



## Tina Duong MPT, PhD

Senior Research Scientist, Adult Neurology

### Bio

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#### BIO

I am a senior research scientist and physical therapist with over 20 years of experience, specializing in neuromuscular clinical research. Currently, I am the Director of clinical outcomes and research developments within the Neuromuscular division and Co-Director, Stanford Innovative Genetic Neurologic Investigation and Treatment Evaluation Center. My doctoral studies focused on understanding the impact of contracture development on subsequent musculoskeletal and biomechanical changes that influence overall functional outcomes. I am deeply committed to advancing my work by creating innovative outcome measures that bridge the gap with traditional metrics for a range of neuromuscular disorders. Furthermore, I am enthusiastic about investigating the therapeutic advantages of incorporating rehabilitation and exercise as complementary treatments for managing neuromuscular conditions.

In my professional pursuits, I prioritize integrity, work ethic, humility, empathy, leadership, initiative, and drive in all my collaborations. Inspired by Ralph Waldo Emerson's poem "to know even one life has breathed easier because you have lived. This is to have succeeded", I strive to contribute meaningfully in both my personal and professional endeavors. While it can be challenging to articulate one's accomplishments and impact, I aspire to uphold the values and principles that guide me, serving as a compass in navigating both my personal and professional path. I find it difficult to summarize my 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=IwAR06tAcgAKivWBXBXFq0sFEbo-VicRNJDRp1Me\\_9lgomqJ8S3sRUOS1hvDc](https://www.myotonic.org/sites/default/files/pages/files/MDF_RoleofPhysicalTherapy_1_21.pdf?fbclid=IwAR06tAcgAKivWBXBXFq0sFEbo-VicRNJDRp1Me_9lgomqJ8S3sRUOS1hvDc)

## Publications

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### PUBLICATIONS

- **Heart Transplantation and Ventricular Assist Device in Duchenne Muscular Dystrophy: A New Era.** *Pediatric transplantation*  
Rosenthal, D. N., Amodeo, A., Butterfield, R. J., Butts, R., Chrzanowski, S., Cripe, L., Day, J., Davies, R., Duong, T., Evers, P., Gambetta, K., Harris, R., Hayes, et al  
2026; 30 (1): e70253
- **New SMA era: A broad-range tiered assessment of function for the evolving SMA phenotype (EVOLVE-SMA).** *Journal of neuromuscular diseases*  
Moore Burk, M., Crawford, T. O., Johnson, K., Apkon, S., Brown, L., Carry, T., Hoffman, K., Kelley, C., Kuntz, N. L., Miller, J., Nance, J., Patel, R., Rao, et al  
2025: 22143602251405346
- **Cerebrospinal fluid proteomic profiling reveals potential biomarkers and altered pathways in myotonic dystrophy type 1** *FRONTIERS IN NEUROSCIENCE*  
Zafarullah, M., Kamali, T., Hagerman, K. A., Ghiglieri, L., Duong, T., Wang, E., Sampson, J. B., Day, J. W.  
2025; 19: 1709678
- **Differences in swallowing efficacy of disease modifying treatment between infants receiving pre-symptomatic and symptomatic administration.** *Orphanet journal of rare diseases*  
McGrattan, K. E., Mohr, A. H., Miles, A., Allen, J., Ochura, J., Hernandez, K., Walsh, K., Rao, V., Stevens, M., McGhee, H., Nichols, K., Turksi, M. E., Spoden, et al  
2025; 20 (1): 526
- **Video-Based Biomechanical Analysis Captures Disease-Specific Movement Signatures of Different Neuromuscular Diseases.** *NEJM AI*  
Ruth, P. S., Uhlrich, S. D., de Monts, C., Falisse, A., Muccini, J., Covitz, S., Vogt-Domke, S., Day, J., Duong, T., Delp, S. L.  
2025; 2 (9)
- **Rehabilitation research in spinal muscular atrophy: a call to action** *JOURNAL OF NEUROMUSCULAR DISEASES*  
Lilien, C., Nelson, L., Edel, L., Forrest, D., Estilow, T., McGrattan, K. E., Duong, T., Coratti, G.  
2025
- **Consensus recommendations and considerations for the delivery and monitoring of gene therapy in patients with Duchenne muscular dystrophy.** *Neuromuscular disorders : NMD*  
Wolff, J. M., Capocci, N., Atas, E., Bharucha-Goebel, D. X., Brandsema, J. F., Butterfield, R. J., Chadwick, C. B., Corti, M., Crawford, T. O., Cripe, L., Day, J. W., Duong, T., ElMallah, et al  
2025; 54: 106208
- **Video-based biomechanical analysis captures disease-specific movement signatures of different neuromuscular diseases.** *bioRxiv : the preprint server for biology*  
Ruth, P. S., Uhlrich, S. D., de Monts, C., Falisse, A., Muccini, J., Covitz, S., Vogt-Domke, S., Day, J., Duong, T., Delp, S. L.  
2025
- **Spinal Muscular Atrophy Functional Composite Score Revised (SMA-FCR) in Untreated and Nusinersen-Treated Patient Cohorts.** *Neurology*  
Pasternak, A., McDermott, M. P., Montes, J., Glanzman, A. M., Coratti, G., Dunaway Young, S., Duong, T. T., Martens, W. B., Day, J. W., Zolkipli-Cunningham, Z., Sansone, V. A., D'Amico, A., Messina, et al  
2025; 105 (2): e213839
- **Correction: Serial casting for contractures in SMA: consensus derived guidelines for treatment.** *Frontiers in neurology*

