



## Tina Duong MPT, PhD

Senior Research Scientist, Neurology

### Bio

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#### BIO

I am a research physical therapist with over 19 years of experience as a practicing clinician and most of it in neuromuscular clinical research. My doctoral studies focused on effects of contracture development on downstream musculoskeletal and biomechanical changes associated with changes in function. I hope to continue work in novel outcomes development with other neuromuscular diseases as well as pursue work on the benefits of rehabilitation and exercise as conjunctive therapies in neuromuscular disease.

Character is the driving force in my work and collaborations. The most important qualities I find in people are integrity, work ethic, humility, empathy, leadership, initiative and drive...I live by Ralph Waldo Emerson's Success poem..."to know even one life has breathed easier because you have lived. This is to have succeeded." I find it difficult at times to summarize ones own accomplishments or impact. So I would like to share a recommendation that was written about me in which I hope to refer back as my north star in both my personal and professional life.

"Dr Tina Duong is a world renown physiotherapist, master trainer, clinical investigator, scientific academician and most importantly an INCREDIBLE person. I had the privilege of working with her side by side during the development of a new therapy for spinal muscular atrophy. Her determination to help the scientific community, patients and carers is truly inspiring. Her knowledge and skills place her at the vanguard of clinical translation of data and meaningful patient outcomes. Her capabilities span everything from publications, meeting moderation, speaker events and clinical training. She has instinctive clinical intuition which allows her to support drug development and translation in both early stage and also during pivotal trial design, data interpretation and patient care and management. Her ethical and moral considerations of medicine and science means she is 100% focused on each individual to support them as best as possible and this is obvious from everyone who has worked or knows her. Her passion, energy and knowledge inspires people, teams and countries! Wherever Tina goes and whatever she embarks on now or in the future, the value she brings is like no one else and her impact is immediate. I personally look forward to the next opportunity to work with Tina as a brilliant scholar. She lives the ambition of: "The world is changed by your example, not by your opinion"

#### LINKS

- Leading Investigators and Rising Stars in DMD: <https://www.monoclonal.com/blog/key-opinion-leaders-dmd/>
- DMD Clinical Outcomes: Northstar Ambulatory Assessment Educational Video: [https://www.youtube.com/watch?v=pku\\_pbPjfCk](https://www.youtube.com/watch?v=pku_pbPjfCk)
- DMD Clinical Outcomes: Performance of Upper Limb (PUL) Video: <https://www.youtube.com/watch?v=jRUfrZLV6i8>
- StretchOUT: Educational Video for DMD: <https://cinrgresearch.org/publications/stretch-out/>
- ATEND Scale: <https://med.stanford.edu/day-lab/atend.html>
- Role of Physical Therapy in Myotonic Dystrophy: [https://www.myotonic.org/sites/default/files/pages/files/MDF\\_RoleofPhysicalTherapy\\_1\\_21.pdf?fbclid=IwAR06tAcgAKivWBXBFq0sFEbo-VicRNJDRp1Me\\_9lgomqJ8S3sRUOS1hvDc](https://www.myotonic.org/sites/default/files/pages/files/MDF_RoleofPhysicalTherapy_1_21.pdf?fbclid=IwAR06tAcgAKivWBXBFq0sFEbo-VicRNJDRp1Me_9lgomqJ8S3sRUOS1hvDc)

