

**FORMATO EUROPEO
PER IL CURRICULUM
VITAE**



INFORMAZIONI PERSONALI

Nome

Indirizzo

Telefono

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Nazionalità

Data di nascita

TITOLI DI STUDIO

• Date (da – a)

• Nome e indirizzo del datore di lavoro

• Tipo di azienda o settore

• Tipo di impiego

• Date (da – a)

• Nome e indirizzo del datore di lavoro

• Tipo di azienda o settore

• Tipo di impiego

• Principali mansioni e responsabilità

• Date (da – a)

• Nome e indirizzo del datore di lavoro

MAZZONE ELENA STACY

**LAURE MAGISTRALE in Scienze Riabilitative delle Professioni Sanitarie
LAUREA IN FISIOTERAPIA**

Dal 11/2020

Fondazione Policlinico Gemelli IRCCS

Largo Gemelli 8, 00168 Roma

Neuropsichiatria infantile, Centro Nemo

Dottoranda in Neuroscienze

Dal 1/2007 – 3/2019

Policlinico Gemelli

Largo Gemelli 8, 00168 Roma

Neuropsichiatria infantile, Ambulatorio di malattie neuromuscolari

Fisioterapista

Valutazione e trattamento pazienti ambulatoriali.

Valutazione pazienti inseriti in trial terapeutici

Dal 2014 - Oggi

- **TREAT-NMD** Network (Translational Research in Europe for the Assessment and Treatment of Neuromuscular Disease): Coordination and training of Italian and European physiotherapists, classification and definition of outcome measures to be used in clinical routine and trials in neuromuscular disorders.
- **BIOGEN**: Clinical trial in SMA: CS11, Nurture: Master Physiotherapist trainer. Advisory board
Nusinersen: Trainer for expanded access global training program
- **CYTOKINETICS**: Master physiotherapist trainer for Revised Upper Limb module
- **ROCHE**: Clinical evaluator trainer for RO6885247 infantile onset SMA study.
- Protocol endpoint consulting for BN39269.
- Master physiotherapist trainer in all Risdipam clinical trials
- **AVEXIS**:101-CL-303; 101-CL-102: Master trainer
- **NOVARTIS**: Clinical trial in SMA: Advisory board and Clinical evaluator trainer
- **SCHOLAR ROCK**: Antimyostatin trials in SMA: advisory board and master trainer

• Tipo di azienda o settore

Aziende farmaceutiche

• Tipo di impiego

Fisioterapista trainer per trial clinici

• Principali mansioni e responsabilità

- Formazione dei terapisti inseriti in trial terapeutici su Atrofia Muscolare Spinale
- Stesura protocollo di valutazione e scelta delle outcome measures
- Analisi risultati in itinere

• Date (da – a)

Dal 2009 - 2016

• Nome e indirizzo del datore di lavoro

- HALO-DMD-01 Master Physiotherapist trainer
- PROSENSA/BIOMARIN 045-053 Clinical trial in DMD: Master Physiotherapist trainer and clinical evaluator
- PROSENSA/BIOMARIN natural history study in DMD: Clinical evaluator
- SAREPTA: Clinical evaluator SRP-4053-101
- Italfarmaco-GIVINOSTAT clinical trial in DMD: Master Physiotherapist trainer and clinical evaluator
- ISOFEN therapeutical approach Advisory board
- Telethon Trainer for Project GUP07009: Outcome measures in DMD
- PTC Study 020: Master Physiotherapist trainer and clinical evaluator
- IONIS-396443-CS2-CS3b-CS4 Clinical trial in SMA: Master Physiotherapist trainer

• Tipo di azienda o settore

Aziende farmaceutiche

• Tipo di impiego

Fisioterapista trainer per trial clinici

• Principali mansioni e responsabilità

- Formazione dei terapisti inseriti in trial terapeutici su Distrofie Muscolari ed Atrofia Muscolare Spinale
- Stesura protocollo di valutazione e scelta delle outcome measures
- Analisi risultati in itinere

