# Quest

MDA Muscular Dystrophy Association

MDA.ORG/QUEST
ISSUE 3 · 2019

EMPOWERING FAMILIES WITH INFORMATION AND INSPIRATION

**THE TOY STORY**
Adaptive fun

**GAME ON**
Finding community through gaming

## better together

The beauty and challenges of raising children while living with neuromuscular disease

NEW

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

Photograph is not of an actual SMA patient and caregiver.

## I'll always remember the day

# we received the one-time-only dose for SMA

ZOLGENSMA® (onasemnogene abeparvovec-xioi) is a prescription gene therapy used to treat children less than 2 years old with spinal muscular atrophy (SMA). ZOLGENSMA is given as a one-time infusion into the vein. ZOLGENSMA was not evaluated in patients with advanced SMA.

To learn more, talk to your child's doctor about **the one-time-only dose** and visit ZOLGENSMA.com.

## Indication and Important Safety Information

**What is ZOLGENSMA?**
ZOLGENSMA is a prescription gene therapy used to treat children less than 2 years old with spinal muscular atrophy (SMA). ZOLGENSMA is given as a one-time infusion into the vein. ZOLGENSMA was not evaluated in patients with advanced SMA.

**What is the most important information I should know about ZOLGENSMA?**

- ZOLGENSMA can cause acute serious liver injury. Liver enzymes could become elevated and may reflect acute serious liver injury in children who receive ZOLGENSMA.
- Patients will receive an oral corticosteroid before and after infusion with ZOLGENSMA and will undergo regular blood tests to monitor liver function.
- Contact the patient's doctor immediately if the patient's skin and/or whites of the eyes appear yellowish, or if the patient misses a dose of the corticosteroid or vomits it up.

**What should I watch for before and after infusion with ZOLGENSMA?**

- Viral respiratory infections before or after ZOLGENSMA infusion can lead to more serious complications. Contact the patient's doctor immediately if you see signs of a possible viral respiratory infection such as coughing, wheezing, sneezing, runny nose, sore throat, or fever.
- Decreased platelet counts could occur following infusion with ZOLGENSMA. Seek immediate medical attention if a patient experiences unexpected bleeding or bruising.

**What do I need to know about vaccinations and ZOLGENSMA?**

- Talk with the patient's doctor to decide if adjustments to the vaccination schedule are needed to accommodate treatment with a corticosteroid.
- Protection against respiratory syncytial virus (RSV) is recommended.

**Do I need to take precautions with the patient's bodily waste?**
Temporarily, small amounts of ZOLGENSMA may be found in the patient's stool. Use good hand hygiene when coming into direct contact with bodily waste for 1 month after infusion with ZOLGENSMA. Disposable diapers should be sealed in disposable trash bags and thrown out with regular trash.

**What are the possible or likely side effects of ZOLGENSMA?**
The most common side effects that occurred in patients treated with ZOLGENSMA were elevated liver enzymes and vomiting.

**The safety information provided here is not comprehensive. Talk to the patient's doctor about any side effects that bother the patient or that don't go away.**

**You are encouraged to report suspected side effects by contacting the FDA at 1-800-FDA-1088 or www.fda.gov/medwatch, or AveXis at 833-828-3947.**

**Please see the Brief Summary of the Full Prescribing Information on the next page.**


US-ZOL-19-0230 08/2019

avexis

**IMPORTANT FACTS ABOUT ZOLGENSMA® (onasemnogene abeparvovec-xioi)**

**USE**

ZOLGENSMA is a prescription gene therapy used to treat children less than 2 years old with spinal muscular atrophy (SMA).

- ZOLGENSMA is given as a one-time infusion into the vein.
- ZOLGENSMA was not evaluated in patients with advanced SMA.

**WARNINGS**

**Acute Serious Liver Injury and Elevated Liver Enzymes**

- ZOLGENSMA can cause acute serious liver injury. Liver enzymes could become elevated and may reflect acute serious liver injury in children who receive ZOLGENSMA.
- Patients will receive an oral corticosteroid before and after infusion with ZOLGENSMA and will undergo regular blood tests to monitor liver function.
- Contact the patient's doctor immediately if the patient's skin and/or whites of the eyes appear yellowish, or if the patient misses a dose of the corticosteroid or vomits it up.

**Decreased platelet counts** could occur following infusion with ZOLGENSMA. Caregivers should seek immediate medical attention if a patient experiences unexpected bleeding or bruising.

**OTHER IMPORTANT INFORMATION**

**Patients should be tested for the presence of anti-AAV9 antibodies** prior to infusion with ZOLGENSMA.

**Vaccination** schedule should be adjusted where possible to accommodate treatment with an oral corticosteroid. Caregivers should talk with the patient's doctor to decide if adjustments to the vaccination schedule are needed during corticosteroid use. Protection against respiratory syncytial virus (RSV) is recommended.

**Viral respiratory infections** before or after ZOLGENSMA infusion can lead to more serious complications. Contact the patient's doctor immediately if you see signs of a possible viral respiratory infection such as coughing, wheezing, sneezing, runny nose, sore throat, or fever.

**Temporarily, small amounts of ZOLGENSMA may be found in the patient's stool.** Use good hand hygiene when coming into direct contact with bodily waste for 1 month after infusion with ZOLGENSMA. Disposable diapers should be sealed in disposable trash bags and thrown out with regular trash.

**COMMON SIDE EFFECTS**

**The most common side effects** that occurred in patients treated with ZOLGENSMA were elevated liver enzymes and vomiting.

**These are not all the possible side effects. Talk to the patient's doctor about any side effects that bother the patient or that don't go away.**

**QUESTIONS?**

To learn more, talk to your doctor and you can visit www.ZOLGENSMA.com for Full Prescribing Information.

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AveXis, Inc.

Bannockburn, IL 60015 US-ZOL-19-0181 06/2019

# Bringing the MDA Community Together

*Elise Qvale*

**At MDA,** families are at the heart of everything we do. And we know that empowering families with knowledge and resources helps individuals with neuromuscular diseases and the people who care for them.

