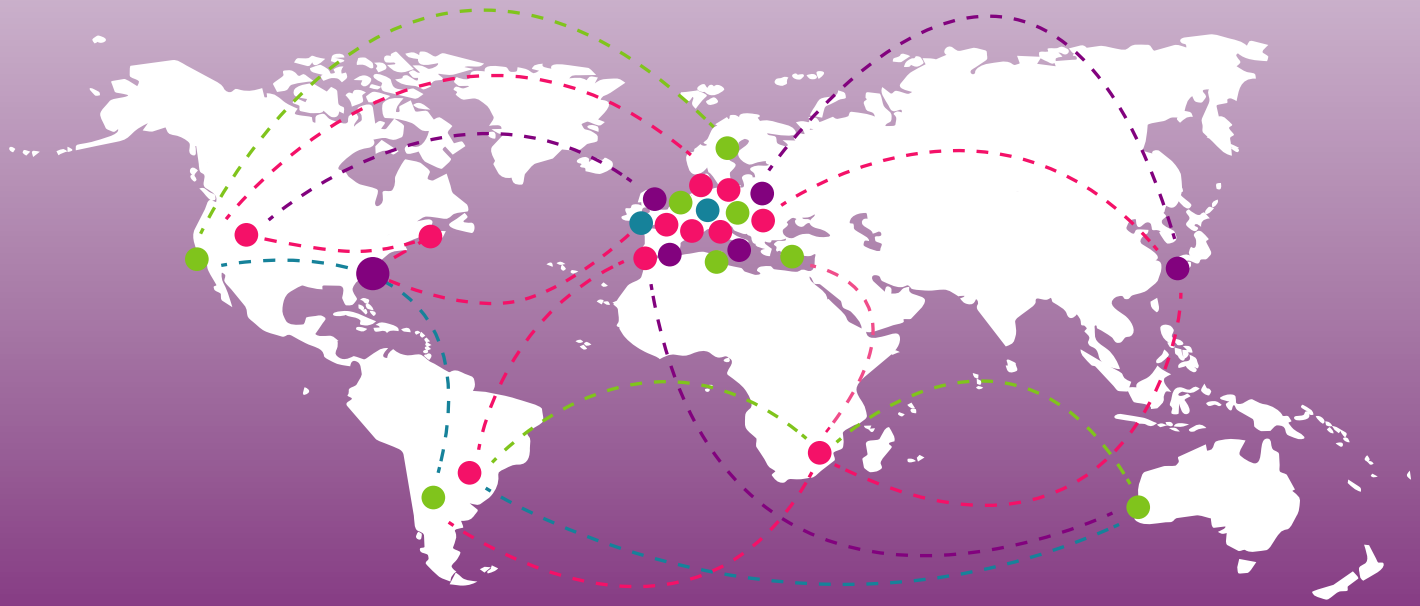
WMS2023

3rd-7th October 2023 Charleston, USA

The 28th Annual Congress of the World Muscle Society



MEET OUR INDUSTRY PARTNERS

WMS
World Muscle Society

WMS2023.COM

Version: September 2023

CONTENTS

President's Welcome

Dear members of the World Muscle Society, friends and colleagues,

Welcome to the 28th annual congress of the World Muscle Society in Charleston, South Carolina.

It's great to be back together again. It's our second consecutive trip to continental North America after our Halifax Congress last year. Our next two Congresses will be in Europe (Prague in 2024 and Vienna in 2025) and then we'll make only our second ever visit to Japan when we host the Hiroshima Congress in 2026. Look out for the announcement during this Congress of where we will go in 2027.

Whether you're attending in person or joining virtually, you can expect all the usual features of a great WMS Congress: lively discussions at the poster boards, informal networking at coffee breaks, and four days of the latest scientific and clinical developments in the field of neuromuscular disorders.

This year, we have also added a suite of new elements: for the first time, we are hosting a 'Career Development Workshop', and a session for 'Interesting Case Discussions' as well as a series of live, informal Myology Café sessions. We have arranged these new activities to promote inclusion, particularly for early career researchers and clinicians and I hope many of you will take part.

On behalf of the Executive Board, I would like to thank our sponsors and exhibitors for their support in enabling this Congress to go ahead. I would also like to extend particular thanks to our Local Organising Committee, Programme Committee and Management Team for their hard work behind the scenes.

I very much look forward to speaking to as many of you as possible over the course of the Congress. I urge you all to take this opportunity to build new connections. Say hello to the people whose work you admire or whose career interests you. Our Society is at its strongest when we make our global work local.

With very best wishes,

Volker Straub
President

Welcome

Dear delegate,

Welcome to Charleston! It is our great pleasure to host you here in the USA.

Our 28th annual Congress is set to be the biggest yet. Not only are we in a venue that enables us to grow our attendance, but we also have a packed programme full of engaging and enlightening sessions.

You can find the full programme in our app and your joining email will include details of the sessions you have selected.

This year we will cover three core topics:

- **Topic 1** - Understanding phenotypic and genetic diversity in neuromuscular disorders
- **Topic 2** - Pathobiology of neuromuscular expansion disorders
- **Topic 3** - The effect of lifestyle, exercise and nutrition on neuromuscular pathology and outcomes

Each day a plenary session will be dedicated to the selected topics. Speakers will summarise the state of the field on the selected topics, covering clinical, molecular and other aspects.

As well as our plenary sessions, we will have opportunities to explore the latest science in any number of areas. Our poster presentations always lead to fascinating discussions and this year promises to be just as lively as any other. Our Prize Giving ceremony on Saturday morning also celebrates innovation and excellence in our field. We'll look forward to seeing you there.

Our thanks to the Executive Board for selecting Charleson, South Carolina as the host city for the 28th Annual Congress, to our fellow members of the Local Organising Committee and Programme Committee for their tireless efforts to ensure a successful event and to everyone involved in organising this year's meeting. It has very much been a team effort!