- Date (da – a) **Dal 4/2006-3/2011**
- Nome e indirizzo del datore di lavoro **UILDM-Unione italiana lotta distrofia muscolare**
Via Prospero Santacroce 5 , 00168 Roma
- Tipo di azienda o settore Centro di Riabilitazione
 - Tipo di impiego Valutazione e trattamento pazienti ambulatoriali
 - Principali mansioni e responsabilità Fisioterapista
- Date (da – a) **1/2003 – 1/2006**
- Nome e indirizzo del datore di lavoro **Casa di cura Villa Nigrisoli**
Viale Ercolani 17, 40100 Bologna
- Tipo di azienda o settore Centro di Riabilitazione
 - Tipo di impiego Fisioterapista
 - Valutazione e trattamento pazienti ambulatoriali
 - **Examining Researcher progetto Telethon GUP030530:** "Validation of outcome measures for SMA using the Hammersmith functional motor scale and abductor digiti minimi CMAP amplitude". Valutazione pazienti tramite metodiche standardizzate.
- Date (da – a) **08/2004-12/2004**
- Nome e indirizzo del datore di lavoro **AIFO-Associazione italiana amici di Roul Follereau**
Via Borselli 4/6 - 40135 Bologna
- Tipo di azienda o settore Organizzazione non governativa
 - Tipo di impiego **Progetto Nampula, Mozambico - Riabilitazione su base comunitaria**
 - Principali mansioni e responsabilità Fisioterapista responsabile dell'implementazione delle attività di prevenzione e riabilitazione delle disabilità nella lebbra.
- Date (da – a) **05/2003-07/2003**
- Nome e indirizzo del datore di lavoro **AIFO-Associazione italiana amici di Roul Follereau**
Via Borselli 4/6 - 40135 Bologna
- Tipo di azienda o settore Organizzazione non governativa
 - Tipo di impiego Progetto Georgetown, Guyana - Riabilitazione su base comunitaria
 - Principali mansioni e responsabilità Fisioterapista responsabile Corso di formazione sulla riabilitazione ortopedica, promosso dall'associazione AIFO in collaborazione con il Ministero della Sanità della Guyana, con l'obiettivo di rafforzare i Servizi di Riabilitazione.
- Date (da – a) **02/1999-01/2003**
- Nome e indirizzo del datore di lavoro **Istituti Ortopedici Rizzoli**
Via G. C. Pupilli- 40100 Bologna

- Tipo di azienda o settore
• Tipo di impiego
 - Principali mansioni e responsabilità
- Ospedale
Ambulatorio Malattie Neuromuscolari
- Fisioterapista

ALLEGATI

PUBBLICAZIONI ALLEGATO A

Il/La Sottoscritto/a, ai sensi degli artt. 46 e 47 D.P.R. n. 445/2000, consapevole delle sanzioni penali previste dall'art. 76 D.P.R. n. 445/2000 nel caso di mendaci dichiarazioni, falsità negli atti, uso o esibizione di atti falsi o contenenti dati non più corrispondenti a verità, dichiara che quanto sopra riportato corrisponde a verità. Dichiaro inoltre che i titoli e gli allegati sono, su richiesta, disponibili in copia fotostatica conforme agli originali.

Autorizzo il trattamento dei miei dati personali ai sensi del Dlgs 196 del 30 giugno 2003"

19-12-2022

Appendix A

Risdiplam in types 2 and 3 spinal muscular atrophy: a randomised, placebo-controlled, dose-finding trial followed by 24 months of treatment.

Mercuri E, Baranello G, Boespflug-Tanguy O, De Waele L, Goemans N, Kirschner J, Masson R, Mazzone ES, Pechmann A, Pera MC, Vuillerot C, Bader-Weder S, Gerber M, Gorni K, Hoffart J, Kletzl H, Martin C, Mciver T, Scalco RS, Yeung WY, Servais L; Sunfish Working Group.

Eur j neurol. 2022 Jul 15. Doi: 10.1111/ene.15499. Online ahead of print.

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.

Mercuri E, Deconinck N, Mazzone ES, Nascimento A, Oskoui M, Saito K, Vuillerot C, Baranello G, Boespflug-Tanguy O, Goemans N, Kirschner J, Kostera-Pruszczyk A, Servais L, Gerber M, Gorni K, Khwaja O, Kletzl H, Scalco RS, Staunton H, Yeung WY, Martin C, Fontoura P, Day JW; SUNFISH Study Group.