One way we do this is through our flagship event series, MDA Engage. This series began in 2018 with MDA Engage Community Education Seminars and Disease Specific Symposia and has expanded in 2019 to include MDA Engage Community Webinars and Social Gatherings.

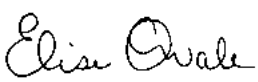
MDA Engage Community Education Seminars and Disease Specific Symposia are informative one-day events typically chaired by an MDA Care Center team member. They help develop a robust educational opportunity with speakers who are experts in the field and individuals living with neuromuscular disease. Key topics include best practices in clinical care, research updates, clinical trials and genetic information. Most MDA Engage Disease Specific Symposia have a drug development roundtable where attendees can talk directly with the drug developers. Each event has a social component to allow attendees to get to know one another.

MDA Engage Community Webinars are online experiences where families can learn about topics important to the neuromuscular disease community. Webinars are led by experts in the field and include question-and-answer sessions.

MDA Engage Social Gatherings facilitate community by bringing MDA families, staff, clinicians, researchers, volunteers, sponsors and supporters together in a casual, social setting. Whether it's a family picnic or a holiday-themed get-together, these local gatherings aim to provide a fun and engaging opportunity for people to connect, share experiences and build a stronger, more dynamic community.

We look forward to seeing you at an upcoming MDA Engage event.

Sincerely,



Elise Qvale
*Director of Program Development & Chief Privacy Officer*
Muscular Dystrophy Association

### *Find an Event Near You*

For a complete list of MDA Engage events and to register, visit **mda.org/care/mda-engage**.

MDA Muscular Dystrophy Association

MDA is committed to transforming the lives of people affected by muscular dystrophy, ALS and related neuromuscular diseases through innovations in science and innovations in care. Since our inception, MDA has committed more than $1 billion to accelerate the discovery of therapies and cures. MDA supports the largest network of multidisciplinary clinics providing best-in-class care at more than 150 of the nation's top medical institutions and serves the community through MDA Summer Camp, the Resource Center and educational conferences, events and materials for families and healthcare providers.

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To update personal information and your *Quest* subscription status, contact the MDA Resource Center at **833-ASK-MDA1**.

***Quest Volume 26, Issue 3***

***Published quarterly by***
**Muscular Dystrophy Association**
161 N. Clark St., Suite 3550
Chicago, IL 60601 | (800) 572-1717
email: **quest@mdausa.org**
Available on the internet at **mda.org/quest**
ISSN 1087-1578
Postage paid at Bolingbrook, IL
Nonprofit postal permit number 1446.

Postmaster: Treat as Standard A Mail Only



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The 2018 Sienna is the only minivan available with a factory-installed power-rotating lift-up seat, giving passengers and caregivers the freedom to do more. The Auto Access Seat's remote control makes getting in, out, and on the road easier than ever. It's just one more addition to the family of mobility options from Toyota.

For more information, visit toyotamobility.com

Prototype shown with options. Available only on Sienna LE with Auto Access Seat and Sienna XLE with Auto Access Seat.
1. Based on manufacturers' data, November 2017. 


issue 3 · 2019

# CONTENTS

DEPARTMENTS




FEATURES





## MORE ONLINE

**SUPPORT GRANTED**
Could scientists provide a definitive genetic diagnosis to more individuals with rare neuromuscular diseases? Could the *SMN2* gene be a target for new therapies for spinal muscular atrophy (SMA)? Researchers are looking for answers to these and other questions — and MDA is providing support to help ensure their success.
**Learn about MDA's recent round of research grants at strongly.mda.org. Click on "Research" at the top of the page.**

MDA-029

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# progress now

**Tracking research updates and breakthroughs that help accelerate treatments and cures across MDA diseases**

## Amyotrophic lateral sclerosis (ALS)

# Enrollment Open for RNS60 Trial

**Phase 2 clinical trial aims to evaluate safety, therapeutic effects**

*Preclinical in vitro and in vivo studies in multiple disease models have indicated that RNS60 may have neuroprotective anti-inflammatory effects.*

A phase 2 trial evaluating the safety and potential therapeutic effects of Tacoma, Wash.-based Revalesio Corporation's experimental ALS drug, RNS60, is currently enrolling participants in Italy and the United States. The trial aims to assess how the drug might affect ALS biomarkers and disease progression.

RNS60 can be administered intravenously or via inhalation and is designed to act on inflammatory processes associated with ALS, thereby preventing damage to cells and tissues.

In a completed open-label pilot study of RNS60 in ALS patients, the drug appeared to be well-tolerated, with no serious side effects.

**Learn more about RNS60 at revalesio.com. For more information about the phase 2 trial and its eligibility requirements, visit ClinicalTrials.gov and enter NCT03456882 in the search box.**

## Charcot-Marie-Tooth disease (CMT)

# Testing Muscle Growth Drug

### ACE-083 granted Orphan Drug status

The U.S. Food and Drug Administration (FDA) has awarded Orphan Drug designation to Acceleron Pharma's ACE-083, a locally acting muscle agent, for the treatment of CMT.

Delivered by intramuscular injection, ACE-083 is designed to enhance the body's own promoters of muscle growth, specifically in the muscles into which the drug is administered. Acceleron is developing the drug to treat diseases such as CMT and facioscapulohumeral muscular dystrophy (FSHD), in which reducing muscle atrophy and weakness in the arms and legs may provide a clinical benefit and improved quality of life.

Orphan Drug designation is important to companies that are developing therapies for rare diseases because it makes them eligible for special incentives including reduced taxes, reduced or waived fees for filing with the FDA, and additional years of market exclusivity (when drugs are protected by law from competition), among others.

ACE-083 is currently being tested in two phase 2 clinical trials, one for CMT and one for FSHD.