With best wishes

Lindsay Alfano and Conrad "Chris" Weihl
Congress Co-Chairs

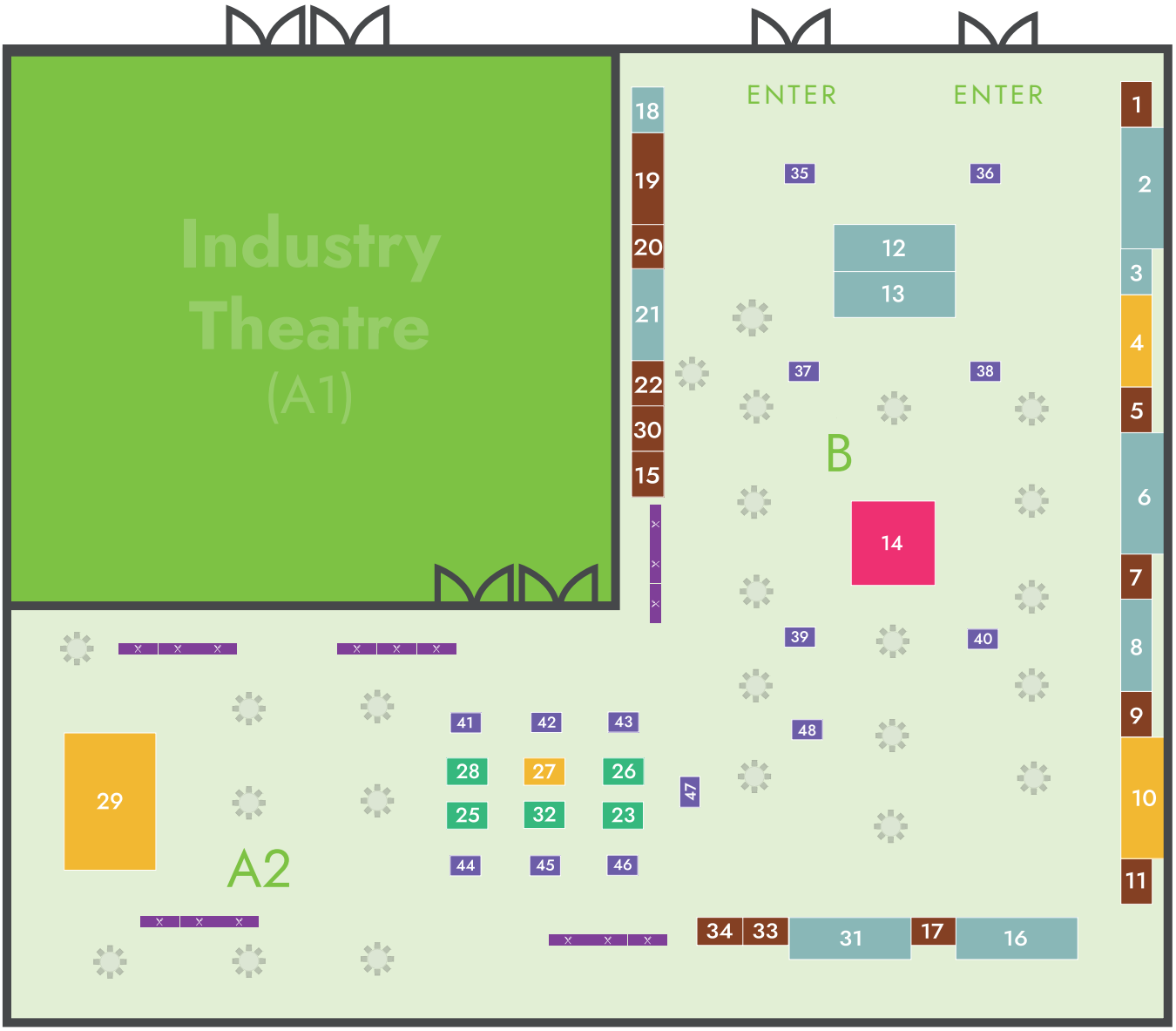
Congress Venue

The Charleston Area Convention Center in North Charleston is located just minutes from the Charleston International Airport and a short shuttle ride to the Historic District.

Charleston Area Convention Center in North Charleston, 5001 Coliseum Drive, North Charleston, SC 29418, U.S.A.

https://www.northcharlestoncoliseumpac.com

Floor Plan



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WMS2023 Congress
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Cure Duchenne – 20 Years

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LGMD2i Fund – Limb Girdle Muscular Dystrophy 2I Research Fund

Family Connection

MDA

Muscular Dystrophy UK – Our Muscles Matter

Parent Project Muscular Dystrophy – Join the Fight. End Duchenne.

SMA Foundation

RYR-I Foundation – Strength in Numbers

Team Titin

Industry Symposia Schedule

		PAC Theatre	Exhibit Hall Theatre A1
Tuesday 3rd October 2023	16:30 - 17:30	WMS Industry Symposium 1 **Bulbar function in spinal muscular atrophy: unmet needs and experiences from clinical practice** Genentech A Member of the Roche Group	WMS Industry Symposium 2 **Becker Muscular Dystrophy Natural History and ARCH, an Open Label Study in BMD: Putting the data in Context** Edgewise THERAPEUTICS
Wednesday 4th October 2023	07:30 - 08:30	WMS Industry Symposium 3 **Duchenne Muscular Dystrophy: What Are the Measures That Matter?** Pfizer	WMS Industry Symposium 4 **Developments in the Management of Late Onset Pompe Disease** Amicus Therapeutics
	19:15 - 20:15	WMS Industry Symposium 5 **Dystrophin in Muscular Dystrophy: Evolving our Understanding** SAREPTA THERAPEUTICS	WMS Industry Symposium 6 **Optimizing Care: Real World Experience in Patients with Late-Onset Pompe Disease** sanofi
Thursday 5th October 2023	08:00 - 09:00	WMS Industry Symposium 7 **Neurodevelopment and Degeneration in SMA: Focus on Upper-Limb Function in Children** Biogen	WMS Industry Symposium 8 **Connecting the dots: all-around care and evidence in Duchenne muscular dystrophy** PTC THERAPEUTICS 25 YEARS

Please see: https://www.wms2023.com/page/industry-symposia for other information.
All times are in local Charleston, South Carolina, USA time.

Myology Café

Come along to our in-person Myology Café Exhibitor Hall B (usually virtual) and find out about how to get involved:

Wednesday 4th October 2023 10:45-11:15 Social Media Committee

Wednesday 4th October 2023 13:45-14:15 New Members' Event

Thursday 5th October 2023 11:00-11:30 Guidelines Committee

Friday 6th October 2023 10:00-10:30
Myology Developments Across the World and Education Committees

WMS World Muscle Society

YOU ARE INVITED

to join the **WMS2023 Industry Symposium Sponsored by Edgewise Therapeutics at the 28th World Muscle Society (WMS) Congress**

Tuesday, October 3, 2023 • 4:30–5:30pm EDT

Becker Muscular Dystrophy Natural History and ARCH, an Open Label Study in Becker: Putting the Data in Context

About the Symposium

Becker muscular dystrophy is a form of muscular dystrophy resulting from mutations in the dystrophin gene, which result in production of a dysfunctional form of that protein. Dystrophin provides a structural link between the contractile elements of the sarcomere and the basement membrane of the myofibers to distribute contractile stress across the muscle. With absent or reduced functional dystrophin, everyday activity produces contraction-induced injury. Though variable in the age of onset, once muscle loss and decline starts it is relentless in its progression. Novel therapies are in development for Becker, including muscle-targeted interventions aimed at positively impacting disease trajectory. In this symposium, **Dr. Erik Niks** will discuss the disease progression and clinical course of Becker muscular dystrophy and review the most recent natural history data. **Dr. Sam Collins** will review the 12-month topline data from the ARCH open label study of EDG-5506 in Becker muscular dystrophy. EDG-5506 is an orally administered small molecule designed to prevent contraction-induced muscle damage in dystrophinopathies including Becker and Duchenne muscular dystrophy. With new natural history data for Becker becoming available, it is important to look at the data from ARCH in that context. To that end, **Dr. Barry Byrne**, will provide context of the data for the Becker patient population.

ORDER OF SPEAKERS

Welcome & Introduction
JOANNE DONOVAN, M.D., PH.D.
Chair; Chief Medical Officer,
Edgewise Therapeutics

"The Natural History of Becker Muscular Dystrophy"
ERIK NIKS, M.D., PH.D.
Pediatric Neurologist,
Leiden University Medical Center

"Twelve-month Data from ARCH, an Open Label Study in Becker Muscular Dystrophy"
SAM COLLINS, M.D., PH.D.
Vice President, Clinical Development,
Edgewise Therapeutics

"Putting the Data in Context"
BARRY BYRNE, M.D., PH.D.
Director, Powell Gene Therapy Center,
University of Florida

PANEL DISCUSSION WITH ALL SPEAKERS TO FOLLOW

Edgewise THERAPEUTICS

Genentech
A Member of the Roche Group

WMS 2023 Industry Symposium sponsored by Genentech, a member of the Roche Group

Join us at our satellite symposium* at WMS 2023 to exchange insights on bulbar function in spinal muscular atrophy (SMA) with our expert panel

Tuesday, 3 October 2023 | 16:30–17:30 EST
Performing Arts Centre (PAC)

Expert Panel

Prof. Katlyn McGrattan (Chair)
University of Minnesota, Minneapolis, USA

Dr Giorgia Coratti
Catholic University of the Sacred Heart, Milan, Italy

Prof. Giovanni Baranello
Great Ormond Street Institute of Child Health, University College London, London, UK

We look forward to seeing you there!

You are also invited to check out our data presentations at WMS!

*This symposium is a non-CME program. CME credits will not be given for attendance.

M-XX-00014006
Date of preparation: July 2023

Duchenne Muscular Dystrophy:
What Are the Measures That Matter?

WEDNESDAY, OCTOBER 4, 2023 | 7:30–8:30 AM EST

WMS Industry Symposium 3 - **Pfizer**
PAC Theatre, Charleston, South Carolina

HYBRID EVENT

Overview

With the arrival of new treatments in Duchenne muscular dystrophy (DMD), we have witnessed a change in the disease trajectory. But what does this mean for the patient journey, and how does it impact the way we monitor disease progression and measure motor function?

Join us for a one-hour symposium during which our expert panel, Prof Laurent Servais and Dr Tina Duong, will deep dive into functional assessment tools used in DMD, focusing on the North Star Ambulatory Assessment and how it correlates with the patients' experience.