Lancet Neurol. 2022 Jan;21(1):42-52. doi: 10.1016/S1474-4422(21)00367-7.

New advances in the treatment of Duchenne muscular dystrophy and spinal muscular atrophy
EMJ-Neurology-10-Supplement-1-2022

Management of motor rehabilitation in individuals with muscular dystrophies. 1st Consensus Conference report from UILDM - Italian Muscular Dystrophy Association (Rome, January 25-26, 2019)
Maria Elena Lombardo, Elena Carraro, Cristina Sancricca, Michela Armando, Michela Catteruccia, Elena Mazzone

ACTA MYOLOGICA 2021; XL: p. 72-87

Consensus Guidelines for Improving Quality of Assessment and Training for Neuromuscular Diseases
Tina Duong, Kristin J. Krosschell, Meredith K. James, Leslie Nelson, Lindsay N. Alfano, Katy Eichinger, Elena Mazzone, Kristy Rose, Linda P. Lowes, Anna Mayhew, Julaine Florence, Wendy King, Claudia R. Senesac and Michelle Eagle

Front. Genet., 10 November 2021

Different trajectories in upper limb and gross motor function in spinal muscular atrophy.

Coratti G, Pera MC, Montes J, Pasternak A, Scoto M, Baranello G, Messina S, Dunaway Young S, Glanzman AM, Duong T, De Sanctis R, Mazzone ES, Milev E, Rohwer A, Civitello M, Pane M, Antonaci L, Frongia AL, Sframeli M, Vita GL, D'Amico A, Mizzoni I, Albamonte E, Darras BT, Bertini E, Sansone VA, Bovis F, Day J, Bruno C, Muntoni F, De Vivo DC, Finkel R, Mercuri E.

Muscle Nerve. 2021 Nov;64(5):552-559

Management of motor rehabilitation in individuals with muscular dystrophies. 1st Consensus Conference report from UILDM - Italian Muscular Dystrophy Association (Rome, January 25-26, 2019).
Lombardo ME, Carraro E, Sancricca C, Armando M, Catteruccia M, Mazzone E, Ricci G, Salamino F, Santorelli FM, Filosto M; UILDM (Italian Muscular Dystrophy Association) and Italian Consensus Conference Group on motor rehabilitation in muscular dystrophy.

Acta Myol. 2021 Jun 30;40(2):72-87.

North Star Ambulatory Assessment changes in ambulant Duchenne boys amenable to skip exons 44, 45, 51, and 53: A 3 year follow up.

Coratti G, Pane M, Brogna C, Ricotti V, Messina S, D'Amico A, Bruno C, Vita G, Berardinelli A, Mazzone E, Magri F, Ricci F, Mongini T, Battini R, Bello L, Pegoraro E, Baranello G, Previtali SC, Politano L, Comi GP, Sansone VA, Donati A, Hogrel JY, Straub V, De Lucia S, Nix E, Servais L, De Groot I, Chesshyre M, Bertini E, Goemans N, Muntoni F, Mercuri E; on behalf on the International DMD Group and the iMDEX Consortium.

PLoS One. 2021 Jun 25;16(6):e0253882

Clinical Variability in Spinal Muscular Atrophy Type III

Giorgia Coratti, Sonia Messina, Simona Lucibello, Maria Carmela Pera, Jacqueline Montes, Amy Pasternak, Francesca Bovis, Jessica Exposito Escudero, Elena Stacy Mazzone, Anna Mayhew, et al
Ann Neurol. 2020 Dec;88(6):1109-1117.

Performance of Upper Limb module for Duchenne muscular dystrophy.

Mayhew AG, Coratti G, Mazzone ES, Klingels K, James M, Pane M, Straub V, Goemans N, Mercuri E; Pul Working Group. Dev Med Child Neurol. 2019 Sep 19. doi: 10.1111/dmcn.14361. [Epub ahead of print]

Nusinersen improves walking distance and reduces fatigue in later-onset spinal muscular atrophy.

Montes J, Dunaway Young S, Mazzone ES, Pasternak A, Glanzman AM, Finkel RS, Darras BT, Muntoni F, Mercuri E, De Vivo DC, Bishop KM, Schneider E, Bennett CF, Foster R, Farwell W; CS2 and CS12 Study Groups.