## Publications

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### PUBLICATIONS

- **Nusinersen Treatment in Adults With Spinal Muscular Atrophy.** *Neurology. Clinical practice*  
Duong, T., Wolford, C., McDermott, M. P., Macpherson, C. E., Pasternak, A., Glanzman, A. M., Martens, W. B., Kichula, E., Darras, B. T., De Vivo, D. C., Zolkipli-Cunningham, Z., Finkel, R. S., Zeineh, et al  
2021; 11 (3): e317-e327
- **Understanding the relationship between the 32-item motor function measure and daily activities from an individual with spinal muscular atrophy and their caregivers' perspective: a two-part study** *BMC NEUROLOGY*  
Duong, T., Braid, J., Staunton, H., Barriere, A., Petridis, F., Reithinger, J., Cruz, R., Jarecki, J., De Lemus, M., Gusset, N., Broekgaarden, R., Randhawa, S., Flynn, et al  
2021; 21 (1): 143
- **The Minimal Clinically Important Difference (MCID) in Annual Rate of Change of Timed Function Tests in Boys with DMD.** *Journal of neuromuscular diseases*  
Duong, T., Canbek, J., Birkmeier, M., Nelson, L., Siener, C., Fernandez-Fernandez, A., Henricson, E., McDonald, C. M., Gordish-Dressman, H.  
2021
- **Use of the Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND) in X-Linked Myotubular Myopathy: Content Validity and Psychometric Performance** *JOURNAL OF NEUROMUSCULAR DISEASES*  
Duong, T., Harding, G., Mannix, S., Abel, C., Phillips, D., Alfano, L. N., Bonnemann, C. G., Lilien, C., Lowes, L. P., Servais, L., Warken-Madelung, B., Bergman, S., James, et al  
2021; 8 (1): 63-77
- **Knee Strength and Ankle Range of Motion Impacts on Timed Function Tests in Duchenne Muscular Dystrophy: In the Era of Glucocorticoids.** *Journal of neuromuscular diseases*  
Duong, T., Canbek, J., Fernandez-Fernandez, A., Henricson, E., Birkmeier, M., Siener, C., Rocha, C. T., McDonald, C., Gordish-Dressman, H.  
2021
- **A Patient-Centered Evaluation of Meaningful Change on the 32-Item Motor Function Measure in Spinal Muscular Atrophy Using Qualitative and Quantitative Data.** *Frontiers in neurology*  
Duong, T., Staunton, H., Braid, J., Barriere, A., Trzaskoma, B., Gao, L., Willgoss, T., Cruz, R., Gusset, N., Gorni, K., Randhawa, S., Yang, L., Vuillerot, et al  
1800; 12: 770423
- **Consensus Guidelines for Improving Quality of Assessment and Training for Neuromuscular Diseases.** *Frontiers in genetics*  
Duong, T., Krossschell, K. J., James, M. K., Nelson, L., Alfano, L. N., Eichinger, K., Mazzone, E., Rose, K., Lowes, L. P., Mayhew, A., Florence, J., King, W., Senesac, et al  
2021; 12: 735936
- **Correction to: Understanding the relationship between the 32-item motor function measure and daily activities from an individual with spinal muscular atrophy and their caregivers' perspective: a two-part study.** *BMC neurology*  
Duong, T., Braid, J., Staunton, H., Barriere, A., Petridis, F., Reithinger, J., Cruz, R., Jarecki, J., De Lemus, M., Gusset, N., Broekgaarden, R., Randhawa, S., Flynn, et al  
2021; 21 (1): 354
- **Determining minimal clinically important differences in the Hammersmith Functional Motor Scale Expanded for untreated spinal muscular atrophy patients: An international study.** *European journal of neurology*  
Coratti, G., Bovis, F., Pera, M. C., Scoto, M., Montes, J., Pasternak, A., Mayhew, A., Muni-Lofra, R., Duong, T., Rohwer, A., Dunaway Young, S., Civitello, M., Salmin, et al  
2024: e16309
- **Safety and Efficacy of Apitegromab in Patients With Spinal Muscular Atrophy Types 2 and 3: The Phase 2 TOPAZ Study.** *Neurology*  
Crawford, T. O., Darras, B. T., Day, J. W., Dunaway Young, S., Duong, T., Nelson, L. L., Barrett, D., Song, G., Bilic, S., Cote, S., Sadanowicz, M., Iarrobino, R., Xu, et al  
2024; 102 (5): e209151
- **Disease Trajectories in the Revised Hammersmith Scale in a Cohort of Untreated Patients with Spinal Muscular Atrophy types 2 and 3.** *Journal of neuromuscular diseases*