- Brown, L., Hoffman, K., Corbo-Galli, C., Kelley, C., Carry, T., Civitello, M., Coratti, G., DeSanctis, R., Duong, T., Driscoll, B., Flickinger, J., Glanzman, A. M., Jones, et al  
2025; 16: 1646078
- **Longitudinal Assessment of 4-Year HFMSE Changes in SMA II and III Patients Treated With Nusinersen.** *European journal of neurology*  
Coratti, G., Bovis, F., Pane, M., Pasternak, A., Albamonte, E., Mizzoni, I., Glanzman, A. M., Morando, S., Montes, J., Cavallina, I., Dunaway Young, S., Duong, T., Rolle, et al  
2025; 32 (7): e70268
  - **Cardiopulmonary Recovery After Maximal Exercise in Individuals with Neuromuscular Disease and Limited Mobility.** *Journal of clinical medicine*  
Blumberg, Y., de Monts, C., Montalvo, S., Tang, W. J., Dunaway Young, S., Hageman, N., Sanchis-Gomar, F., Ashley, E. A., Amar, D., Myers, J., Wheeler, M. T., Day, J. W., Duong, et al  
2025; 14 (12)
  - **A real-world analysis of the impact of X-linked myotubular myopathy on caregivers in the United States.** *Orphanet journal of rare diseases*  
Duong, T., Haselkorn, T., Miller, B., Coats, J., Jensen, I., Ward, E., Wood, M., Graham, R. J., Servais, L.  
2025; 20 (1): 224
  - **Serial casting for contractures in SMA: consensus derived guidelines for treatment.** *Frontiers in neurology*  
Brown, L., Hoffman, K., Corbo-Galli, C., Kelley, C., Carry, T., Civitello, M., Coratti, G., DeSanctis, R., Duong, T., Driscoll, B., Flickinger, J., Glanzman, A. M., Jones, et al  
2025; 16: 1502495
  - **Cardiopulmonary exercise testing as an integrative approach to explore physiological limitations in Duchenne muscular dystrophy.** *Journal of neuromuscular diseases*  
Bomma, M., Lott, D., Forbes, S., Shih, R., Coppola, J. A., Christle, J. W., Duong, T., Russo, J., Pant, A., Leon-Astudillo, C., Berthy, J., Cousins, C., Corti, et al  
2025: 22143602251319170
  - **First-in-human study of epidural spinal cord stimulation in individuals with spinal muscular atrophy.** *Nature medicine*  
Prat-Ortega, G., Ensel, S., Donadio, S., Borda, L., Boos, A., Yadav, P., Verma, N., Ho, J., Carranza, E., Frazier-Kim, S., Fields, D. P., Fisher, L. E., Weber, et al  
2025
  - **Characterization of swallowing biomechanics and function in untreated infants with spinal muscular atrophy: A natural history dataset.** *Journal of neuromuscular diseases*  
McGrattan, K. E., Graham, R. J., Mohr, A. H., Miles, A., Allen, J., Ochura, J., Hernandez, K., Walsh, K., Rao, V., Stevens, M., Alfano, L., Coker, M., Leon-Astudillo, et al  
2025; 12 (1): 22143602241308762
  - **Gene therapy for children with X-linked myotubular myopathy: a plain language summary of publication for the ASPIRO study.** *Therapeutic advances in rare disease*  
Shieh, P. B., Hughes, W., Wood, M., Beggs, A. H., Lawlor, M. W., Coats, J., Varfaj, F., Graham, R. J., Kuntz, N. L., Dowling, J. J., Muller-Felber, W., Bonnemann, C. G., Buj Bello, et al  
2025; 6: 26330040251362885
  - **Long-term natural history in type II and III spinal muscular atrophy: a 4-year international study on the Hammersmith Functional Motor Scale Expanded.** *European journal of neurology*  
Coratti, G., Bovis, F., Pera, M. C., Civitello, M., Rohwer, A., Salmin, F., Glanzman, A. M., Montes, J., Pasternak, A., De Sanctis, R., Dunaway Young, S., Duong, T., Mizzoni, et al  
2024: e16517
  - **Exercise And Recovery Ventilatory Responses Between Individuals With Neuromuscular Disease And Healthy Adults**  
Montalvo, S., Blumberg, Y., de Monts, C., Tang, W. J., Ataide, P., Young, S., Ghiollagain, N., Parker, D. M., Day, J. W., Myers, J. N., Wheeler, M. T., Duong, T., Christle, et al  
LIPPINCOTT WILLIAMS & WILKINS.2024: 925-926
  - **Investigating Recovery From Maximal Exercise In Patients With Neuromuscular Disease**  
Blumberg, Y., Patti, A., De Monts, C., Montalvo, S., Ataide, P., Tang, W., Young, S., Myers, J., Wheeler, M. T., Duong, T., Christle, J. W., Day, J. W.  
LIPPINCOTT WILLIAMS & WILKINS.2024: 657