Muscle Nerve. 2019 Oct;60(4):409-414. doi: 10.1002/mus.26633. Epub 2019 Jul 27

Long-term progression in type II spinal muscular atrophy: A retrospective observational study.

Mercuri E, Lucibello S, Pera MC, Carnicella S, Coratti G, de Sanctis R, Messina S, Mazzone E, Forcina N, Fanelli L, Norcia G, Antonaci L, Frongia AL, Pane M.

Neurology. 2019 Sep 24;93(13):e1241-e1247.

Long-term natural history data in Duchenne muscular dystrophy ambulant patients with mutations amenable to skip exons 44, 45, 51 and 53.

Brogna C, Coratti G, Pane M, Ricotti V, Messina S, D'Amico A, Bruno C, Vita G, Berardinelli A, Mazzone E, Magri F, Ricci F, Mongini T, Battini R, Bello L, Pegoraro E, Baranello G, Previtali SC, Politano L, Comi GP, Sansone VA, Donati A, Bertini E, Muntoni F, Goemans N, Mercuri E; on behalf on the International DMD group.

PLoS One. 2019 Jun 25;14(6):e0218683. doi: 10.1371/journal.pone.0218683. eCollection 2019.

Erratum in: PLoS One. 2019 Jul 31;14(7):e0220714.

"Be an ambassador for change that you would like to see": a call to action to all stakeholders for co-creation in healthcare and medical research to improve quality of life of people with a neuromuscular disease.

Ambrosini A, Quinlivan R, Sansone VA, Meijer I, Schrijvers G, Tibben A, Padberg G, de Wit M, Sterrenburg E, Mejat A, Breukel A, Rataj M, Lochmüller H, Willmann R; 235th ENMC workshop study group.

Orphanet J Rare Dis. 2019 Jun 7;14(1):126. doi: 10.1186/s13023-019-1103-8

The Position of Neuromuscular Patients in Shared Decision Making. Report from the 235th ENMC Workshop: Milan, Italy, January 19-20, 2018

Lochmüller, Hanns; * | Ambrosini, Anna | van Engelen, Baziël | Hansson, Mats | Tibben, Aad | Breukel, Alexandra | Sterrenburg, Ellen | Schrijvers, Guus | Meijer, Ingeborg | Padberg, George | Peay, Holly | Monaco, Lucia | Snape, Mike | Lennox, Anne | Mazzone, Elena | Bere, Nathalie | de Lemus, Mencia | Landfeldt, Erik | Willmann, Raffaella | on behalf of the 235th ENMC workshop study group

Journal of Neuromuscular Diseases 6 (2019) 161-172

Revised Upper Limb Module for Spinal Muscular Atrophy: 12 month changes.

Pera MC, Coratti G, Mazzone ES, Montes J, Scoto M, De Sanctis R, Main M, Mayhew A, Muni Lofra R, Dunaway Young S, Glanzman AM, Duong T, Pasternak A, Ramsey D, Darras B, Day JW, Finkel RS, De Vivo DC, Sormani MP, Bovis F, Straub V, Muntoni F, Pane M, Mercuri E; iSMAC Consortium Group.

Muscle Nerve. 2019 Jan 24. doi: 10.1002/mus.26419. [Epub ahead of print]

Quantitative Evaluation of Lower Extremity Joint Contractures in Spinal Muscular Atrophy: Implications for Motor Function.

Salazar R, Montes J, Dunaway Young S, McDermott MP, Martens W, Mazzone ES, Main M, Mayhew A, Ramsey D, Muni Lofra R, Coratti G, Fanelli L, De Sanctis R, Mercuri E, De Vivo DC.

Pediatr Phys Ther. 2018 Jul;30(3):209-215

Evaluator Training and Reliability for SMA Global Nusinersen Trials1.

Glanzman AM, Mazzone ES, Young SD, Gee R, Rose K, Mayhew A, Nelson L, Yun C, Alexander K, Darras BT, Zolkipli-Cunningham Z, Tennekoon G, Day JW, Finkel RS, Mercuri E, De Vivo DC, Baldwin R, Bishop KM, Montes J.