- Wolfe, A., Stimpson, G., Ramsey, D., Coratti, G., Dunaway Young, S., Mayhew, A., Pane, M., Rohwer, A., Muni Lofra, R., Duong, T., O'Reilly, E., Milev, E., Civitello, et al  
2024
- **Assessing the Assisted Six-Minute Cycling Test as a Measure of Endurance in Non-Ambulatory Patients with Spinal Muscular Atrophy (SMA).** *Journal of clinical medicine*  
Tang, W. J., Gu, B., Montalvo, S., Dunaway Young, S., Parker, D. M., de Monts, C., Ataide, P., Ni Ghiollagain, N., Wheeler, M. T., Tesi Rocha, C., Christle, J. W., He, Z., Day, et al  
2023; 12 (24)
  - **Safety and efficacy of gene replacement therapy for X-linked myotubular myopathy (ASPIRO): a multinational, open-label, dose-escalation trial.** *The Lancet. Neurology*  
Shieh, P. B., Kuntz, N. L., Dowling, J. J., Muller-Felber, W., Bonnemann, C. G., Seferian, A. M., Servais, L., Smith, B. K., Muntoni, F., Blaschek, A., Foley, A. R., Saade, D. N., Neuhaus, et al  
2023; 22 (12): 1125-1139
  - **Cerebrospinal Fluid Proteomic Changes after Nusinersen in Patients with Spinal Muscular Atrophy.** *Journal of clinical medicine*  
Beaudin, M., Kamali, T., Tang, W., Hagerman, K. A., Dunaway Young, S., Ghiglieri, L., Parker, D. M., Lehallier, B., Tesi-Rocha, C., Sampson, J. B., Duong, T., Day, J. W.  
2023; 12 (20)
  - **Patient reported outcome measure for upper limb in Duchenne muscular dystrophy: correlation with PUL2.0.** *Neuromuscular disorders : NMD*  
Cicala, G., Pane, M., Coratti, G., Brogna, C., Fanelli, L., Norcia, G., Forcina, N., Mazzone, E., Stanca, G., Ferrante, R., Vento, A., Ferraroli, E., Ricci, et al  
2023
  - **Correction to: Risdiplam in Patients Previously Treated with Other Therapies for Spinal Muscular Atrophy: An Interim Analysis from the JEWELFISH Study.** *Neurology and therapy*  
Chiriboga, C. A., Bruno, C., Duong, T., Fischer, D., Mercuri, E., Kirschner, J., Kostera-Pruszczyk, A., Jaber, B., Gorni, K., Kletzl, H., Carruthers, I., Martin, C., Warren, et al  
2023
  - **Major Adverse Dystrophinopathy Events (MADE) Score as Marker of Cumulative Morbidity and Risk for Mortality in Boys with Duchenne Muscular Dystrophy.** *Progress in pediatric cardiology*  