Faculty

Laurent Servais, MD, PhD
Professor of Paediatric Neuromuscular Diseases
MDUK Oxford Neuromuscular Center
Department of Paediatrics
University of Oxford

Tina Duong, PT, PhD
Director of Clinical Outcomes Research & Development
Division of Neuromuscular Medicine
Stanford University

Agenda

Introduction

Considerations in Measuring DMD Function Today

Motor Function in DMD: Focus on the North Star Ambulatory Assessment

Q&A and Summary

This symposium is funded by Pfizer Inc
EM-USA-DMD-0029
WMS Industry Symposium

Pfizer

Presented by Medscape Medical Affairs

WMS 2023 Industry Symposium sponsored by PTC Therapeutics

Join us for an engaging and educational symposium led by expert faculty

Connecting the dots: all-around care and evidence in Duchenne muscular dystrophy

Thursday, October 5, 2023
8:00–9:00 AM EDT
Exhibit Hall Theatre A1,
Charleston Convention Center

Breakfast will be provided[a]

John Brandsema, MD, USA (Chair)

Continuity of all-around care: the importance of corticosteroid therapy in DMD

Alexandra Prufer de Queiroz Campos Araújo, PhD, Brazil

The big picture: totality of evidence for ataluren[b] use in nmDMD

Eugenio Mercuri, MD, PhD, Italy

7 years in: real-world evidence of ataluren[b] use in nmDMD

PTC THERAPEUTICS™

[a]Please note, breakfast will be provided for in-person attendees only. PTC Therapeutics International Limited is committed to complying with state laws related to our interactions with healthcare practitioners. If you are a healthcare practitioner licensed in Vermont, we may not be able to provide you with the food offered at this symposium.
[b]Ataluren is not licensed in the United States. Ataluren is indicated for the treatment of Duchenne muscular dystrophy resulting from a nonsense mutation in the dystrophin gene, in ambulatory patients aged 2 years and older in the European Member States and Iceland, Liechtenstein, Norway, Great Britain, Northern Ireland, Kazakhstan, Israel, Republic of Korea, Belarus, Russia, Brazil, Peru, Chile, Macedonia and Uruguay, and aged 5 years and older in the Kingdom of Saudi Arabia, and Ukraine (under special state registration). In Brazil, the indication is specific to male pediatric patients. The presence of a nonsense mutation in the dystrophin gene should be determined by genetic testing (ataluren Summary of Product Characteristics for respective countries).

DMD, Duchenne muscular dystrophy; nmDMD, nonsense mutation Duchenne muscular dystrophy.

This WMS symposium is organized and funded by PTC Therapeutics International Limited and is intended for healthcare professionals only. This session is not included in the main event CME/CPD credit offering.

MED-ALL-ATLN-2300016 | Date of preparation: September 2023

Nexviazyme®
(avalglucosidase alfa-ngpt)

MEET US AT 2023 WMS BOOTH #16

SEE WHAT'S NEX AT NEXVIAZYME.COM/HCP

sanofi

Please see full Prescribing Information for complete details, including **Boxed WARNING.**

SAREPTA THERAPEUTICS' INDUSTRY SYMPOSIUM

Dystrophin in Muscular Dystrophy

Evolving Our Understanding

Join us for an expert-guided presentation where we will review the importance of dystrophin, heterogeneity of dystrophinopathies caused by mutations within the *DMD* gene, and explore how quantity, quality, and distribution of dystrophin can play a role in determining its function.

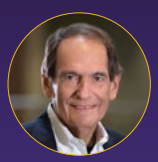
Wednesday, October 4, 2023
7:15 PM – 8:15 PM EST

Charleston Area Convention Center
PAC Theater
North Charleston, SC

Speakers

Moderator
Craig M. McDonald, MD
Department of Physical Medicine & Rehabilitation, Department of Pediatrics
Director, MDA Neuromuscular Disease Clinics
University of California Davis Health
Sacramento, CA

Emma Ciafaloni, MD, FAAN
Director, Pediatric Neuromuscular Program
Co-Director, MDA Neuromuscular Clinic
Professor of Neurology and Pediatrics
University of Rochester
Rochester, NY



Jerry R. Mendell, MD
Professor, Nationwide Children's Hospital
The Ohio State University College of Medicine
The Research Institute of Nationwide Children's Hospital
Columbus, OH

SAREPTA THERAPEUTICS

WMS 2023 Industry Symposium

Biogen

Neurodevelopment and Degeneration in SMA: Focus on Upper-Limb Function in Children

Thursday, October 5, 2023
8:00 AM to 9:00 AM EDT | PAC Theatre

A light breakfast will be provided

Basil Darras, MD
Boston Children's Hospital
Boston, MA, USA

Amy Pasternak, PT, DPT, PCS
Boston Children's Hospital
Boston, MA, USA

Angela Paradis, ScD
Biogen
Cambridge, MA, USA

Accumulated clinical experience is challenging us to rethink the relationship between motor pool composition and motor function. Please join our faculty as they critically examine the clinical data in nusinersen-treated non-ambulatory children, with a particular focus on assessing change in upper-limb function.

About the Artist

"I became an artist because the most important form of freedom is to realize yourself and to be who you are. I love creating magic, putting something together that is completely unusual and so unexpected that it takes people's breath away. Something that is ahead of its time. Five steps ahead of what people think."

Phil L. Herold is an internationally recognized artist who describes himself as a "cyberspace expressionist of the 21st century". Born in Munich in April 1980, and diagnosed with SMA type 2 in early childhood, today Phil designs his large-format works of art on a PC, using a small joystick on his wheelchair. Thus, through only the minimal movement that he is left with, he continues to create, inspire, delight and challenge us.

Nusinersen Prescribing Information. Biogen. Cambridge, MA, USA. Available from: https://www.spinraza.com/content/dam/commercial/spinraza/caregiver/en_us/pdf/spinraza-prescribing-information.pdf. Accessed July 2023.

This symposium has been organized and funded by Biogen Global Medical. This session is not included in main conference CME/CPD credit.

Biogen-214429. June 2023

UCB is committed to

improving the lives of patients living with gMG

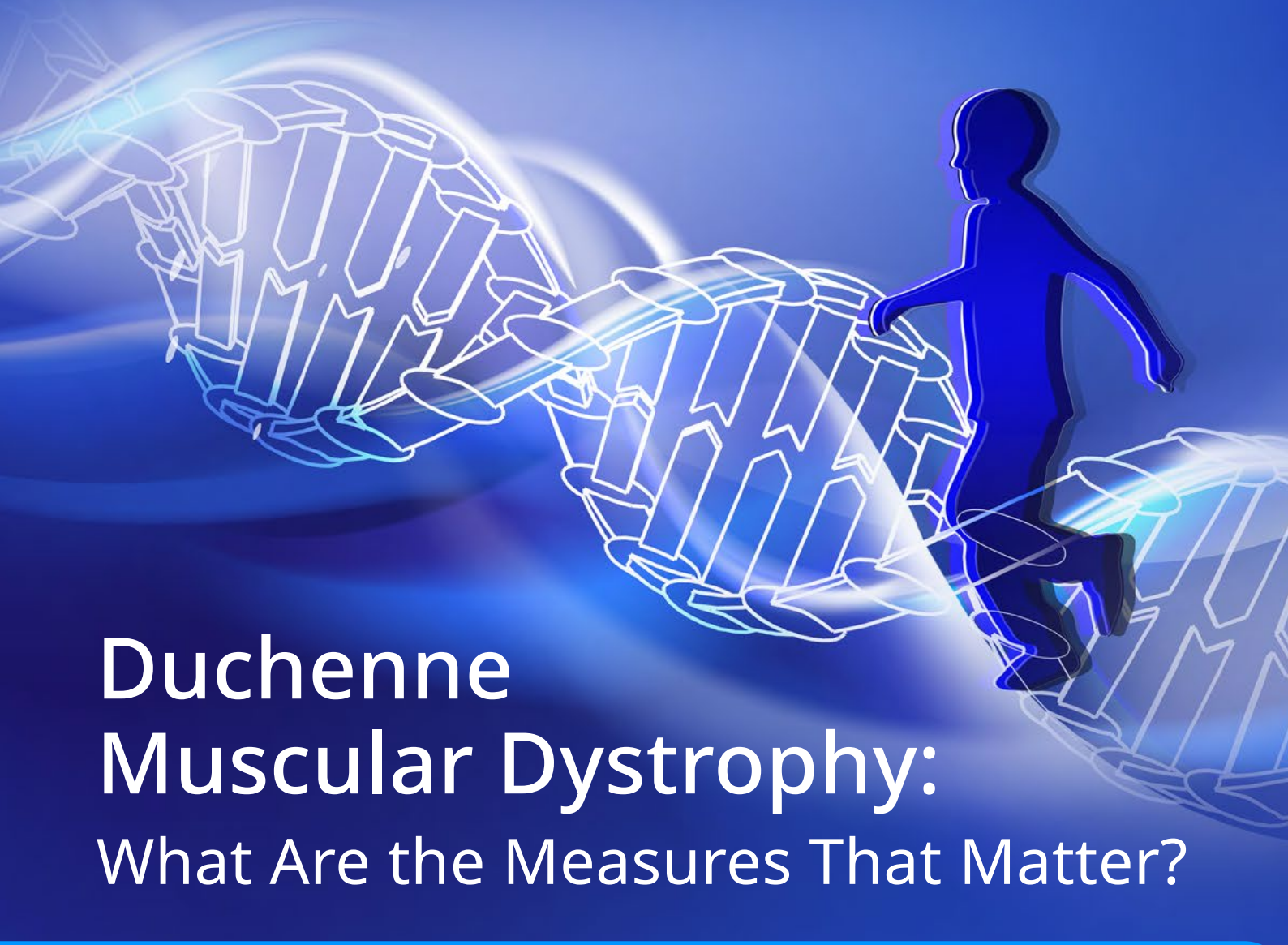
For decades, we've been focused on discovering solutions for people living with chronic diseases. Today, we're building on that legacy by developing multiple innovative solutions for patients living with generalized myasthenia gravis (gMG).