J Neuromuscul Dis. 2018;5(2):159-166

Ambulatory function in spinal muscular atrophy: Age-related patterns of progression.

Montes J, McDermott M, Mirek E, Mazzone ES, Main M, Glanzman AM, Duong T, Young SD, Salazar R, De Vivo DC, Mercuri E.

PLoS One. 2018 Jun 26;13(6):e0199657 eCollection 2018

Upper limb function in Duchenne muscular dystrophy: 24 month longitudinal data.

Pane M, Coratti G, Brogna C, Mazzone ES, Mayhew A, Fanelli L, Messina S, D'Amico A, Sormani MP, Mercuri E.

PLoS One. 2018 Jun 20;13(6) eCollection 2018.

Diagnosis and management of spinal muscular atrophy: Part 2: Pulmonary and acute care; medications, supplements and immunizations; other organ systems; and ethics.

Finkel RS, Mercuri E, Meyer OH, Simonds AK, Schroth MK, Graham RJ, Kirschner J, Iannaccone ST, Crawford TO, Woods S, Muntoni F, Wirth B, Montes J, Main M, Mazzone ES, Vitale M, Snyder B, Quijano-Roy S, Bertini E, Davis RH, Qian Y, Sejersen T; SMA Care group.

Neuromuscul Disord. 2018 Mar;28(3):197-207

Diagnosis and management of spinal muscular atrophy: Part 1: Recommendations for diagnosis, rehabilitation, orthopedic and nutritional care.

Mercuri E, Finkel RS, Muntoni F, Wirth B, Montes J, Main M, Mazzone ES, Vitale M, Snyder B, Quijano-Roy S, Bertini E, Davis RH, Meyer OH, Simonds AK, Schroth MK, Graham RJ, Kirschner J, Iannaccone ST, Crawford TO, Woods S, Qian Y, Sejersen T; SMA Care Group.

Neuromuscul Disord. 2018 Feb;28(2):103-115

Nusinersen versus Sham Control in Later-Onset Spinal Muscular Atrophy.

Mercuri E, Darras BT, Chiriboga CA, Day JW, Campbell C, Connolly AM, Iannaccone ST, Kirschner J, Kuntz NL, Saito K, Shieh PB, Tulinius M, Mazzone ES, Montes J, Bishop KM, Yang Q, Foster R, Gheuens S, Bennett CF, Farwell W, Schneider E, De Vivo DC, Finkel RS; CHERISH Study Group.

N Engl J Med. 2018 Feb 15;378(7):625-635

Clinical phenotypes and trajectories of disease progression in type 1 spinal muscular atrophy.

De Sanctis R, Pane M, Coratti G, Palermo C, Leone D, Pera MC, Abiusi E, Fiori S, Forcina N, Fanelli L, Lucibello S, Mazzone ES, Tiziano FD, Mercuri E.

Neuromuscul Disord. 2018 Jan;28(1):24-28

Clinical phenotypes and trajectories of disease progression in type 1 spinal muscular atrophy.

De Sanctis R, Pane M, Coratti G, Palermo C, Leone D, Pera MC, Abiusi E, Fiori S, Forcina N, Fanelli L, Lucibello S, Mazzone ES, Tiziano FD, Mercuri E.

Neuromuscul Disord. 2017 Oct 10. pii: S0960-8966(17)31178-1.

6MWT can identify type 3 SMA patients with neuromuscular junction dysfunction.

Pera MC, Luigetti M, Pane M, Coratti G, Forcina N, Fanelli L, Mazzone ES, Antonaci L, Lapenta L, Palermo C, Ranalli D, Granata G, Lomonaco M, Servidei S, Mercuri E.

Neuromuscul Disord. 2017 Jul 14. pii: S0960-8966(17)30476-5.

Workshop report: First international workshop on rehabilitation management and clinical outcome measures for spinal muscular atrophy

Montes J, Young SD, Mazzone E, Main M; International Spinal Muscular Atrophy Consortium Clinical Evaluator Working Group.

Neuromuscul Disord. 2017 Jul 12. pii: S0960-8966(17)30577-1. [Epub ahead of print] No abstract

available.