Kaufman, B. D., Garcia, A., He, Z., Tesi-Rocha, C., Buu, M., Rosenthal, D., Gordish-Dressman, H., Almond, C. S., Duong, T.  
2023; 69
  - **Major Adverse Dystrophinopathy Events (MADE) score as marker of cumulative morbidity and risk for mortality in boys with Duchenne muscular dystrophy** *PROGRESS IN PEDIATRIC CARDIOLOGY*  
Kaufman, B. D., Garcia, A., He, Z., Tesi-Rocha, C., Buu, M., Rosenthal, D., Gordish-Dressman, H., Almond, C. S., Duong, T., CINRG DUCHENNE NAT HIST STUDY  
2023; 69
  - **Development of an International SMA Bulbar Assessment for Inter-professional Administration.** *Journal of neuromuscular diseases*  
Young, S. D., McGrattan, K., Johnson, E., van der Heul, M., Duong, T., Bakke, M., Werlauff, U., Pasternak, A., Cattaneo, C., Hoffman, K., Fanelli, L., Breaks, A., Allison, et al  
2023
  - **JEWELFISH: 24-month Safety, Pharmacodynamic and Exploratory Efficacy Data in Non-Treatment-Naive Patients with Spinal Muscular Atrophy (SMA) Receiving Treatment with Risdiplam**  
Chiriboga, C., Bruno, C., Duong, T., Fischer, D., Kirschner, J., Scoto, M., Mercuri, E., Gerber, M., Gorni, K., Kletzl, H., Carruthers, I., Martin, C., Gidaro, et al  
LIPPINCOTT WILLIAMS & WILKINS.2023
  - **Antisense oligonucleotide targeting DMPK in patients with myotonic dystrophy type 1: a multicentre, randomised, dose-escalation, placebo-controlled, phase 1/2a trial** *LANCET NEUROLOGY*  
Thornton, C. A., Iii, R., Eichinger, K., Heatwole, C., Mignon, L., Arnold, W., Ashizawa, T., Day, J. W., Dent, G., Tanner, M. K., Duong, T., Greene, E. P., Herbelin, et al  
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  - **Antisense oligonucleotide targeting DMPK in patients with myotonic dystrophy type 1: a multicentre, randomised, dose-escalation, placebo-controlled, phase 1/2a trial.** *The Lancet. Neurology*  
Thornton, C. A., Moxley, R. T., Eichinger, K., Heatwole, C., Mignon, L., Arnold, W. D., Ashizawa, T., Day, J. W., Dent, G., Tanner, M. K., Duong, T., Greene, E. P., Herbelin, et al