- **Upper limb function changes over 12 months in untreated SMA II and III individuals: an item-level analysis using the Revised Upper Limb Module.** *Neuromuscular disorders : NMD*  
Coratti, G., Civitello, M., Rohwer, A., Albamonte, E., Montes, J., Glanzman, A. M., Pasternak, A., De Sanctis, R., Young, S. D., Duong, T., Mizzone, I., Milev, E., Sframeli, et al  
2024: 104449
- **Performance of upper limb entry item to predict forced vital capacity in dysferlin-deficient limb girdle muscular dystrophy.** *Neuromuscular disorders : NMD*  
Borland, H., Moore, U., Dressman, H. G., Human, A., Mayhew, A. G., Hilsden, H., Rufibach, L. E., Duong, T., Maron, E., DeWolf, B., Rose, K., Siener, C., Thiele, et al  
2024; 43: 20-28
- **Patients' Perceptions of Nusinersen Effects According to Their Responder Status.** *Journal of clinical medicine*  
Lilien, C., Vrscaj, E., Thapaliya, G., Deconinck, N., De Waele, L., Duong, T., Haberlova, J., Kumhera, M., Peirens, G., Szabo, L., Tahon, V., Tang, W. J., Benmhammed, et al  
2024; 13 (12)
- **JEWELFISH: 24-month results from an open-label study in non-treatment-naïve patients with SMA receiving treatment with risdiplam.** *Journal of neurology*  
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., Scalco, et al  
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- **Changes in abilities over the initial 12 months of nusinersen treatment for type II SMA.** *Neuromuscular disorders : NMD*  
Coratti, G., Civitello, M., Rohwer, A., Salmin, F., Glanzman, A. M., Montes, J., Pasternak, A., De Sanctis, R., Young, S. D., Duong, T., Mizzone, I., Milev, E., Sframeli, et al  
2024; 41: 42-50
- **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., Larrobino, 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
- **Long-term efficacy, safety, and patient-reported outcomes of apitegromab in patients with spinal muscular atrophy: results from the 36-month TOPAZ study.** *Frontiers in neurology*  
Crawford, T. O., Day, J. W., De Vivo, D. C., Krueger, J. M., Mercuri, E., Nascimento, A., Pasternak, A., Mazzone, E. S., Duong, T., Song, G., Marantz, J. L., Baver, S., Yu, et al  
2024; 15: 1419791
- **Towards Video-Based Movement Biomarkers for Neuromuscular Diseases**  
Uhlrich, S. D., Ruth, P. S., de Monts, C., Falisse, A., Muccini, J., Ataide, P., Day, J., Duong, T., Delp, S. L.  
edited by Pons, J. L., Tornero, J., Akay, M.  
SPRINGER INTERNATIONAL PUBLISHING AG.2024: 501-504
- **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., Christie, 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  
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- **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  
2023; 22 (3): 218-228
- **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  
2023; 22 (3): 218-228
- **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  
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- **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  
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- **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.  
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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  
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- **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.  
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- **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  
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- **Assessing the Relationship of Patient Reported Outcome Measures With Functional Status in Dysferlinopathy: A Rasch Analysis Approach.** *Frontiers in neurology*  
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