**For more information about the phase 2 trial for CMT, visit ClinicalTrials.gov and enter NCT03124459 in the search box. For more information about the phase 2 trial for FSHD, visit ClinicalTrials.gov and enter NCT02927080 in the search box.**

## Duchenne muscular dystrophy (DMD)

# Emflaza Labeling Expanded

### Treatment is now available for patients 2 years of age and older

The U.S. Food and Drug Administration (FDA) has approved expanded labeling for Emflaza (deflazacort) to include treatment of patients with DMD who are between 2 and 5 years old. Emflaza was approved by the FDA in February 2017 for the treatment of DMD in patients 5 years and older, making it the first drug approved to treat DMD regardless of genetic mutation. With this expanded labeling, the drug is approved to treat DMD patients 2 years of age and older.

PTC Therapeutics' Emflaza is a corticosteroid that exerts anti-inflammatory and immunosuppressant effects. The standard of care is to start Emflaza at the time of diagnosis.

**Learn more about Emflaza at emflaza.com. For more information about PTC Cares, call 1-844-4PTC-CARES (1-844-478-2227), or visit ptccares.com.**

# SIDEROS Trial

### Phase 3 study is seeking participants

Santhera Pharmaceuticals is seeking boys and men ages 10 years or older to participate in its phase 3 SIDEROS clinical trial to test the experimental drug Raxone (idebenone) in people with DMD. The study is designed to help researchers determine the safety and efficacy of Raxone, which researchers hope will delay the loss of respiratory function in DMD. This study aims to compare Raxone to a placebo in individuals currently receiving steroid treatment.

Raxone is an oral tablet. Scientists hypothesize it may work by improving the way muscles utilize fuel to power movement. Participants are expected to make nine clinical visits over 22 months. These visits will include a series of lung function tests to determine changes in lung capacity.

**To learn more about trial sites and eligibility requirements, or to inquire about participation, email SIDEROS@santhera.com, visit Santhera's website at santhera.com or visit ClinicalTrials.gov and enter NCT02814019 in the search box.**

# DMD Participants Sought

### Survey to determine most critical DMD symptoms

Researchers at the University of Rochester in New York are seeking individuals diagnosed with DMD to help develop disease-specific patient-reported outcome measures for future clinical trials. This study will help to determine the most critical symptoms of DMD in children, young adult and adult ➤

patients so researchers can measure small but clinically significant therapeutic gains during future clinical trials. During a phone interview, researchers will use open-ended questions to ask participants to identify key symptoms of DMD that have an impact on the quality of life. Researchers specifically want to focus on two groups: ages 11–17 and ages 18 and above. Minors will need a parent's verbal permission granted to the study coordinator and would need to offer their own verbal consent. Adults are required to give their own verbal consent. All interviews will be recorded, transcribed for later reference and kept in a secure location. Participants must be 11 years or older, speak English and have a DMD diagnosis based on a report from their physician or MDA's registry. No travel is required.

**To learn more and to inquire about participation, contact Christine Zizzi at 585-276-7772 or Christine.zizzi@chet.rochester.edu.**

Lambert-Eaton myasthenic syndrome **(LEMS)**

# Ruzurgi Approved for Kids

## Drug is first approved treatment for pediatric LEMS patients

The U.S. Food and Drug Administration (FDA) approved Ruzurgi (amifampridine) for the treatment of children with LEMS who are between 6 and 17 years of age.

Ruzurgi, developed by Jacobus Pharmaceuticals, is an oral potassium channel inhibitor designed to prolong signals released from nerves to allow greater stimulation of muscle. Ruzurgi works in a similar mechanism to Firdapse (amifampridine phosphate), which was approved in 2018 for the treatment of adults with LEMS.

Evidence from controlled studies in adults with LEMS was used to support the effectiveness of Ruzurgi to treat LEMS in patients 6 to less than 17 years of age, according to the FDA.

Effectiveness was also measured by a self-assessment scale for LEMS-related weakness, with scores indicating that patients switched to placebo perceived greater muscle weakening.

**Read more about Ruzurgi at mda.org.**

IMAGE: ISTOCK.COM/FATCAMERA

# The National ALS Registry: Get The Facts

The National Amyotrophic Lateral Sclerosis (ALS) Registry enables persons with ALS to fight back and help defeat ALS (Lou Gehrig's Disease). By signing up, being counted, and answering brief questions about your disease, you can help researchers find answers to critical questions.

**Learn more at www.cdc.gov/als or (800) 232-4636**

## Who can sign-up?

Anyone with ALS

## What do I need?

- A computer with an internet connection
- An email address

***No computer?*** Don't worry! A family member, caregiver or friend with a computer can help you. You can also contact your local ALSA chapter or use the computer at your public library

## What if I need help?

Caregivers and others can help you in person or even over the phone

## What kind of information is collected?

- Basic demographics (e.g., age, sex, height, weight)
- Military history
- Physical activity
- Family history

## Will my information be private?

- YES! Only approved registry scientists can see it, NOT employers or insurers
- You CANNOT be looked up in the registry by name

## Do I need to update my information?

YES! Every six months – you'll get an email reminder

**YOU JOINING**

More information for research

A better understanding of ALS

The chance to help create a better future for persons with ALS

Myasthenia gravis (MG)

# Soliris Pediatric Trial

**Phase 3 trial testing eculizumab seeks participants**

Researchers at Alexion Pharmaceuticals are looking for children with generalized myasthenia gravis (gMG) to participate in an open-label phase 3 study. The goal of the study is to evaluate the safety and efficacy of Soliris (eculizumab) in pediatric patients. This therapy may improve muscle strength by reducing inflammation caused by the immune system.

All participants in the trial will be treated with Soliris. The total study duration for each patient will be up to 4.7 years, with clinic visits every two weeks. The primary endpoint for efficacy is the change from baseline in the quantitative myasthenia gravis (QMG) total score. Throughout the study, participants will continue to see their regular doctor for routine care. Travel support may be available.

**To learn more about participation and complete eligibility requirements, contact Edward Miretsky at Edward.Miretsky@alexion.com.**

Myotonic dystrophy (DM)

# New MVP Award

**Gene therapy may reverse some features of DM**

*Locana's vice president of research and development, Ranjan (Ron) Batra, Ph.D. (right), will serve as the principal investigator on the project. Gene Yeo, Ph.D., MBA (left), is a co-founder of Locana and chair of its scientific advisory board.*

MDA awarded Locana, a leading RNA-targeting gene therapy company, MDA Venture Philanthropy (MVP) funding totaling $550,000 to advance Locana's development program for DM, the most common form of adult-onset muscular dystrophy.