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- **2-Year Change in Revised Hammersmith Scale Scores in a Large Cohort of Untreated Paediatric Type 2 and 3 SMA Participants.** *Journal of clinical medicine*  
Stimpson, G., Ramsey, D., Wolfe, A., Mayhew, A., Scoto, M., Baranello, G., Muni Lofra, R., Main, M., Milev, E., Coratti, G., Pane, M., Sansone, V., D'Amico, et al  
2023; 12 (5)
- **Risdiplam in Patients Previously Treated with Other Therapies for Spinal Muscular Atrophy: An Interim Analysis from the JEWELFISH Study.** *Neurology and therapy*  
Chiriboga, C. A., Bruno, C., Duong, T., Fischer, D., Mercuri, E., Kirschner, J., Kostera-Pruszczyk, A., Jaber, B., Gorni, K., Kletzl, H., Carruthers, I., Martin, C., Warren, et al  
2023
- **Assessing Bulbar Function in Spinal Muscular Atrophy Using Patient-Reported Outcomes.** *Journal of neuromuscular diseases*  
Young, S. D., Pasternak, A., Duong, T., McGrattan, K. E., Stranberg, S., Maczek, E., Dias, C., Tang, W., Parker, D., Levine, A., Rohan, A., Wolford, C., Martens, et al  
2023
- **Safety and efficacy of leriglitazone for preventing disease progression in men with adrenomyeloneuropathy (ADVANCE): a randomised, double-blind, multi-centre, placebo-controlled phase 2-3 trial.** *The Lancet. Neurology*  
Kohler, W., Engelen, M., Eichler, F., Lachmann, R., Fatemi, A., Sampson, J., Salsano, E., Gamez, J., Molnar, M. J., Pascual, S., Rovira, M., Vila, A., Pina, et al  
2023; 22 (2): 127-136
- **Safety and efficacy of leriglitazone for preventing disease progression in men with adrenomyeloneuropathy (ADVANCE): a randomised, double-blind, multi-centre, placebo-controlled phase 2-3 trial** *LANCET NEUROLOGY*  
Koehler, W., Engelen, M., Eichler, F., Lachmann, R., Fatemi, A., Sampson, J., Salsano, E., Gamez, J., Molnar, M., Pascual, S., Rovira, M., Vila, A., Pina, et al  
2023; 22 (2): 127-136
- **Safety and efficacy of risdiplam in patients with type 1 spinal muscular atrophy (FIREFISH part 2): secondary analyses from an open-label trial.** *The Lancet. Neurology*  
Masson, R., Mazurkiewicz-Beldzinska, M., Rose, K., Servais, L., Xiong, H., Zanoteli, E., Baranello, G., Bruno, C., Day, J. W., Deconinck, N., Klein, A., Mercuri, E., Vlodayets, et al  
2022
- **Motor Responses in Pediatric Pompe Disease in the ADVANCE Participant Cohort.** *Journal of neuromuscular diseases*  
Duong, T., Kishnani, P. S., An Haack, K., Foster, M. C., Gibson, J. B., Wilson, C., Hahn, S. H., Hillman, R., Kronn, D., Leslie, N. D., Peña, L. D., Sparks, S. E., Stockton, et al  
2022
- **Emerging therapies for Duchenne muscular dystrophy** *LANCET NEUROLOGY*  
Markati, T., Oskoui, M., Farrar, M. A., Duong, T., Goemans, N., Servais, L.  
2022; 21 (9): 814-829
- **Emerging therapies for Duchenne muscular dystrophy.** *The Lancet. Neurology*  
Markati, T., Oskoui, M., Farrar, M. A., Duong, T., Goemans, N., Servais, L.  
2022
- **INCEPTUS Natural History, Run-in Study for Gene Replacement Clinical Trial in X-Linked Myotubular Myopathy.** *Journal of neuromuscular diseases*  
Dowling, J. J., Muller-Felber, W., Smith, B. K., Bonnemann, C. G., Kuntz, N. L., Muntoni, F., Servais, L., Alfano, L. N., Beggs, A. H., Bilder, D. A., Blaschek, A., Duong, T., Graham, et al  
2022
- **Genetic modifiers of upper limb function in Duchenne muscular dystrophy.** *Journal of neurology*  
Sabbatini, D., Fusto, A., Vianello, S., Villa, M., Janik, J., D'Angelo, G., Diella, E., Magri, F., Comi, G. P., Panicucci, C., Bruno, C., D'Amico, A., Bertini, et al  
2022
- **Comparison of strength testing modalities in dysferlinopathy.** *Muscle & nerve*  
Reash, N. F., James, M. K., Alfano, L. N., Mayhew, A. G., Jacobs, M., Iammarino, M. A., Holsten, S., Sakamoto, C., Tateishi, T., Yajima, H., Duong, T., de Wolf, B., Gee, et al  
2022