Learn more about our recent advancements at booth 31.

Inspired by **patients.**
Driven by **science.**

US-P-RZ-MG-2300269

Kamilla,
living with MG



Duchenne Muscular Dystrophy:

What Are the Measures That Matter?

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Join us for a one-hour symposium during which our expert panel, Prof Laurent Servais and Dr Tina Duong, will deep dive into functional assessment tools used in DMD, focusing on the North Star Ambulatory Assessment and how it correlates with the patients' experience.

This symposium is funded by Pfizer Inc
EM-USA-DMD-0024
WMS Industry Symposium

Pfizer

Presented by Medscape Medical Affairs

Duchenne Muscular Dystrophy:

What Are the Measures That Matter?

Faculty

Laurent Servais, MD, PhD
Professor of Paediatric
Neuromuscular Diseases
MDUK Oxford Neuromuscular Center
Department of Paediatrics
University of Oxford

Tina Duong, PT, PhD
Director of Clinical Outcomes
Research & Development
Division of Neuromuscular Medicine
Stanford University

Learning Objectives

- Understand the typical patient journey in DMD and the disease trajectory
- Discuss the importance of functional assessment tools in DMD and what clinicians and patients can and should expect from these
- Understand the strengths and limitations of different assessment tools, such as the North Star Ambulatory Assessment (NSAA) rating scale
- Discuss how functional assessment tools can be best utilized in clinical practice and in research

Agenda

Introduction

Considerations in Measuring DMD Function Today

Motor Function in DMD: Focus on the North Star Ambulatory Assessment

Q&A and Summary

This symposium is funded by Pfizer Inc
EM-USA-DMD-0024
WMS Industry Symposium

Pfizer

Presented by
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PLAYING LIKE BROTHERS BECAUSE THEY CAN

Actual EMFLAZA patients.

EMFLAZA® has been shown to preserve muscle strength and function

In a clinical trial of 196 boys aged 5 to 15 with Duchenne muscular dystrophy, the effectiveness and safety of EMFLAZA was compared with placebo (sugar pills) and prednisone. EMFLAZA improved muscle strength at 12 weeks compared with placebo ***(0.15 change in strength score vs -0.10 change in strength score)***

*These findings were not considered statistically significant. This means that because the two groups studied were not large enough, the results could have occurred by chance.

STUDY INFORMATION

Real-world outcomes of long-term prednisone and deflazacort use in patients with Duchenne muscular dystrophy: experience at a single, large care center.

Objective: To assess outcomes among patients with DMD receiving deflazacort or prednisone in real-world practice.

Methods: Clinical data for 435 boys with DMD from Cincinnati Children's Hospital Medical Center were studied retrospectively using time-to-event and regression analyses.

Results: Median ages at loss of ambulation were 15.6 and 13.5 years among deflazacort- and prednisone-initiated patients, respectively. Deflazacort was also associated with a lower risk of scoliosis, improved ambulatory function, greater % lean body mass, shorter stature, and lower weight, after adjusting for age and steroid duration. No differences were observed in whole body bone mineral density or left ventricular ejection fraction.

Marden JR, Freimark J, Yao Z, Signorovitch J, Tian C, Wong BL. Real-world outcomes of long-term prednisone and deflazacort use in patients with Duchenne muscular dystrophy: experience at a single, large care center. J Comp Eff Res. 2020;9(3):177-189. doi:10.2217/ cer-2019-0170.

Delayed onset of scoliosis (curved spine)

LOWERED RISK OF SCOLIOSIS compared to prednisone

Over 11.9 years, 17.9% of patients developed scoliosis taking prednisone vs 7.9% taking deflazacort.

Preserved lung function

9.24% (CI: 3.06–15.41) higher forced vital capacity %-predicted vs prednisone

*Forced vital capacity is a type of test that measures the amount of air your son can inhale and exhale.

Emflaza®
(deflazacort)

6 mg | 18 mg | 30 mg | 36 mg tablets
22.75 mg/mL oral suspension

Summary of Information for EMFLAZA®

What is EMFLAZA® (deflazacort) used for?
Emflaza is a prescription medicine used to treat Duchenne muscular dystrophy (DMD) in patients 2 years of age and older.

When should I not take EMFLAZA?
Do not use if you have had hypersensitivity, including allergic reactions, to deflazacort or any of the inactive ingredients.

What warnings should I know about EMFLAZA?

- EMFLAZA can cause changes in endocrine function. Do not stop taking EMFLAZA, or change the amount you are taking, without first checking with your healthcare provider, as there may be a need for gradual dose reduction to decrease the risk of adrenal insufficiency and steroid "withdrawal syndrome". Acute adrenal insufficiency can occur if corticosteroids are withdrawn abruptly, and can be fatal. A steroid "withdrawal syndrome," seemingly unrelated to adrenocortical insufficiency, may also occur following abrupt discontinuance of corticosteroids. For patients already taking corticosteroids during times of stress, the dosage may need to be increased.
- There is an increased risk of infection when taking EMFLAZA. Tell the healthcare provider if the patient has had recent or ongoing infections or if they have recently received a vaccine. Medical advice should be sought immediately if the patient develops fever or other signs of infection. Patients and/or caregivers should be made aware that some infections can potentially be severe and fatal. Warn patients who are on corticosteroids to avoid exposure to chickenpox or measles and to alert their healthcare provider immediately if they are exposed.
- EMFLAZA can cause an increase in blood pressure and water retention. If this occurs, dietary salt restriction and potassium supplementation may be needed.
- There is an increased risk of developing a hole in the stomach or intestines in patients with certain stomach or intestine disorders when taking corticosteroids like EMFLAZA.
- EMFLAZA can cause severe behavioral and mood changes. Seek medical attention from the health care provider if any behavioral or mood changes develop.
- There is a risk of osteoporosis with prolonged use of EMFLAZA, which can lead to vertebral and long bone fractures.
- EMFLAZA may cause cataracts or glaucoma and a health care provider should monitor for these conditions if corticosteroid therapy is continued for more than 6 weeks.
- Immunizations should be up-to-date according to immunization guidelines prior to starting therapy with EMFLAZA. Live-attenuated or live vaccines should be administered at least 4 to 6 weeks prior to starting EMFLAZA. Live-attenuated or live vaccines should not be used in patients taking EMFLAZA.
- EMFLAZA can cause serious skin rashes. Seek medical attention at the first sign of a rash.
- Rare instances of anaphylaxis have occurred in patients receiving corticosteroid therapy, including EMFLAZA.

