Siranosian BA, Lyons NJ, Eichelberger EJ, Garner R, Da Silva Duarte Lepez S, Johnstone AJ, Subramanian A, Swoboda KJ. Ann Clin Transl Neurol. 2020 Jun 17. doi: 10.1002/acn3.51092. Online ahead of print. PMID: 32558393

Neuroanatomical Models of Muscle Strength and Relationship to Ambulatory Function in Spinal Muscular Atrophy Rodriguez-Torres, Rafael<sup>a; 1</sup> | Fabiano, Julia<sup>a; 1</sup> | Goodwin, Ashley<sup>a</sup> | Rao, Ashwini K.<sup>a; c</sup> | Kinirons, Stacy<sup>a</sup> | De Vivo, Darryl<sup>b</sup> | Montes, Jacqueline<sup>a; b; \*</sup> Journal of Neuromuscular Diseases, vol. 7, no. 4, pp. 459-466, 2020

Quality of life data for individuals affected by spinal muscular atrophy: a baseline dataset from the Cure SMA Community Update Survey. Belter L, Cruz R, Jarecki J. Orphanet J Rare Dis. 2020 Aug 24;15(1):217. doi: 10.1186/s13023-020-01498-2.

The SMA Clinical Trial Readiness Program: creation and evaluation of a program to enhance SMA trial readiness in the United States. Peterson I, Cruz R, Sarr F, Stanley AM, Jarecki J. Orphanet J Rare Dis. 2020 May 22;15(1):118. doi: 10.1186/s13023-020-01387-8.

Revised Recommendations for the Treatment of Infants Diagnosed with Spinal Muscular Atrophy Via Newborn Screening Who Have 4 Copies of SMN2. Glascock J, Sampson J, Connolly AM, Darras BT, Day JW, Finkel R, Howell RR, Klinger KW, Kuntz N, Prior T, Shieh PB, Crawford TO, Kerr D, Jarecki J. J Neuromuscul Dis. 2020;7(2):97-100. doi: 10.3233/JND-190468.

The Cure SMA Membership Surveys: Highlights of Key Demographic and Clinical Characteristics of Individuals with Spinal Muscular Atrophy. Belter L, Jarecki J, Reyna SP, Cruz R, Jones CC, Schroth M, O'Toole CM, O'Brien S, Hall SA, Johnson NB, Paradis AD.

Prospective Cohort Study of Nusinersen Treatment in Adults with Spinal Muscular Atrophy. Yeo CJ, Simeone SD, Townsend EL, Zhang RZ, Swoboda KJ. J Neuromuscul Dis. 2020;7(3):257-268. doi: 10.3233/JND-190453.

Clinical Variability in Spinal Muscular Atrophy Type III.

Coratti G, Messina S, Lucibello S, Pera MC, Montes J, Pasternak A, Bovis F, Exposito Escudero J, Mazzone ES, Mayhew A, Glanzman AM, Young SD, Salazar R, Duong T, Muni Lofra R, De Sanctis R, Carnicella S, Milev E, Civitello M, Pane M, Scoto M, Bettolo CM, Antonaci L, Frongia A, Sframeli M, Vita GL, D'Amico A, Van Den Hauwe M, Albamonte E, Goemans N, Darras BT, Bertini E, Sansone V, Day J, Nascimento Osorio A, Bruno C, Muntoni F, De Vivo DC, Finkel RS, Mercuri E. *Ann Neurol.* 2020 Dec;88(6):1109-1117. doi: 10.1002/ana.25900. Epub 2020 Oct 2.

Short-duration splice promoting compound enables a tunable mouse model

of spinal muscular atrophy. Rietz A, Hodgetts KJ, Lusic H, Quist KM, Osman EY, Lorson CL, Androphy EJ. *Life Sci Alliance.* 2020 Nov 24;4(1):e202000889. doi: 10.26508/lsa.202000889. Print 2021 Jan. PMID: 33234679

Economic burden of spinal muscular atrophy: an analysis of claims data.

Belter L, Cruz R, Kulas S, McGinnis E, Dabbous O, Jarecki J.J *Mark Access Health Policy.* 2020 Nov 8;8(1):1843277. doi: 10.1080/20016689.2020.1843277. PMID: 33224449