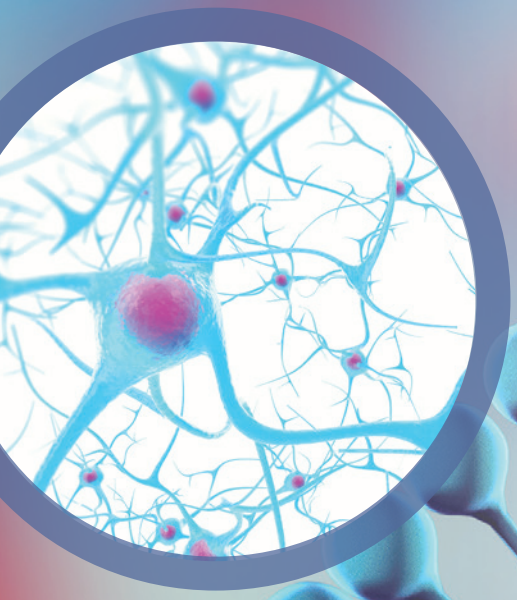


Spinal Muscular Atrophy:
Current Advances in Treatment and Recommendations for Evaluation and Rehabilitation

ONLINE CME

*Originally presented live and recorded on December 6-7, 2019.

A Continuing Medical Education Conference presented by the Department of Neurology, Neuromuscular Division at the Stanford University School of Medicine

Sponsored by the Stanford University School of Medicine

Stanford MEDICINE

STATEMENT OF NEED

This two day course is provided to first, teach healthcare providers on the clinical perspective of spinal muscular atrophy (SMA) including the broad phenotypic spectrum of pediatric and adult patients, evidence of natural history of the disease, new updates to standards of care, clinical outcome measures/standardized assessments, and the influence of therapies both approved and in development; and second, to support healthcare providers, outside of specialized SMA centers, to manage rehabilitation programs and evaluate patient progress using validated standardized clinical assessments in SMA.

At the end of this course, participants will have an understanding of the current advances in treatment as well as recommendations for the evaluation and rehabilitation management in SMA. Attendees will comprehend how to relate the standards of care to clinical practice and assess SMA patients with standardized clinical measures.

TARGET AUDIENCE

This is an international program designed for physicians, nurses, advance practice nurses (APNs), professional nurses (RNs) physician assistants, physical therapists, administrators, researchers, social workers, allied health professionals, occupational therapists, speech language pathologists, residents, fellows and medical students specializing in Neurology, Pediatrics, and Neuromuscular Specialties.

LEARNING OBJECTIVES

At the conclusion of this activity, participants should be able to:

- Summarize the clinical perspectives of Spinal Muscular Atrophy including disease pathology, phenotypic spectrum, and evidence of natural history.
- Choose and organize suitable rehabilitation programs based on available standards of care recommendations.
- Determine and apply appropriate clinical outcome measures based on age and function with correct administration and scoring of motor performance using these common Spinal Muscular Atrophy measures.

ACCREDITATION

The Stanford University School of Medicine is accredited by the Accreditation Council for Continuing Medical Education (ACCME) to provide continuing medical education for physicians.

CREDIT DESIGNATION

The Stanford University School of Medicine designates this enduring materials activity for a maximum of 14.0 *AMA PRA Category 1 Credits*™. Physicians should claim only the credit commensurate with the extent of their participation in the activity.

The California Board of Registered Nursing recognizes that Continu-ing Medical Education (CME) is acceptable for meeting RN con-tinuing education requirements as long as the course is certified for *AMA PRA Category 1 Credit*™ (rn.ca.gov). Nurses will receive a Certificate of Participation following this activity that may be used for license renewal.

FACULTY DISCLOSURE

The Stanford University School of Medicine adheres to ACCME Criteria, Standards and Policies regarding industry support of continuing medical education. Disclosure of faculty and their commercial relationships will be made prior to the activity.