- **Exploring Protein Changes in Cerebrospinal Fluid of Spinal Muscular Atrophy Patients Pre-Nusinersen vs. Post-Nusinersen Treatment using Bayesian Machine Learning Algorithms**  
Kamali, T., Hagerman, K., Duong, T., Parker, D., Young, S., Tang, W., Sampson, J., Day, J.  
LIPPINCOTT WILLIAMS & WILKINS.2022
- **MANATEE: A Study of R07204239 in Combination with Risdiplam Treatment in Pediatric Patients with SMA**  
Duong, T., Darras, B. T., Morrow, J., Muntoni, F., Servais, L., Carruthers, I., Gerber, M., Kletzl, H., Martin, C., Zhang, B. B., Scalco, R. S., Mercuri, E.  
LIPPINCOTT WILLIAMS & WILKINS.2022
- **JEWELFISH: Safety, Pharmacodynamic and Exploratory Efficacy Data in Non -Na ve Patients with Spinal Muscular Atrophy (SMA) Receiving Treatment with Risdiplam**  
Chiriboga, C. A., Bruno, C., Duong, T., Fischer, D., Kirschner, J., Mercuri, E., Gerber, M., Gorni, K., Kletzl, H., Carruthers, I., Martin, C., Warren, F., Scoto, et al  
LIPPINCOTT WILLIAMS & WILKINS.2022
- **Assessing the Relationship of Patient Reported Outcome Measures With Functional Status in Dysferlinopathy: A Rasch Analysis Approach.** *Frontiers in neurology*  
Mayhew, A. G., James, M. K., Moore, U., Sutherland, H., Jacobs, M., Feng, J., Lowes, L. P., Alfano, L. N., Muni Lofra, R., Rufibach, L. E., Rose, K., Duong, T., Bello, et al  
2022; 13: 828525
- **Safety and efficacy of once-daily risdiplam in type 2 and non-ambulant type 3 spinal muscular atrophy (SUNFISH part 2): a phase 3, double-blind, randomised, placebo-controlled trial.** *The Lancet. Neurology*  
Mercuri, E., Deconinck, N., Mazzone, E. S., Nascimento, A., Oskoui, M., Saito, K., Vuillerot, C., Baranello, G., Boespflug-Tanguy, O., Goemans, N., Kirschner, J., Kostera-Pruszczyk, A., Servais, et al  
1800; 21 (1): 42-52
- **Safety and efficacy of once-daily risdiplam in type 2 and non-ambulant type 3 spinal muscular atrophy (SUNFISH part 2) : a phase 3, double-blind, randomised, placebo-controlled trial** *LANCET NEUROLOGY*  
Mercuri, E., Deconinck, N., Mazzone, E. S., Nascimento, A., Oskoui, M., Saito, K., Vuillerot, C., Baranello, G., Boespflug-Tanguy, O., Goemans, N., Kirschner, J., Kostera-Pruszczyk, A., Servais, et al  
2022; 21 (1): 42-52
- **Revised upper limb module in type II and III spinal muscular atrophy: 24-month changes.** *Neuromuscular disorders : NMD*  
Coratti, G., Carmela Pera, M., Montes, J., Scoto, M., Pasternak, A., Bovis, F., Sframeli, M., D'Amico, A., Pane, M., Albamonte, E., Antonaci, L., Lia Frongia, A., Mizzoni, et al  
1800
- **Correction to: Reldesemtiv in Patients with Spinal Muscular Atrophy: a Phase 2 Hypothesis-Generating Study.** *Neurotherapeutics : the journal of the American Society for Experimental NeuroTherapeutics*  
Rudnicki, S. A., Andrews, J. A., Duong, T., Cockroft, B. M., Malik, F. I., Meng, L., Wei, J., Wolff, A. A., Genge, A., Johnson, N. E., Tesi-Rocha, C., Connolly, A. M., Darras, et al  
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- **Different trajectories in upper limb and gross motor function in spinal muscular atrophy.** *Muscle & nerve*  
Coratti, G., Pera, M. C., Montes, J., Pasternak, A., Scoto, M., Baranello, G., Messina, S., Dunaway Young, S., Glanzman, A. M., Duong, T., de Sanctis, R., Mazzone, E. S., Milev, et al  
2021
- **Risdiplam-Treated Infants with Type 1 Spinal Muscular Atrophy versus Historical Controls** *NEW ENGLAND JOURNAL OF MEDICINE*  
Darras, B. T., Masson, R., Mazurkiewicz-Beldzinska, M., Rose, K., Xiong, H., Zanoteli, E., Baranello, G., Bruno, C., Vlodayets, D., Wang, Y., El-Khairi, M., Gerber, M., Gorni, et al  
2021; 385 (5): 427-435
- **Advances in the therapy of Spinal Muscular Atrophy.** *The Journal of pediatrics*  
Klotz, J., Rocha, C. T., Young, S. D., Duong, T., Buu, M., Sampson, J., Day, J. W.  
2021
- **Nusinersen in pediatric and adult patients with type III spinal muscular atrophy.** *Annals of clinical and translational neurology*  
Pera, M. C., Coratti, G., Bovis, F., Pane, M., Pasternak, A., Montes, J., Sansone, V. A., Dunaway Young, S., Duong, T., Messina, S., Mizzoni, I., D'Amico, A., Civitello, et al  
2021