Ataluren in patients with nonsense mutation Duchenne muscular dystrophy (ACT DMD): a multicentre, randomised, double-blind, placebo-controlled, phase 3 trial.

McDonald CM, Campbell C, Torricelli RE, Finkel RS, Flanigan KM, Goemans N, Heydemann P, Kaminska A, Kirschner J, Muntoni F, Osorio AN, Schara U, Sejersen T, Shieh PB, Sweeney HL, Topaloglu H, Tulinius M, Vilchez JJ, Voit T, Wong B, Elfring G, Kroger H, Luo X, McIntosh J, Ong T, Riebling P, Souza M, Spiegel RJ, Peltz SW, Mercuri E; Clinical Evaluator Training Group; ACT DMD Study Group. *Lancet*. 2017 Sep 23;390(10101):1489-1498

218th ENMC International Workshop:: Revisiting the consensus on standards of care in SMA

Finkel RS, Sejersen T, Mercuri E; ENMC SMA Workshop Study Group. *Neuromuscul Disord*. 2017 Jun;27(6):596-605

Revised upper limb module for spinal muscular atrophy: Development of a new module.

Mazzone ES, Mayhew A, Montes J, Ramsey D, Fanelli L, Young SD, Salazar R, De Sanctis R, Pasternak A, Glanzman A, Coratti G, Civitello M, Forcina N, Gee R, Duong T, Pane M, Scoto M, Pera MC, Messina S, Tennekoon G, Day JW, Darras BT, De Vivo DC, Finkel R, Muntoni F, Mercuri E. *Muscle Nerve*. 2017 Jun;55(6):869-874.

Content validity and clinical meaningfulness of the HFMSE in spinal muscular atrophy.

Pera MC, Coratti G, Forcina N, Mazzone ES, Scoto M, Montes J, Pasternak A, Mayhew A, Messina S, Sframeli M, Main M, Lofra RM, Duong T, Ramsey D, Dunaway S, Salazar R, Fanelli L, Civitello M, de Sanctis R, Antonaci L, Lapenta L, Lucibello S, Pane M, Day J, Darras BT, De Vivo DC, Muntoni F, Finkel R, Mercuri E. *BMC Neurol*. 2017 Feb 23;17(1):39.

Revised Hammersmith Scale for spinal muscular atrophy: A SMA specific clinical outcome assessment tool.

Ramsey D, Scoto M, Mayhew A, Main M, Mazzone ES, Montes J, de Sanctis R, Dunaway Young S, J, Darras BT, De Vivo D, Finkel R, Mercuri E, Muntoni F. *PLoS One*. 2017 Feb 21;12(2):e0172346.

Developmental milestones in type I spinal muscular atrophy.

De Sanctis R, Coratti G, Pasternak A, Montes J, Pane M, Mazzone ES, Young SD, Salazar R, Quigley J, Pera MC, Antonaci L, Lapenta L, Glanzman AM, Tiziano D, Muntoni F, Darras BT, De Vivo DC, Finkel R, Mercuri E. *Neuromuscul Disord*. 2016 Nov;26(11):754-759

Sleep disorders in spinal muscular atrophy.

Pera MC, Romeo DM, Graziano A, Palermo C, Sivo S, Mazzone ES, Antonaci L, de Sanctis R, Vita GL, Pane M, Mercuri E. *Sleep Med*. 2017 Feb;30:160-163.

Categorizing natural history trajectories of ambulatory function measured by the 6-minute walk distance in patients with Duchenne muscular dystrophy.

Mercuri E, Signorovitch JE, Swallow E, Song J, Ward SJ; DMD Italian Group; Trajectory Analysis Project (cTAP). *Neuromuscul Disord*. 2016 Sep;26(9):576-83.

Revised North Star Ambulatory Assessment for Young Boys with Duchenne Muscular Dystrophy.

Mercuri E, Coratti G, Messina S, Ricotti V, Baranello G, D'Amico A, Pera MC, Albamonte E, Sivo S, Mazzone ES, Arnoldi MT, Fanelli L, De Sanctis R, Romeo DM, Vita GL, Battini R, Bertini E, Muntoni F, Pane M. *PLoS One*. 2016 Aug 5;11(8):e0160195

Timed Rise from Floor as a Predictor of Disease Progression in Duchenne Muscular Dystrophy: An

Observational Study.