SPEAKERS

COURSE DIRECTORS

John W. Day, MD, PhD
Professor of Neurology and Pediatrics *(Genetics)* and by courtesy, Pathology

Carolina Tesi Rocha, MD
Clinical Associate Professor, Pediatric Neurology & Neurological Science

GUEST FACULTY

Terri Carry, PT
Physical Therapist, Children's Hospital Colorado

Matthew Civitello, MPT
Physical Therapist, Nemours Children's Hospital

Basil Darras, MD
Associate Neurologist-in-Chief, Division of Clinical Neurology
Director, Neuromuscular Center and Spinal Muscular Atrophy Program
Harvard Medical School

Darryl C. De Vivo, MD
Sidney Carter Professor of Neurology
Professor of Pediatrics; Associate Chairman *(Neurology)* for Pediatric Neurosciences
Co-Director, Center for Motor Neuron Biology and Disease
Columbia University Irving Medical Center, The Neurological Institute

Richard S. Finkel, MD
Division Chief, Division of Neurology, Department of Pediatrics, Nemours

Allan Glanzman, PT, DPT, PCS
Clinical Specialist IV in Physical Therapy, Children's Hospital of Philadelphia

Oscar H. Mayer, MD
Professor of Clinical Pediatrics
Attending Pulmonologist and Director of the Pulmonary Function Laboratory, Children's Hospital of Philadelphia

Jacqueline Montes, PT, EdD, NCS
Assistant Professor of Rehabilitation and Regenerative Medicine in the Programs for Physical Therapy,
Columbia University Irving Medical Center

Leslie Nelson, PT, PhD, OCS
Assistant Professor, School of Health Professions,
UT Southwestern Medical Center

Julie Parsons, MD
Professor of Clinical Pediatrics and Neurology
Haberfeld Family Endowed Chair in Pediatric Neuromuscular Disorders
Co-Director, Neuromuscular Clinic, Children's Hospital Colorado
University of Colorado School of Medicine

Amy Pasternak, PT, DPT, PCS
Physical Therapist, Department of Physical Therapy and Occupational Therapy Services, Boston Children's Hospital

Perry B. Shieh, MD, PhD
Associate Professor of Neurology, University of California Los Angeles

STANFORD FACULTY

MyMy Buu, MD, FAAP
Clinical Assistant Professor, Pediatrics - Pulmonary Medicine

John W. Day, MD, PhD
Professor of Neurology and Pediatrics (Genetics) and by courtesy, Pathology

Sally Dunaway Young, PT, DPT
Physical Therapist, Division of Neuromuscular Medicine

Tina Duong, MPT
Physical Therapist, Division of Neuromuscular Medicine

Richard Gee, PT
Physical Therapist, Lucile Packard Children's Hospital

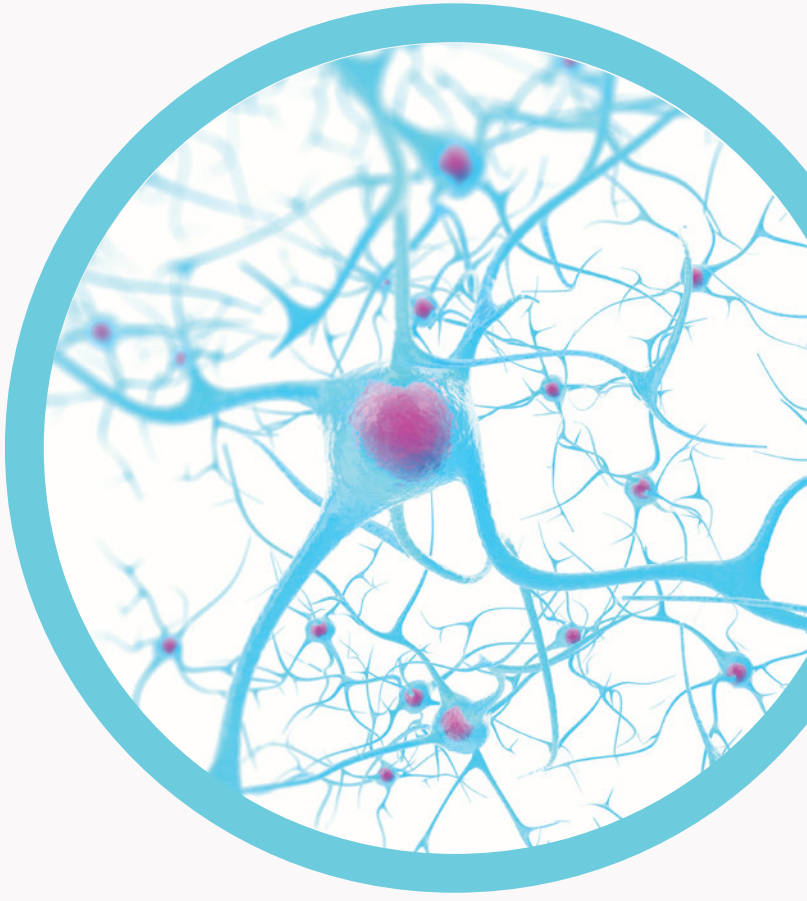
Ava Lin, MD
Neuromuscular Fellow, Neurology and Neurological Sciences