MVP is MDA's drug development program that is exclusively focused on funding the discovery and clinical application of treatments and cures for neuromuscular disorders.

MDA and Locana are hopeful that the company's novel approach to designing highly specific, RNA-targeting therapeutic

candidates for DM can address the significant unmet need for treatments for this genetic disease.

Locana's technology aims to address a wide spectrum of human genetic diseases via an approach that is distinct from DNA gene-editing approaches (such as CRISPR/Cas9) and nucleic acid-based RNA-targeting approaches (such as antisense oligonucleotides). The therapy to treat DM will use a small virus to deliver the RNA-targeting therapeutic into the cells of the affected tissues. The goal is to develop a single-dose treatment that could provide a long-lasting benefit to patients.

**For more information, visit locanabio.com.**

Spinal muscular atrophy **(SMA)**

IMAGE: ISTOCK.COM/UNDEFINED

# Zolgensma Approved

## One-time treatment is first gene therapy for a neuromuscular disease

In May, the U.S. Food and Drug Administration (FDA) approved Zolgensma, the first gene therapy for a neuromuscular disease. Zolgensma is a one-time intravenous infusion for the treatment of pediatric patients younger than 2 years of age who have SMA with mutations in both copies of the survival motor neuron 1 gene (*SMN1*), including those who do not yet have symptoms at the time of diagnosis.

Zolgensma is designed to target the genetic root cause of SMA by delivering the *SMN* gene, which is missing or mutated in SMA. Zolgensma is available in the United States and marketed by AveXis, a Novartis company.

In clinical trials, treatment with Zolgensma was associated with an increased survival rate compared to the normal course of the disease and the achievement and maintenance of motor milestones that infants with SMA type 1 normally would not be expected to achieve.

The OneGene Program, AveXis' patient support program, provides a dedicated, personalized support team focused on the needs of each family throughout the Zolgensma treatment journey. This includes answering questions about Zolgensma, verifying reimbursement assistance and coordinating financial assistance programs for eligible patients.

**For more information, call 1-855-441-GENE (1-855-441-4363).**

PTC *Cares*™

# YOUR TREATMENT NEEDS ARE OUR PRIORITY

**PTC *Cares*™ is a patient support program that provides personalized case management support before your first prescription and throughout treatment.**

## PTC *Cares* is here to help with:

- Benefits investigation support
- Information about EMFLAZA® (deflazacort) and PTC *Cares* services
- Securing access to EMFLAZA

**Call 1-844-4PTCCARES (1-844-478-2227)**

8 AM - 8 PM ET Monday - Friday to speak with a PTC *Cares* case manager.

**Visit www.PTCCares.com for more information.**

**EMFLAZA is indicated for the treatment of Duchenne muscular dystrophy in patients 2 years of age and older.**

**Do not use if you are allergic to deflazacort or any of the inactive ingredients in EMFLAZA.**

**Please see full Prescribing Information at www.emflaza.com.**

Emflaza® (deflazacort)

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

## INDICATION & IMPORTANT SAFETY INFORMATION FOR EMFLAZA® (deflazacort)

### INDICATION

EMFLAZA® is a corticosteroid indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 2 years of age and older.

### IMPORTANT SAFETY INFORMATION

**Contraindication:** Do not use if you are allergic to deflazacort or any of the inactive ingredients in EMFLAZA.

**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.

- **Hyperglycemia:** Corticosteroids can increase blood glucose, worsen pre-existing diabetes, predispose those on long-term treatment to diabetes mellitus, and may reduce the effect of anti-diabetic drugs. Monitor blood glucose at regular intervals. For patients with hyperglycemia, anti-diabetic treatment should be initiated or adjusted accordingly.
- **Increased Risk of Infection:** Tell your healthcare provider if you have had recent or ongoing infections or if you have recently received a vaccine or are scheduled for a vaccination. Seek medical advice at once should you develop fever or other signs of infection, as some infections can potentially be severe and fatal. Avoid exposure to chickenpox or measles, but if you are exposed, medical advice should be sought without delay.
- **Alterations in Cardiovascular/Kidney Function:** EMFLAZA can cause an increase in blood pressure, salt and water retention, or a decrease in your potassium and calcium levels. If this occurs, dietary salt restriction and potassium supplementation may be needed.
- **Behavioral and Mood Disturbances:** There is a potential for severe behavioral and mood changes with EMFLAZA and you should seek medical attention if psychiatric symptoms develop.
- **Effects on Bones:** There is a risk of osteoporosis or decrease in bone mineral density with prolonged use of EMFLAZA, which can potentially lead to vertebral and long bone fractures.
- **Effects on Growth and Development:** Long-term use of corticosteroids, including EMFLAZA may slow growth and development in children.
- **Ophthalmic Effects:** EMFLAZA may cause cataracts, ocular infections and glaucoma and you should be monitored if corticosteroid therapy is continued for more than 6 weeks.
- **Vaccination:** The administration of live or live attenuated vaccines is not recommended in patients on EMFLAZA. Live-attenuated or live vaccines can be administered at least 4 to 6 weeks prior to starting EMFLAZA.
- **Serious Skin Rashes:** Seek medical attention at the first sign of a rash.
- **Drug Interactions:** 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.

**Common side effects that could occur with 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.

**Please see www.emflaza.com for the full Prescribing Information.**

For medical information, product complaints, or to report an adverse event, please call **1-866-562-4620** or email at **usmedinfo@ptcbio.com.**

**You may report adverse events to FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.**



PTC THERAPEUTICS™

*Katrina Gossett Kelly and her son, Gosho*

# wheels LIKE MINE

## *The beauty and challenge of raising a child with SMA while living with SMA*

BY KATRINA GOSSETT KELLY

I am a mom. I have spinal muscular atrophy (SMA). My son has SMA, too. Because SMA is a genetic disorder, these statements may not seem surprising. It wouldn't be all that strange for a person with SMA to pass on their condition to their child. But that is not our story. Our journey to family is more convoluted and, dare I say, even more beautiful than most would guess. Ours is a story of love and adoption and beauty where others see tragedy.