Mazzone ES, Coratti G, Sormani MP, Messina S, Pane M, , Previtali SC, Bruno C, Politano L, Comi GP, D'Angelo MG, Bertini E, Mercuri E.

PLoS One. 2016 Mar 16;11(3):e0151445.

Health-related quality of life and functional changes in DMD: A 12-month longitudinal cohort study.

Messina S, Vita GL, Sframeli M, Mondello S, Mazzone E, D'Amico A, Mercuri E.

Neuromuscul Disord. 2016 Mar;26(3):189-96.

Development of a patient-reported outcome measure for upper limb function in Duchenne muscular dystrophy: DMD Upper Limb PROM.

Klingels K, Mayhew AG, Mazzone ES, Duong T, Decostre V, Werlauff U, Vroom E, Mercuri E, Goemans NM; Upper Limb Clinical Outcome Group.

Dev Med Child Neurol. 2017 Feb;59(2):224-231.

Patterns of disease progression in type 2 and 3 SMA: Implications for clinical trials.

Mercuri E, Finkel R, Montes J, Mazzone ES, Sormani MP, Main M, Mercuri E.

Neuromuscul Disord. 2016 Feb;26(2):126-31.

Longitudinal effect of eteplirsen versus historical control on ambulation in Duchenne muscular dystrophy.

Mendell JR, Goemans N, Lowes LP, Alfano LN, Berry K, Shao J, Kaye EM, Mercuri E; Eteplirsen Study Group and Telethon Foundation DMD Italian Network.

Ann Neurol. 2016 Feb;79(2):257-71.

Spinal muscular atrophy functional composite score: A functional measure in spinal muscular atrophy.

Montes J, Glanzman AM, Mazzone ES, Martens WB, Dunaway S; Pediatric Neuromuscular Clinical Research Network, Muscle Study Group, SMA Europe.

Muscle Nerve. 2015 Dec;52(6):942-7.

Correction: Long Term Natural History Data in Ambulant Boys with Duchenne Muscular Dystrophy: 36-Month Changes.

Pane M, Mazzone ES, Sivo S, Sormani MP, Messina S, D'Amico A, Carlesi A, Vita G, Fanelli L, Berardinelli A, Torrente Y, Lanzillotta V, Viggiano E, D'Ambrosio P, Cavallaro F, Frosini S, Barp A, Bonfiglio S, Scalise R, De Sanctis R, Rolle E, Graziano A, Magri F, Palermo C, Rossi F, Donati MA, Sacchini M, Arnoldi MT, Baranello G, Mongini T, Pini A, Battini R, Pegoraro E, Previtali S, Bruno C, Politano L, Comi GP, Bertini E, Mercuri E.

PLoS One. 2015 Dec 4;10(12):e0144079.

Benefits of glucocorticoids in non-ambulant boys/men with Duchenne muscular dystrophy: A multicentric longitudinal study using the Performance of Upper Limb test.

Pane M, Fanelli L, Mazzone ES, Olivieri G, D'Amico A, Messina S, Scutifero M, Mercuri E.

Neuromuscul Disord. 2015 Oct;25(10):749-53

Old measures and new scores in spinal muscular atrophy patients.

Mazzone E, Montes J, Main M, Mayhew A, Ramsey D, Glanzman AM, Dunaway S, Mercuri E.

Muscle Nerve. 2015 Sep;52(3):435-7.

209th ENMC International Workshop: Outcome Measures and Clinical Trial Readiness in Spinal Muscular Atrophy 7-9 November 2014, Heemskerk, The Netherlands.

Finkel R, Bertini E, Muntoni F, Mercuri E; ENMC SMA Workshop Study Group.

Neuromuscul Disord. 2015 Jul;25(7):593-602.

Upper limb module in non-ambulant patients with spinal muscular atrophy: 12 month changes.

Sivo S, Mazzone E, Antonaci L, De Sanctis R, Fanelli L, Palermo C, Montes J, Pane M, Mercuri E.

Neuromuscul Disord. 2015 Mar;25(3):212-5.

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