Jacinda Sampson, MD, PhD
Clinical Associate Professor, Neurology and Neurological Sciences

Carly Siskind, MS, LCGC
Clinical Assistant Professor of Neurology (Affiliated); Genetic Counselor

Carolina Tesi Rocha, MD
Clinical Associate Professor, Pediatric Neurology & Neurological Science

Connie Wolford, MSN, FNP-BC
Nurse Practitioner, Stanford Neuromuscular Program



PROGRAM

Day 1 | Recorded Friday, December 6, 2019

8:00–8:15 am	**Welcome and Introductions** • *John Day, MD, PhD and Carolina Tesi Rocha, MD*
8:15–9:00 am	**SMA Pathophysiology, Diagnosis and Clinical Presentations** • *Darryl C. De Vivo, MD and John Day, MD, PhD*
9:00–9:45 am	**Approved Treatment and Upcoming Therapeutic Strategies** • *Perry Shieh, MD, PhD and Basil Darras, MD*

Clinical Phenotypes and Disease Progression

10:15–11:00 am	**Infantile Onset Spinal Muscular Atrophy** • *Carolina Tesi Rocha, MD and Richard S. Finkel, MD*
11:00–11:45 am	**Later Onset Spinal Muscular Atrophy** • *Julie Parsons, MD and Basil Darras, MD*
11:45 am–12:30 pm	**Pre-symptomatic Spinal Muscular Atrophy** • *Richard S. Finkel, MD and Darryl C. De Vivo, MD*
1:30–2:15 pm	**Managing Chronic Adults with Spinal Muscular Atrophy** • *Connie Wolford, MSN, FNP-BC and Jacinda Sampson, MD, PhD*
2:15–3:00 pm	**Respiratory Management in Spinal Muscular Atrophy** • *Oscar Mayer, MD and MyMy Buu, MD, FAAP*
3:30–4:15 pm	**The Patient Experience** • *Moderators: Ava Lin, MD and Carly Siskind, MS, LCGC*
4:15–5:00 pm	**Panel Discussion**

Day 1 | Recorded Saturday December 7, 2019

8:00–8:45 am	**Clinical Evaluations/Assessments for Spinal Muscular Atrophy** • *Tina Duong, MPT*
8:45–9:30 am	**Updated Rehabilitation Standards of Care** • *Richard Gee, PT*
9:30–10:15 am	**Exercise Recommendations in Spinal Muscular Atrophy** • *Jacqueline Montes, PT, EdD, NCS*

Breakout Sessions • Evaluations for Non-sitters

10:30–11:25 am	**Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND)** *Allan M. Glanzman, PT, DPT and Leslie Nelson, PT, PhD, OCS*	**Hammersmith Infant Neurological Exam – Part 2 Motor Milestones** *Basil Darras, MD and Richard S. Finkel, MD*
11:25–11:35 am	Rotate Speakers	
11:35 am–12:30 pm	**Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND)** *Allan M. Glanzman, PT, DPT and Leslie Nelson, PT, PhD, OCS*	**Hammersmith Infant Neurological Exam – Part 2 Motor Milestones** *Basil Darras, MD and Richard S. Finkel, MD*

Breakout Sessions • Evaluations for Sitters and Walkers

1:30–2:25 pm	**Hammersmith Functional Motor Scale Expanded (HFMSE)** *Sally Dunaway Young, PT, DPT and Terri Carry, PT*	**Revised Upper Limb Module (RULM)** *Amy Pasternak, PT, DPT, PCS and Matthew Civitello, MPT*
2:25–2:35 am	Rotate Speakers	
2:35–3:30 pm	**Hammersmith Functional Motor Scale Expanded (HFMSE)** *Sally Dunaway Young, PT, DPT and Terri Carry, PT*	**Revised Upper Limb Module (RULM)** *Amy Pasternak, PT, DPT, PCS and Matthew Civitello, MPT*

3:45–4:15 pm	**Six-Minute Walk Test** • *Jacqueline Montes, PT, EdD, NCS and Leslie Nelson, PT, PhD OCS*
4:15–4:45 pm	**Community Resource Discussion – Network Initiatives** • *Sally Dunaway Young, PT, DPT and Jacqueline Montes, PT, EdD*
4:45 –5:00 pm	**Group Discussion**
5:00–5:15 pm	**Closing Remarks** • *John Day, MD, PhD and Carolina Tesi Rocha, MD*