## THE STARS ALIGN

I've wanted to be a mother all my life. When I was 5, I didn't have imaginary friends; I had imaginary children ranging from ages 3 to 16. In the following years, I assembled quite the family of Cabbage Patch Kids. I never viewed SMA as a barrier to parenting. But, of course, things were a bit easier when my kids were imaginary or made of plastic.

Two decades later, I met my husband, Russel. Shortly after we were married, we got "baby fever" and had to come up with a plan for becoming parents. I had already decided that pregnancy wasn't for me. Russel and I had both considered adoption, as it was a part of our families. We were drawn to international adoption, and, in particular, Bulgaria, because it is more open to parents with disabilities.

I pored through pictures of children available for adoption in Bulgaria. Most of them have disabilities, as it is harder for them to find families because of cultural stigma. I had no qualms about adopting a disabled child, but I was not necessarily looking for a kiddo just like me. That is, until fate stepped in.

I saw his profile. He was an adorable 4-year-old named Gosho, and his profile said he was a caring little boy who loved to assist his visually impaired friend reach his toys. My heart was aflutter. And then I read the next sentence: "This little boy has spinal muscular atrophy." My heart stopped, then it burst wide open. I was looking at my son. I'd never thought about adopting a child with SMA, but there he was, and I had no choice.

This was the start of our family.

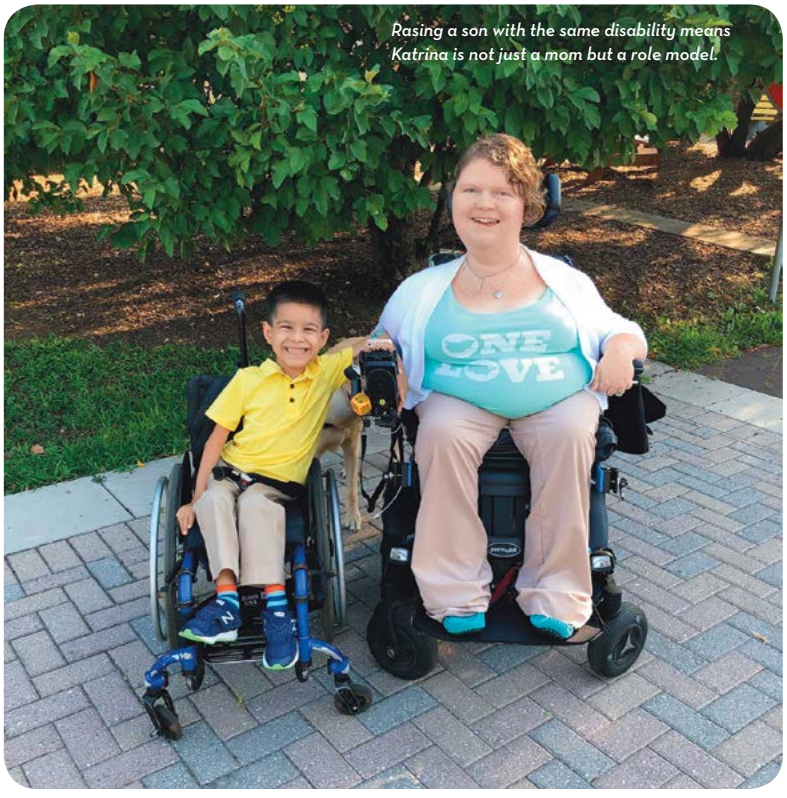
## CHALLENGES AND SUCCESSES

I'll be the first to admit that it's not always simple to parent a child with the same disability. There are times when Gosho is having a bad day, and I just want to scoop him up into my arms and hug him tight. Instead, we work around it. We hold hands. He rests his head on my feet when he needs a good cry. And Daddy lifts him up to give me a hug every night before bed.

I also asked local engineering students to design a seat for Gosho that attaches to my wheelchair so I can carry him around. He loves going

*"'This little boy has spinal muscular atrophy.' My heart stopped, then it burst wide open. I was looking at my son. I'd never thought about adopting a child with SMA, but there he was, and I had no choice."*

➤ *continues on page 18*




*Rasing a son with the same disability means Katrina is not just a mom but a role model.*

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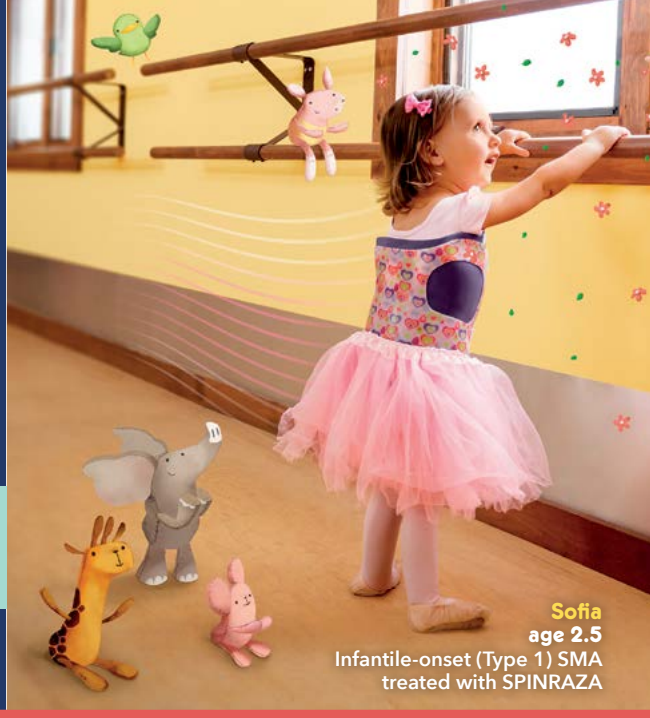
SPINRAZA® (nusinersen) injection 12 mg/5 mL

# Robust clinical trial

## and real-world experience




## 2700+ individuals in the US have been treated at sites in all 50 states*

**>80%** of individuals are in the maintenance dosing phase

**>95%** of individuals who started on SPINRAZA remain on treatment

**2+** years of real-world experience

**Find a treatment center near you at TreatWithSPINRAZA.com**

*Based on commercial patients in the US (including Puerto Rico) as of March 2019.