What should I tell my health care provider?
Tell the health care provider about all medical conditions, including if the patient:

- is pregnant or planning to become pregnant. EMFLAZA® (deflazacort) can harm your unborn baby.
- is breastfeeding or planning to breastfeed. EMFLAZA may appear in breastmilk and could affect a nursing child.

Certain medications can cause an interaction with EMFLAZA. Tell your healthcare provider of all the medicines you are taking, including over-the-counter medicines (such as insulin, aspirin or other NSAIDS), dietary supplements, and herbal products. Alternate treatment, dosage adjustment, and/or special test(s) may be needed during the treatment.

What are the side effects of EMFLAZA?
The most common side effects of EMFLAZA include facial puffiness or Cushingoid appearance, weight increased, increased appetite, upper respiratory tract infection, cough, frequent daytime urination, unwanted hair growth, central obesity, and colds. These are not all of the possible side effects of EMFLAZA. Call your doctor for medical advice about side effects.

To report an adverse event, please call 1-866-562-4620 or email at usmedinfo@ptcbio.com. You may also report side effects to FDA at 1-800-FDA-1088 or at www.fda.gov/medwatch.

PTC THERAPEUTICS™

AVIDITY® BIOSCIENCES

DELIVERING A NEW CLASS OF RNA THERAPEUTICS

"It is very exciting to think about the future of Avidity and, most importantly, the opportunity to make a positive difference for people with serious diseases who could benefit from AOCs and RNA therapies."

— Sarah Boyce
President & CEO

Avidity Biosciences aims to profoundly improve people's lives by revolutionizing the delivery of RNA therapeutics. Beginning with our muscle disease franchise, our **Antibody Oligonucleotide Conjugates** (AOC™) platform is designed to more effectively target the root cause of disease. Our pipeline includes three lead clinical-stage programs for three distinct rare diseases – myotonic dystrophy type 1 (DM1), Duchenne muscular dystrophy (DMD), and facioscapulohumeral muscular dystrophy (FSHD) – and the potential to expand into additional tissues and cell types, including cardiac tissue and immune cells, through a combination of partnerships and internal discovery that leverages our team's unparalleled expertise in development of RNA and rare disease therapies.

AVIDITY'S PROPRIETARY AOC PLATFORM – FIRST EVER TARGETED DELIVERY OF RNA INTO MUSCLE

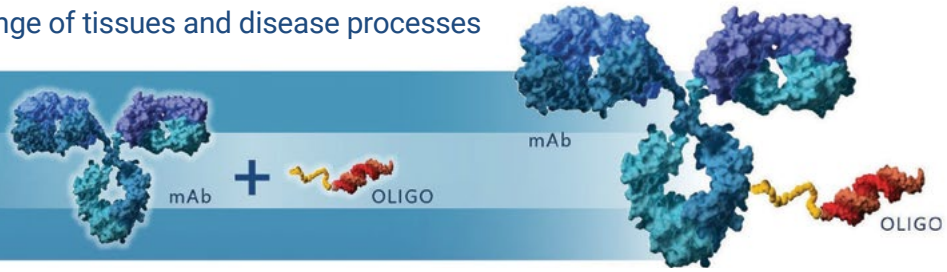
Avidity's proprietary AOC platform combines the specificity of monoclonal antibodies (mAbs) with the precision of oligonucleotide therapies to target a range of different cells and tissues beyond the liver, which up until now have been inaccessible with existing RNA-based therapeutics. In December 2022, we demonstrated the first-ever successful targeted delivery of RNA into muscle, a revolutionary advancement for the field of RNA therapeutics. These data were reported as part of a preliminary assessment of AOC 1001 from a Phase 1/2 clinical trial in DM1. The effective targeted delivery of siRNA into muscle further reinforces the broad and disruptive potential of Avidity's proprietary AOC platform and expands the ability to address targets and diseases previously unreachable with existing RNA therapies. We also have research and development efforts focused on targeting cardiac tissue, immune cells and other cell types.

The broad flexibility of our AOC platform allows us to deploy various types of oligonucleotides, including small interfering RNAs (siRNAs) and phosphorodiamidate morpholino oligomers (PMOs), each of which can be engineered to modify RNA function in different ways to modulate specific disease processes.

continued on reverse

The AOC Platform: Targeting a broad range of tissues and disease processes

Designed to combine the specificity of mAbs with the precision of oligonucleotide therapies



MONOCLONAL ANTIBODIES | **OLIGONUCLEOTIDE THERAPIES** | **ANTIBODY OLIGONUCLEOTIDE CONJUGATE (AOC)**

A DIVERSE AND EXPANDING AOC PIPELINE

PROGRAM/INDICATION	TARGET	LEAD OPTIMIZATION	IND ENABLING	PHASE 1/2	PHASE 3
AOC 1001* Myotonic Dystrophy Type 1 (DM1)	DMPK			MARINA™	MARINA-OLE™
AOC 1044 Duchenne Muscular Dystrophy (DMD)	Exon 44			explore44™	
AOC 1020 Facioscapulohumeral Muscular Dystrophy (FSHD)	DUX4			FORTITUDE™	
Additional DMD Programs	Exon 45 & Undisclosed				
Rare Skeletal Muscle Program	Undisclosed				
Rare Cardiac Program	Undisclosed				

*Sept. 2022, U.S. Food and Drug Administration (FDA) placed a partial clinical hold on new participant enrollment. All current participants may continue in their current dosing cohort. All participants in MARINA may roll over into the MARINA-OLE where they will receive AOC 1001 as planned. Avidity is working to resolve the partial clinical hold as quickly as possible.

Kristl, Zen & Loraine, living with DM1

Nathan, living with DMD

Josh, living with FSHD

OUR THREE LEAD CLINICAL-STAGE PROGRAMS

Avidity's most advanced program is AOC 1001 for the treatment of adults with DM1, which is being assessed in the Phase 1/2 MARINA™ and MARINA-OLE™ clinical trials. In April 2023, Avidity announced positive topline AOC 1001 data from the Phase 1/2 MARINA trial demonstrating functional improvement in multiple clinical outcome measures, disease modification and a favorable safety and tolerability profile in adults with DM1. The disease impacts more than 40,000 people in the U.S. and currently has no approved therapies.

AOC 1044 is being assessed in the Phase 1/2 EXPLORE44™ clinical trial in healthy volunteers and people with DMD amenable to exon 44 skipping (DMD44). DMD is characterized by progressive muscle damage and weakness and is caused by lack of functional dystrophin protein. People living with DMD often require special aid and assistance throughout their lives and have significantly shortened life expectancy. DMD is a monogenic, X-linked, recessive disease that primarily affects males, with 1 in 3,500 to 5,000 boys born worldwide having Duchenne. Currently there are no approved therapies to treat the underlying mechanism of disease for people living with DMD44.

AOC 1020 is currently being assessed in the Phase 1/2 FORTITUDE™ clinical trial in adults living with FSHD, a rare, progressive and variable hereditary muscle-weakening condition marked by progressive loss of function, significant pain, fatigue, and disability. The disease is caused by the abnormal expression of the DUX4 gene and affects approximately 16,000-38,000 people in the U.S. There are currently no approved therapies to treat the underlying cause of FSHD.

AVIDITY® BIOSCIENCES

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to engage with our Medical Affairs team, and learn about our current therapies and exciting clinical trial developments

We are a highly focused, research-driven biopharmaceutical company working in rare diseases. Our current goal is to optimize the potential of exon-skipping therapy in treating Duchenne muscular dystrophy (DMD).

For more information visit NSPHARMA.COM

astellas
GENE THERAPIES

Astellas Gene Therapies is committed to developing innovative genetic medicines for patients with rare neuromuscular disorders.

Please Join our Poster Presentation

P52: Experiences of parents/caregivers of children in the ASPIRO X-Linked Myotubular Myopathy (XLMTM) gene therapy clinical trial: A qualitative study

Session: Clinical trial highlights

October 4, 2023, 14:30-15:30

Presenter: Clara Juando-Pratz

Clinical Research Specialist,
Li Ka Shing Knowledge Institute
(St. Michael's Hospital, Toronto)

Assistant Professor,
Dalla Lana School of Health
University of Toronto

Did you know?