- **Konectom™ Smartphone-based Digital Outcome Assessments for Adults Living with Spinal Muscular Atrophy (SMA): A Conceptual Framework**  
Arteaga-Bracho, E. E., Dai, Y., Drory, V., Duong, T., Kleinschnitz, C., Rodrigue, X., Sacconi, S., Sansone, V. A., Valente, M., Guymard, T., Belachew, S., Guo, C.  
LIPPINCOTT WILLIAMS & WILKINS.2021
- **JEWELFISH: Safety and Pharmacodynamic Data in Non-Naive Patients with Spinal Muscular Atrophy (SMA) Receiving Treatment with Risdiplam**  
Chiriboga, C. A., Bruno, C., Duong, T., Fischer, D., Kirschner, J., Mercuri, E., Gerber, M., Gorni, K., Kletzl, H., McIver, T., Scalco, R. S., Warren, F., Scoto, et al  
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- **Age related treatment effect in type II Spinal Muscular Atrophy pediatric patients treated with nusinersen.** *Neuromuscular disorders : NMD*  
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- **Gaussian Process Regression for COP Trajectory Estimation in Healthy and Pathological Gait Using Instrumented Insoles**  
Duong, T. H., Uher, D., Young, S., Duong, T., Sangco, M., Cornett, K., Montes, J., Zanotto, D., IEEE  
IEEE.2021: 9548-9553
- **Reldesemtiv in Patients with Spinal Muscular Atrophy: a Phase 2 Hypothesis-Generating Study.** *Neurotherapeutics : the journal of the American Society for Experimental NeuroTherapeutics*  
Rudnicki, S. A., Andrews, J. A., Duong, T. n., Cockroft, B. M., Malik, F. I., Meng, L. n., Wei, J. n., Wolff, A. A., Genge, A. n., Johnson, N. E., Tesi-Rocha, C. n., Connolly, A. M., Darras, et al  
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- **Risdiplam in Type 1 Spinal Muscular Atrophy.** *The New England journal of medicine*  
Baranello, G. n., Darras, B. T., Day, J. W., Deconinck, N. n., Klein, A. n., Masson, R. n., Mercuri, E. n., Rose, K. n., El-Khairi, M. n., Gerber, M. n., Gorni, K. n., Khwaja, O. n., Kletzl, et al  
2021
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Jacobs, M. n., James, M. K., Lowes, L. P., Alfano, L. N., Eagle, M. n., Lofra, R. M., Moore, U. n., Feng, J. n., Rufibach, L. E., Rose, K. n., Duong, T. n., Bello, L. n., Pedrosa-Hernández, et al  
2021
- **Improving Care and Empowering Adults Living with SMA: A Call to Action in the New Treatment Era** *JOURNAL OF NEUROMUSCULAR DISEASES*  
Walter, M. C., Chiriboga, C., Duong, T., Goemans, N., Mayhew, A., Ouillade, L., Oskoui, M., Quinlivan, R., Vazquez-Costa, J. F., Vissing, J., Servais, L.  
2021; 8 (4): 543-551
- **E-Health & Innovation to Overcome Barriers in Neuromuscular Diseases. Report from the 1st eNMD Congress: Nice, France, March 22-23, 2019** *JOURNAL OF NEUROMUSCULAR DISEASES*  
Pini, J., Siciliano, G., Lahaut, P., Braun, S., Segovia-Kueny, S., Kole, A., Hernando, I., Selb, J., Schirinzi, E., Duong, T., Hogrel, J., Serrano Olmedo, J., Vissing, et al  
2021; 8 (4): 743-754
- **Clinical variability in spinal muscular atrophy type III.** *Annals of neurology*  
Coratti, G., Messina, S., Lucibello, S., Pera, M. C., Montes, J., Pasternak, A., Stat, F. B., Escudero, J. E., Mazzone, E. S., Mayhew, A., Glanzman, A. M., Young, S. D., Salazar, et al  
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- **Gain and loss of abilities in type II SMA: A 12-month natural history study.** *Neuromuscular disorders : NMD*  
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- **Determining An Appropriate Cardiopulmonary Exercise Testing Protocol For Individuals With Neuromuscular Disease**  
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