### INDICATION

SPINRAZA is a prescription medicine used to treat spinal muscular atrophy (SMA) in pediatric and adult patients.

### IMPORTANT SAFETY INFORMATION

**Increased risk of bleeding complications** has been observed after administration of similar medicines. Your healthcare provider should perform blood tests before you start treatment with SPINRAZA and before each dose to monitor for signs of these risks. Seek medical attention if unexpected bleeding occurs.

**Increased risk of kidney damage, including potentially fatal acute inflammation of the kidney**, has been observed after administration of similar medicines. Your healthcare provider should perform urine testing before you start treatment with SPINRAZA and before each dose to monitor for signs of this risk.

**The most common side effects of SPINRAZA include** lower respiratory infection, fever, constipation, headache, vomiting, back pain, and post-lumbar puncture syndrome.

These are not all of the possible side effects of SPINRAZA. Call your healthcare provider for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

**Before taking SPINRAZA**, tell your healthcare provider if you are pregnant or plan to become pregnant.

**For additional Important Safety Information, please see brief summary of full Prescribing Information on the next page.**

This information is not intended to replace discussions with your healthcare provider.


**Individual results may vary based on several factors, including severity of disease, initiation of treatment, and duration of therapy.**

## IMPORTANT FACTS ABOUT SPINRAZA® (nusinersen)

### USES

SPINRAZA is a prescription medicine used to treat spinal muscular atrophy (SMA) in pediatric and adult patients.

### WARNINGS

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**Increased risk of kidney damage, including potentially fatal acute inflammation of the kidney**, has been observed after administration of similar medicines. Your healthcare provider should perform urine testing before you start treatment with SPINRAZA and before each dose to monitor for signs of this risk.

### COMMON SIDE EFFECTS

- **The most common side effects of SPINRAZA include** lower respiratory infection, fever, constipation, headache, vomiting, back pain, and post-lumbar puncture syndrome (headache related to the intrathecal procedure).
- Serious side effects of complete or partial collapse of a lung or lobe of a lung have been reported.

**Talk to your healthcare provider about any side effect that bothers you or that does not go away.**

### OTHER INFORMATION

SPINRAZA is a medication that should be administered as an injection into the lower back (a procedure called intrathecal injection) by, or under the direction of, an experienced healthcare professional.

**Before taking SPINRAZA**, tell your healthcare provider if you are pregnant or plan to become pregnant.

### QUESTIONS?

The risk information provided here is not comprehensive. To learn more, talk about SPINRAZA with your healthcare provider or pharmacist. The FDA-approved product labeling can be found at www.spinraza.com or 1-844-4SPINRAZA (1-844-477-4672).

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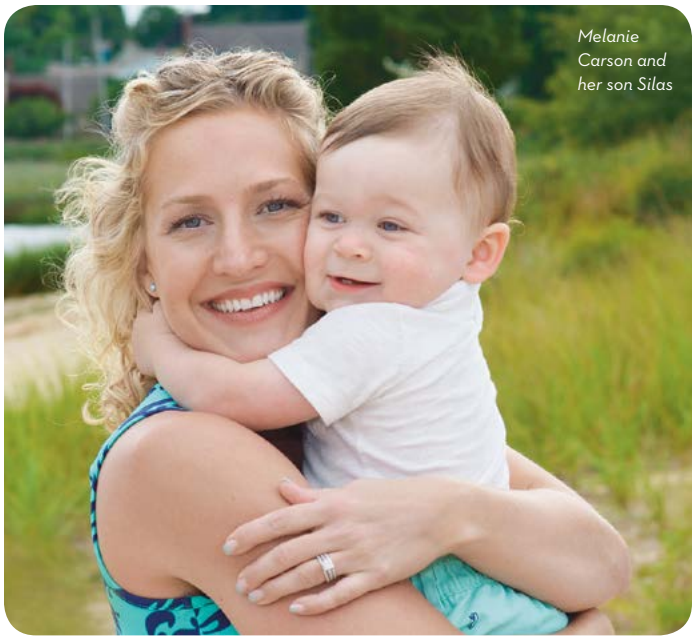


Melanie Carson and her son Silas

James Parsell and his son Christofer

## PRIMED FOR PARENTING

Anybody with kids will tell you that being a parent is the toughest job they've ever loved. Navigating parenthood while living with limited mobility due to neuromuscular disease adds an extra challenge. Here are four strategies from people who have been there.

**1 Cultivate your verbal skills.** Parenting is every bit as verbal and emotional as it is physical. If your limb movement is restricted, consider delegating tasks such as feeding and bathing to a caregiver while you take on the all-important roles of ouchie-soother, homework helper and pep talker.

Melanie Carson breaks down instructions to her 2-year-old, Silas, into single actions. "I'll say, 'Pick up your shoes. Now walk to the closet. Set the shoes inside. Now close the door,'" says Carson, whose movement is slow because of limb-girdle muscular dystrophy (LGMD).

James Parsell's Duchenne muscular dystrophy (DMD) prevents him from cooking, but he can guide his 9-year-old son, Christofer, through making his own simple meals.

"Most neuromuscular diseases are physical, not mental," says physical therapist Polly Swingle, CEO of The Recovery Project in Detroit. "These parents are in fact the ideal candidates to be sitting alongside their child with the timer, saying, 'You do numbers 1 through 10 while I check your finished work.'"

**2 Don't shy away from mobility aids.** In her work at Michigan-area MDA Care Centers, Swingle frequently encounters adults who are resistant to using mobility devices. "They fear becoming dependent on the device," she says.

Swingle aims to show her mom and dad patients that braces, walkers, wheelchairs and accessible vans are not to be feared, and can even facilitate awesome interactions with their children. "That chair or van might be the only thing between you and your kid's softball game," she says.