X-linked Myotubular Myopathy (XLMTM) is a rare, life-threatening monogenic congenital myopathy with an estimated incidence of 1 in 40,000-50,000 newborn males.[1,2]

1. Vandersmissen I, et al. 2018;28:766-77.
2. Graham RJ, et al. Arch Dis Child. 2020;105(4):332-8.

To learn more:

Visit our website
AstellasGeneTherapies.com

zolgensma®
(onasemnogene abeparvovec-xioi)
suspension for intravenous infusion

3000+ PATIENTS TREATED AS OF JANUARY 2023[1,a]

The one-time-only dose to stop SMA progression

ZOLGENSMA is a gene therapy for pediatric patients less than 2 years of age with spinal muscular atrophy (SMA), that is delivered as a single-dose, 1-hour intravenous infusion[2]

Event-free survival

91% (20/22) of patients were alive and free of permanent ventilation at the 14-months-of age study visit, **a primary endpoint**, and at 18 months of age[3,b-d]

Motor milestones achieved

59% (13/22) of patients achieved the ability to sit without support for ≥30 seconds at the 18-month study visit, **a primary endpoint**[3,b]

86% (19/22) of patients achieved one or more motor milestones by 18 months of age[3,b]

Rapid onset

As early as 1 month post infusion, CHOP INTEND scores increased from baseline by a mean of 6.9 points (N=22)[3,b]

The efficacy of ZOLGENSMA was evaluated in STR1VE, a completed, open-label, single-arm, multicenter, Phase 3 clinical trial of patients with SMA Type 1 (genetically confirmed bi-allelic *SMN1* deletion, 2 copies of *SMN2*, and <6 months of age at symptom onset and treatment; N=22).[2,b,c]

Visit us at booth #4, or learn more at ZOLGENSMA-hcp.com.

Not an official event of the 2023 WMS Clinical & Scientific Conference. Not sponsored, endorsed, or accredited by WMS.

[a]Globally, including clinical trials, commercially, and through the managed access programs.

[b]One patient was initially classified as presymptomatic and removed from the intent-to-treat (ITT) data set included in the Prescribing Information. The patient was later confirmed to be symptomatic at baseline and included in the final ITT analysis.[4]

[c]One patient died at age 7.8 months due to respiratory failure, which was considered unrelated to treatment. One patient withdrew consent at 11.9 months of age; this patient required permanent ventilation at 11.0 months prior to withdrawal of consent. One patient discontinued participation at the age of 18.0 months, before the month 18 end-of-study visit, due to an adverse event of respiratory distress, which was considered unrelated to treatment.[3]

[d]Event is defined as death or the need for permanent ventilatory support consisting of ≥16 hours of respiratory assistance per day continuously for ≥14 days in the absence of an acute reversible illness, excluding perioperative ventilation.[2]

Indication and Important Safety Information

Indication

ZOLGENSMA is an adeno-associated virus vector-based gene therapy indicated for the treatment of pediatric patients less than 2 years of age with spinal muscular atrophy (SMA) with bi-allelic mutations in the *survival motor neuron 1* (*SMN1*) gene.

Limitations of Use
The safety and effectiveness of repeat administration or the use in patients with advanced SMA (e.g., complete paralysis of limbs, permanent ventilator dependence) has not been evaluated with ZOLGENSMA

Important Safety Information

BOXED WARNING: Serious Liver Injury and Acute Liver Failure

Cases of acute liver failure with fatal outcomes have been reported. Acute serious liver injury, acute liver failure, and elevated aminotransferases can also occur with ZOLGENSMA. Patients with preexisting liver impairment may be at higher risk. Prior to infusion, assess liver function of all patients by clinical examination and laboratory testing. Administer systemic corticosteroid to all patients before and after ZOLGENSMA infusion. Continue to monitor liver function for at least 3 months after infusion, and at other times as clinically indicated. If acute serious liver injury or acute liver failure is suspected, promptly consult a pediatric gastroenterologist or hepatologist.

WARNINGS AND PRECAUTIONS

Systemic Immune Response
Patients with underlying active infection, either acute or chronic uncontrolled, could be at an increased risk of serious systemic immune response. Administer ZOLGENSMA to patients who are clinically stable in their overall health status (e.g., hydration and nutritional status, absence of infection). Postpone ZOLGENSMA in patients with infections until the infection has resolved and the patient is clinically stable.

Thrombocytopenia
Transient decreases in platelet counts, some of which met the criteria for thrombocytopenia, were typically observed within the first two weeks after ZOLGENSMA infusion. Monitor platelet counts before ZOLGENSMA infusion and on a regular basis for at least 3 months afterwards.

Thrombotic Microangiopathy
Cases of thrombotic microangiopathy (TMA) were reported to occur generally within the first two weeks after ZOLGENSMA infusion. TMA can result in life-threatening or fatal outcomes. Obtain baseline creatinine and complete blood count before ZOLGENSMA infusion. Following infusion, monitor platelet counts closely as well as other signs and symptoms of TMA. Consult a pediatric hematologist and/or pediatric nephrologist immediately to manage as clinically indicated.

Elevated Troponin-I
Increases in cardiac troponin-I levels were observed following ZOLGENSMA infusion. Monitor troponin-I before ZOLGENSMA infusion and on a regular basis for at least 3 months afterwards. Consider consultation with a cardiologist if troponin elevations are accompanied by clinical signs or symptoms.

ADVERSE REACTIONS
The most commonly observed adverse reactions (incidence ≥5%) in clinical studies were elevated aminotransferases and vomiting.

Please see Brief Summary of Prescribing Information on the adjacent page.

References: 1. Data on file. Novartis Gene Therapies, Inc. 2023. **2.** ZOLGENSMA [prescribing information]. Bannockburn, IL: Novartis Gene Therapies, Inc.; 2023. **3.** Day JW, Finkel RS, Chiriboga CA, *et al.* Onasemnogene abeparvovec gene therapy for symptomatic infantile-onset spinal muscular atrophy in patients with two copies of *SMN2* (STR1VE): an open-label, single-arm, multicentre, phase 3 trial. *Lancet Neurol.* 2021;20(4):284-293. **4.** Data on file. AveXis, Inc. 2020.

NOVARTIS

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07/2023 US-ZOL-23-0074

ZOLGENSMA® (onasemnogene abeparvovec-xioi) **Rx Only**
Suspension for intravenous infusion
Brief Summary of the Full Prescribing Information.
See Full Prescribing Information.

BOXED WARNING: SERIOUS LIVER INJURY AND ACUTE LIVER FAILURE
- **Cases of acute liver failure with fatal outcomes have been reported. Acute serious liver injury and elevated aminotransferases can also occur with ZOLGENSMA.**
- **Patients with preexisting liver impairment may be at higher risk.**
- **Prior to infusion, assess liver function of all patients by clinical examination and laboratory testing. Administer systemic corticosteroid to all patients before and after ZOLGENSMA infusion. Continue to monitor liver function for at least 3 months after infusion, and at other times as clinically indicated.**

INDICATIONS AND USAGE

ZOLGENSMA is an adeno-associated virus vector-based gene therapy indicated for the treatment of pediatric patients less than 2 years of age with spinal muscular atrophy (SMA) with bi-allelic mutations in the *survival motor neuron 1* (*SMN1*) gene.

Limitations of Use: The safety and effectiveness of repeat administration of ZOLGENSMA or the use in patients with advanced SMA (e.g., complete paralysis of limbs, permanent ventilator dependence) have not been evaluated.

DOSAGE AND ADMINISTRATION

For single-dose intravenous infusion only.

The recommended dosage of ZOLGENSMA is 1.1×10^{14} vector genomes (vg) per kg of body weight.
- Administer ZOLGENSMA as an intravenous infusion over 60 minutes.
- Postpone ZOLGENSMA in patients with infections until the infection has resolved and the patient is clinically stable. Clinical signs or symptoms of infection should not be evident at the time of ZOLGENSMA infusion.
- Starting one day prior to ZOLGENSMA infusion, administer systemic corticosteroids equivalent to oral prednisolone at 1 mg/kg of body weight per day for a total of 30 days.
- At the end of the 30-day period of systemic corticosteroid treatment, check liver function by clinical examination and by laboratory testing. For patients with unremarkable findings, taper the corticosteroid dose gradually over the next 28 days. If liver function abnormalities persist, continue systemic corticosteroids (equivalent to oral prednisolone at 1 mg/kg/day) until findings become unremarkable, and then taper the corticosteroid dose gradually over the next 28 days or longer if needed. Do not stop systemic corticosteroids abruptly.
- If liver function abnormalities continue to persist ≥ 2 x ULN after the 30-day period of systemic corticosteroids, promptly consult a pediatric gastroenterologist or hepatologist.