**3 Invite the therapist over.** Give your physical and occupational therapists a tour of your home. "That's the only way we can see the specific obstacles and opportunities in your house and come away with a completely customized strategy," Swingle says.

Furnishings and accessories like mini fridges, strap handles, roll-out drawers and low-height cribs and changing tables are just a few of the things therapists can suggest to help with the daily tasks of childrearing.

Therapists will often suggest home visits during consultations, but if not, just ask.

**4 Help me, help you.** Enlisting kids to help with physically challenging tasks is a great way to teach them needed skills. "I used to have Christofer help me put my shoes on when he was learning to tie his shoes," Parsell says.

Carson encourages Silas to bring her objects from the couch and other low furniture to conserve her energy and teach him the safe way to climb. "He learned how to safely climb into the pool from swim lessons, and I've taught him that's the same way to climb off furniture," she says.

on adventures on Mommy's wheelchair.

There are other smaller challenges. I can't help him open his snacks, so I bought a pair of scissors he can use. I can't pick him up when he falls, but I can give him a footrest boost and some pretty awesome cheers. In fact, Gosho has become more independent because he knows he can't rely on my physical help to do things.

Sometimes, his disability actually makes it easier for me to parent him. I can stop him from running into traffic by blocking his wheelchair with my own. When he is having a meltdown, I can keep him in place and get down to his level so that we can work through it. On those days, I am grateful that I can outrun my spirited little boy.

## ROLL MODELING

There are also moments when I am humbled by the opportunity to raise a little boy with the same disability as me, one who will face many of the same challenges. In those moments, I am grateful that I've been down the same accessible (and sometimes inaccessible) paths, where I can be a role (or roll) model.

Recently, we were talking about his last days of kindergarten and how he is getting bigger. Out of the blue, Gosho asked, "When will I walk?"

I paused. I had wondered the same thing when I was little. I wished I could give him the answer he wanted to hear, but at least I could give him the answer he needed: "Well, you might never walk. But, do you know what — I never walked, and it's OK."

Gosho rides with his mom on a specially designed seat.

I reminded him that's why he has a wheelchair and that he does a great job getting around. Instead of being disappointed, he quickly pivoted to telling me how fast he can go on the ramp at school. I felt blessed to be able to navigate that difficult question from my own wheelchair.

My experiences will also guide Gosho to become a strong advocate. He will attend every individualized education program meeting. He will push for change. He will speak up for himself. He will speak up for others. Growing up, I faced the same naysayers he will encounter, but he will be ready.

## BEAUTY, NOT TRAGEDY

I think some people view our family with pity, but they are missing the big, wonderful picture. Not long ago, Russel met with a shortsighted respiratory therapist. He explained that he didn't need much training on equipment for Gosho because his wife uses a lot of the same equipment. She responded, "Aw, how sad." Russel was taken aback.

I wasn't there, but I wish had been to tell her that the better response would have been, "How cool." How cool is it that a mom and son can share these experiences and learn together? How cool is it that his dad gets to be a part of that? How cool, and moreover how beautiful, is it that my son has wheels like mine? Q

*Katrina Gossett Kelly is a business litigation attorney at Faegre Baker Daniels in Indianapolis. She also performs improv at ComedySportz Indianapolis and serves as a disability advocate throughout Indiana. Her favorite job of all, however, is being mom to 7-year-old Gosho.*

*"When he is having a meltdown, I can keep him in place and get down to his level so that we can work through it. On those days, I am grateful that I can outrun my spirited little boy."*

### Get Personal

Quest loves to hear stories from around the MDA community. If you'd like to share your story with Quest, contact us at **quest@mdausa.org**.

## TALK TO YOUR DOCTOR IF YOU HAVE EXPERIENCED ANY OF THE FOLLOWING:

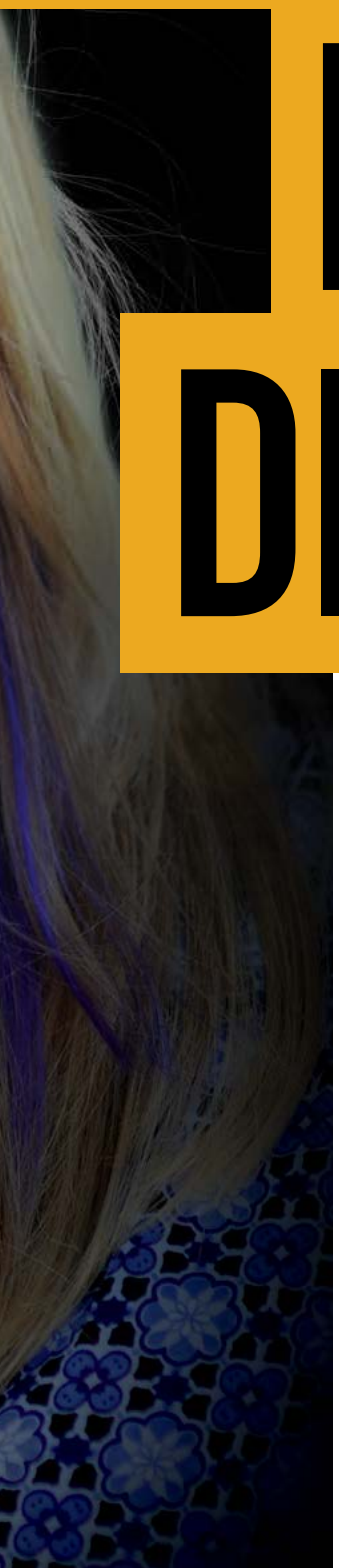
- Morning headaches
- Difficulty climbing stairs
- Trouble getting up from a chair
- Unexplained weight loss
- Difficulty breathing—especially while lying down
- Sleep disorders (sleep apnea)
- Excessive daytime fatigue

*Actual patient



SANOFI GENZYME

# IT COULD BE POMPE DISEASE



The average Pompe disease patient goes undiagnosed or misdiagnosed for up to **12 years**.