WARNINGS AND PRECAUTIONS

Acute Serious Liver Injury, Acute Liver Failure or Elevated Aminotransferases

Acute serious liver injury, acute liver failure and elevated aminotransferases can occur with ZOLGENSMA. Hepatotoxicity (which may be immune-mediated), generally manifested as elevated ALT and/or AST levels. Acute serious liver injury and acute liver failure, including fatal cases, have been reported with ZOLGENSMA use. In order to mitigate potential aminotransferase elevations, administer systemic corticosteroid to all patients before and after ZOLGENSMA infusion. Immune-mediated hepatotoxicity may require adjustment of the corticosteroid treatment regimen, including longer duration, increased dose, or prolongation of the corticosteroid taper. Patients with preexisting liver impairment or acute hepatic viral infection may be at higher risk of acute serious liver injury/acute liver failure. Patients with ALT, AST, or total bilirubin levels (except due to neonatal jaundice) > 2 × ULN have not been studied in clinical trials with ZOLGENSMA. Carefully consider the risks and benefits of ZOLGENSMA therapy in patients with preexisting liver impairment. Although in the clinical trials and in postmarketing experience, asymptomatic aminotransferase elevations were very commonly reported, in the managed access program and in the postmarketing setting, cases of acute serious liver injury and acute liver failure including a few cases with fatal outcomes, have been reported. Some patients have experienced elevations in ALT and AST > 20 × ULN, prolonged prothrombin time and have been symptomatic (e.g., vomiting, jaundice), which required the use of corticosteroids, sometimes with prolonged duration and/or a higher dose. If acute serious liver injury or acute liver failure is suspected, promptly consult a pediatric gastroenterologist or hepatologist. Prior to ZOLGENSMA infusion, assess liver function by clinical examination and laboratory testing (hepatic aminotransferases [AST and ALT], total bilirubin level, albumin, prothrombin time, PTT, and INR). Continue to monitor liver function (AST, ALT, total bilirubin, prothrombin time, INR) for at least 3 months after ZOLGENSMA infusion, and at other times as clinically indicated. Promptly assess and closely monitor patients with worsening liver function test results and/or signs or symptoms of acute illness (e.g., vomiting, deterioration in health). In case hepatic injury is suspected, further testing of albumin, PTT, and INR is recommended. Monitor liver function weekly for the first month after ZOLGENSMA infusion and during the corticosteroid taper period (28 days or longer if needed). If the patient is clinically stable with unremarkable findings at the end of the corticosteroid taper period, continue to monitor liver function every other week for another month.

Systemic Immune Response

Due to activation of humoral and cellular immunity following ZOLGENSMA infusion, patients with underlying active infection, either acute (e.g., respiratory, gastrointestinal) or chronic uncontrolled (e.g., chronic active hepatitis B), could be at an increased risk of serious systemic immune response, potentially resulting in more severe clinical courses of the infection. Serious systemic immune response can present with a variety of findings (e.g., high fever, hypotension, etc.). Patients with infection were excluded from participation in ZOLGENSMA clinical trials. Recommend increased vigilance in the prevention, monitoring, and management of infection before and after ZOLGENSMA infusion.

To mitigate the risk of serious and life-threatening systemic immune response, administer ZOLGENSMA to patients who are clinically stable in their overall baseline health status (e.g., hydration and nutritional status, absence of infection) prior to infusion. Postpone ZOLGENSMA in patients with infections until the infection has resolved and the patient is clinically stable. Clinical signs or symptoms of infection should not be evident at the time of ZOLGENSMA infusion. Recommend seasonal prophylaxis against influenza and respiratory syncytial virus (RSV) and vaccination status should be up-to-date prior to ZOLGENSMA administration.

Thrombocytopenia

Transient decreases in platelet counts, some of which met the criteria for thrombocytopenia, were typically observed within the first two weeks after ZOLGENSMA infusion. Monitor platelet counts before ZOLGENSMA infusion and closely monitor platelet counts within the first two weeks following infusion and on a regular basis afterwards (at least weekly for the first month; every other week for the second and third months or until platelet counts return to baseline).

Thrombotic Microangiopathy

Cases of thrombotic microangiopathy (TMA) were reported to occur generally within the first two weeks after ZOLGENSMA infusion in the post-marketing setting. TMA is characterized by thrombocytopenia, microangiopathic hemolytic anemia, and acute kidney injury. Concurrent immune system activation (e.g., infections, vaccinations) was identified in some cases.

Prompt attention to signs and symptoms of TMA is advised, as TMA can result in life-threatening or fatal outcomes.

Monitor platelet counts closely within the first two weeks following infusion and on a regular basis afterwards, as well as signs and symptoms of TMA, such as hypertension, increased bruising, seizures, or decreased urine output. In case these signs and symptoms occur in the presence of thrombocytopenia, further diagnostic evaluation for hemolytic anemia and renal dysfunction should be promptly undertaken. If clinical signs, symptoms and/or laboratory findings consistent with TMA occur, consult a pediatric hematologist and/or pediatric nephrologist immediately to manage TMA as clinically indicated.

Elevated Troponin-I

Increases in cardiac troponin-I levels (up to 0.176 mcg/L) were observed following ZOLGENSMA infusion in clinical trials. The clinical importance of these findings is not known. However, cardiac toxicity was observed in animal studies. Monitor troponin-I before ZOLGENSMA infusion and on a regular basis for at least 3 months afterwards (weekly for the first month, and then monthly for the second and third months until troponin-I level returns to baseline). Consider consultation with a cardiologist, if troponin elevations are accompanied by clinical signs or symptoms (e.g., heart rate changes, cyanosis, tachypnea and respiratory distress).

ADVERSE REACTIONS

The safety data described in this section reflect exposure to ZOLGENSMA in four open-label studies conducted in the United States, including one completed clinical trial, two ongoing clinical trials, and one ongoing observational long-term follow-up study of the completed trial. A total of 44 patients with SMA received intravenous infusion of ZOLGENSMA, 41 patients at or above the recommended dose, and 3 patients at a lower dose. The patient population ranged in age from 0.3 months to 7.9 months at the time of infusion (weight range 3.0 kg to 8.4 kg). The most frequent adverse reactions (incidence ≥ 5%) observed in the 4 studies were elevated aminotransferases* 27.3% (12/44) and vomiting 6.8% (3/44).

* Elevated aminotransferases include elevation of alanine aminotransferase (ALT) and/or aspartate aminotransferase (AST). In the completed clinical trial, one patient (the first patient infused in that study) was enrolled prior to the protocol amendment instituting administration of prednisolone before and after ZOLGENSMA infusion.

One patient in an ongoing non-United States clinical trial initially presented with respiratory insufficiency 12 days after ZOLGENSMA infusion and was found to have RSV and parainfluenza in respiratory secretions. The patient had episodes of serious hypotension, followed by seizures, and was found to have leukoencephalopathy (brain white matter defects) approximately 30 days after ZOLGENSMA infusion. The patient died after withdrawal of life support 52 days after ZOLGENSMA infusion.

Immunogenicity

In ZOLGENSMA clinical trials, patients were required to have baseline anti-AAV9 antibody titers of ≤ 1:50, measured using an enzyme-linked immunosorbent assay (ELISA). Evidence of prior exposure to AAV9 was uncommon. The safety and efficacy of ZOLGENSMA in patients with anti-AAV9 antibody titers above 1:50 have not been evaluated. Perform baseline testing for the presence of anti-AAV9 antibodies prior to ZOLGENSMA infusion. Retesting may be performed if anti-AAV9 antibody titers are reported as > 1:50.

Following ZOLGENSMA infusion, increases from baseline in anti-AAV9 antibody titers occurred in all patients. In the completed clinical trial, anti-AAV9 antibody titers reached at least 1:102,400 in every patient, and titers exceeded 1:819,200 in most patients. Re-administration of ZOLGENSMA in the presence of high anti-AAV9 antibody titer has not been evaluated.

DRUG INTERACTIONS

Where feasible, adjust a patient's vaccination schedule to accommodate concomitant corticosteroid administration prior to and following ZOLGENSMA infusion. Certain vaccines, such as measles, mumps, and rubella (MMR) and varicella, are contraindicated for patients on a substantially immunosuppressive steroid dose (i.e., ≥ 2 weeks of daily receipt of 20 mg or 2 mg/kg body weight of prednisone or equivalent). Seasonal RSV prophylaxis is recommended.

USE IN SPECIAL POPULATIONS

Pediatric Use

Administration of ZOLGENSMA to premature neonates before reaching full-term gestational age is not recommended, because concomitant treatment with corticosteroids may adversely affect neurological development. Delay ZOLGENSMA infusion until the corresponding full-term gestational age is reached. There is no information on whether breastfeeding should be restricted in mothers who may be seropositive for anti-AAV9 antibodies. The safety of ZOLGENSMA was studied in pediatric patients who received ZOLGENSMA infusion at age 0.3 to 7.9 months (weight range 3.0 kg to 8.4 kg). The efficacy of ZOLGENSMA was studied in pediatric patients who received ZOLGENSMA infusion at age 0.5 to 7.9 months (weight range 3.6 kg to 8.4 kg).

Hepatic Impairment

ZOLGENSMA therapy should be carefully considered in patents with liver impairment. Cases of acute serious liver injury and acute liver failure have been reported with ZOLGENSMA in patients with preexisting liver abnormalities. In clinical trials, elevation of aminotransferases was observed in patients following ZOLGENSMA infusion.

PATIENT COUNSELING INFORMATION

See the ZOLGENSMA Full Prescribing Information for the Patient Counseling Information.

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03/2023 US-ZOL-19-0202 V5

Limb-Girdle Muscular Dystrophy

ICD-10 Codes Information Sheet

Did you know? New ICD-10 codes for limb-girdle muscular dystrophy (LGMD) became available for use in October 2022.[1]

The ability to accurately record a patient's LGMD subtype through specific ICD-10 codes, when possible, is an important advancement for several reasons. Adoption of the new ICD-10 codes will support clinical and research communities in ongoing efforts to[2,3]:

- Understand LGMD epidemiology
- Assess natural history / disease progression of the condition
- Understand economic burden of LGMD
- Help manage care of patients
- Facilitate reimbursement and patient access when targeted therapies become available in the future

What should healthcare professionals do?

Up until now, LGMD patients were likely coded under G71.0 "Muscular Dystrophy"[4]:

- G71.00 – "Muscular dystrophy, unspecified"
- G71.09 – "Other specified muscular dystrophies"

If you have LGMD patients captured under G71.00 or G71.09, please refer to the updated ICD-10 codes to accurately capture their diagnosis at their next appointment.[1]

Diagnosis Code	Description	Specific LGMD Subtype, if applicable
G71.031	Autosomal dominant limb-girdle muscular dystrophy	LGMD1/D
G71.032	Autosomal recessive limb-girdle muscular dystrophy due to calpain-3 dysfunction (calpainopathy)	LGMD2A/R1
G71.033	Limb-girdle muscular dystrophy due to dysferlin dysfunction (dysferlinopathy)	LGMD2B/R2
G71.0340	Limb-girdle muscular dystrophy due to sarcoglycan dysfunction, unspecified (sarcoglycanopathy)	
G71.0341	Limb-girdle muscular dystrophy due to alpha sarcoglycan dysfunction (alpha-sarcoglycanopathy)	LGMD2D/R3
G71.0342	Limb-girdle muscular dystrophy due to beta sarcoglycan dysfunction (beta-sarcoglycanopathy)	LGMD2E/R4
G71.0349	Limb-girdle muscular dystrophy due to other sarcoglycan dysfunction	LGMD2C/R5* LGMD2F/R6*
G71.035	Limb-girdle muscular dystrophy due to anoctamin-5 dysfunction (anoctaminopathy)	LGMD2L/R12
G71.038	Other limb-girdle muscular dystrophy	
G71.039	Limb-girdle muscular dystrophy, unspecified	

*LGMD2C/R5 is caused by mutations in the SGCG gene, which encodes gamma-sarcoglycan. LGMD2F/R6 is caused by mutations in the SGCD gene, which encodes delta-sarcoglycan.

G71.038 is intended for all other forms of autosomal recessive LGMD. G71.039 is intended for patients that do not have a genetically confirmed LGMD diagnosis. If your patient has not yet received genetic testing to confirm their LGMD diagnosis, explore no-charge sponsored genetic testing options.

References:
1 Centers for Medicare and Medicaid Services. 2023 ICD-10-CM. Updated January 11, 2023. Accessed March 20, 2023. https://www.cms.gov/medicare/icd-10/2023-icd-10-cm.
2 American Academy of Professional Coders. What is ICD-10? Updated May 20, 2021. Accessed March 20, 2023. https://www.aapc.com/icd-10/
3 Centers for Disease Control and Prevention. International Classification of Diseases, (ICD-10-CM/PCS) Transition – Background. Updated November 6, 2015. Accessed March 20, 2023. https://www.cdc.gov/nchs/icd/icd10cm_pcs_background.htm
4 Centers for Medicare and Medicaid Services. 2022 ICD-10-CM. Updated May 26, 2022. Accessed March 20, 2023. https://www.cms.gov/medicare/icd-10/2022-icd-10-cm

SAREPTA THERAPEUTICS

For people with LGMD

Genetic testing generates new possibilities

A no-charge genetic test can provide a conclusive diagnosis and identify the limb-girdle muscular dystrophy (LGMD) subtype. This can reduce diagnostic delays, aid in family planning, and create a better path forward for disease management.

Order a genetic test today. Impact the care of LGMD tomorrow.

Click to learn more about no-charge genetic testing options.

SAREPTA THERAPEUTICS

IS IT LEMS OR MG?

Lambert-Eaton myasthenic syndrome (LEMS) and myasthenia gravis (MG) share several hallmark signs and symptoms, including **muscle weakness** and **oculobulbar involvement**.[1,2]

ONE WAY TO DIFFERENTIATE THESE TWO IMMUNE-MEDIATED NEUROMUSCULAR DISORDERS IS TO LOOK FOR THESE SPECIFIC SIGNS AND SYMPTOMS[1,2]:

LEMS	MG
Symmetrical muscle weakness	Typically asymmetrical muscle weakness
Caudal-to-cranial pattern of spread	Cranial-to-caudal pattern of spread
Late-onset and mild oculobulbar involvement	Early and prominent oculobulbar involvement
Absent or diminished tendon reflexes	Preserved tendon reflexes
Autonomic dysfunction	No autonomic dysfunction
Transient improvement of muscle strength with exercise, with fatigue that follows	Muscle weakness worsens with exercise
Antibodies to voltage-gated calcium channels (VGCC) are usually found	Antibodies to AChR or MuSK are usually found

SCAN THE CODE

Discover another way to differentiate LEMS from MG with no-cost VGCC antibody testing from Catalyst Pharmaceuticals.

References: 1. Titulaer MJ, Lang B, Verschuuren JJ. Lambert-Eaton myasthenic syndrome: from clinical characteristics to therapeutic strategies. *Lancet Neurol*. 2011;10(12):1098-1107. **2.** Merino-Ramírez MÁ, Bolton CF. Review of the diagnostic challenges of Lambert-Eaton syndrome revealed through three case reports. *Can J Neurol Sci*. 2016;43(5):635-647.

LEMS aware

Catalyst pharmaceuticals

Project Mercury

A new patient-driven global collaboration to speed the delivery of therapies for FSHD

AIMS

- Optimize clinical trial readiness
- Address barriers to patient access to treatments
- Facilitate productive collaboration in FSHD stakeholder ecosystem

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Driven by **science.**

UCB is proud to be a sponsor of the
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Did You *Know?*

Mitochondrial disorders are among the **most prevalent** group of inherited **neurological diseases**.[1]

MayBe*Mito?*

To learn more about mitochondrial disease and thymidine kinase 2 deficiency (TK2d), visit **MayBeMito.com/#tk2d**

Early genetic testing can give patients a path forward by confirming a diagnosis and allowing them to seek the care they need.

Reference: 1. Gorman GS, et al. Prevalence of nuclear and mitochondrial DNA mutations related to adult mitochondrial disease. *Ann Neurol.* 2015;77(5):753-9.