## Genetic Testing is Vital.

No two cases are the same. Check with your doctor about testing options for Pompe disease.

**Know the Pompe Spectrum**
at pompe.com

# WHAT'S the Toy STORY?

*Here's what's new in the world of adaptive play*

BY SHAILA WUNDERLICH

Barbies were a favorite toy of young Jessica Hetzel, despite the fact that the dolls looked nothing like her. It took until February 2019, when Jessica was 17, for Mattel to release a Barbie in a wheelchair. "I'm not playing with Barbies these days," says Hetzel, who has spinal muscular atrophy (SMA). "But I was excited to see they finally did it."

Adaptive toys have come a long way over the past decade — just not far enough. While technology has improved possibilities and performance, the selection is still slim and prices tend to be high. Even the gaming industry, whose technological potential is ripe for players with disabilities, has struggled to keep pace in this corner of the market.

What's the hold up? Adaptive toys require a high level of customization.

"Usually in this business we're doing everything by hand," says Denise Bandl, owner of Adaptive Tech Solutions, a company that makes adaptive toys and adapts standard toys. "There's a lot of labor involved." Bandl has observed some adaptive toy makers pulling out of the business over the past few years.

*"Usually in this business we're doing everything by hand. There's a lot of labor involved."*
*—Denise Bandl, owner of Adaptive Tech Solutions*

IMAGES: ISTOCK.COM/STUDIOCASPER; NOSUA

## ADAPTING STANDARD TOYS

In her work with children with neuromuscular diseases at the Children's Hospital of Michigan's MDA Care Center, clinical occupational therapist specialist (OT) Karen Glugla often chooses simple toys, like "rattles" made with Tic Tac boxes, balloons and pinwheels. "It has to be light enough for them to operate," she says. "Just a half-ounce can be the difference between a child being able to play or not."

Glugla's other go-to move is outfitting mainstream toys — and sometimes her patients' limbs — with Coflex bandages, foam-grip

*Karen Glugla often chooses simple toys, like "rattles" made with Tic Tac boxes, balloons and pinwheels.*

tubing or silicone adaptive aid to make toys easier to grip. "These are the same tools we'd use to assist in holding toothbrushes, forks, etc.," she says. "Our goal is for families to be able to take those materials with them when they leave and repeat the applications with toys in the home setting."

## ADAPTIVE TOYS

There are some great adaptive toys on the market today. Generally, the lighter, brighter and more stable a toy is, the better.

Toys with cause-and-effect functionality are also important.

"Cause and effect is crucial for brain development," says Bandl, who, in addition to being a certified assistive technology practitioner for her company, also works as a speech therapist. "Pressing this button in order to get this sound translates later to, 'Saying this word alerts my caregiver that I need help.'"

IMAGE: ISTOCK.COM/IGORGEIGER

## OUT OF THE BOX

**These four adaptive toys recently hit shelves.**

**SpinAgain.** This Fat Brain Toys bestseller is commonly used in clinical settings to test kids' reaching, sequencing, grasping and releasing abilities. The plastic base can be adjusted to wobble or stand immobile, depending on your child's needs.
***$30, fatbraintoys.com***

**Xbox Adaptive Controller.** Released in spring 2018, this customizable gaming controller can connect to a switch, button, mount or joystick device via USB and jack ports.
***$100, xbox.com***


**Fruit Friends 3-in-1 Toddler Toy.** This ultra-light, extra-bright silicone ensemble was developed by Fat Brain Toys in coordination with occupational therapists at Children's Mercy in Kansas City, Mo. The watermelon, orange and pear pieces stack, pull apart and wobble at the softest of touches.
***$30, fatbraintoys.com***

**Barbie Fashionistas Doll, Wheelchair.** Mattel's new Fashionista line includes dolls with disabilities, including this one in a wheelchair, complete with a ramp and extra joints for positioning in her chair.
***$20, amazon.com***

**Gears! Gears! Gears!** This 121-piece, snap-and-pull light-up toy is full of valuable cause-effect stimulation and comes with an adaptive switch to support play with siblings and peers of any ability.
***$80, adaptivetechsolutions.com***

Gaming remains a favorite activity for adolescents and teens with neuromuscular diseases. Gamer Erin Hawley reviews trends and products on her blog The Geeky Gimp (read "From Where I Sit" on page 30). "Most kids and even a lot of adults love video games," she says. "These days, you can see disabled players streaming on platforms like Twitch, which has made it something that kids can participate in on more equal ground."

Hawley, who has congenital muscular dystrophy (CMD), was psyched for 2018's release of the first-ever adaptive controller from Xbox. But she also relies on her own DIY adaptations to achieve perfect player positioning. "I use pillows to prop myself up, and my dad made me a table that allows me to rest my hands while playing to avoid fatigue."

Hetzel, too, loves gaming, and has found the Nintendo Switch, which is smaller than most controllers, works for her hands and fingers.

In any case, toys and activities should be fun.

"Play is the precursor to all the learning that comes after it, and it has to be fun to motivate play," Bandl says. "We try really hard to work in the just plain fun stuff along with the developmental toys — things like fart machines and water guns that shoot 25 feet. What kid doesn't want to play with those?" Q

*Shaila Wunderlich is a St. Louis-based writer with more than 20 years' experience in the publishing industry.*

*Jessica Hetzel and her caregiver Carley*

*"I'm not playing with Barbies these days. But I was excited to see they finally [made a doll in a wheelchair]."*
*—Jessica Hetzel*

# DON'T COUNT YOURSELF OUT

*By Ian*

At first glance, you wouldn't be able to tell that I have a rare neuromuscular disease that affects muscle strength and my ability to move. But when I was 14 years old, I was diagnosed with a progressive condition called spinal muscular atrophy (SMA), which meant I could eventually lose the ability to walk and perform daily activities.

"Fitting in" at 14 was everything to me – I felt like so much emphasis was put on physical attributes and I didn't want to be seen as different. But, I wasn't "normal," so I chose to hide. I continued to hide from people until in college a friend told me some girls were interested in me. "Me?" I thought. And that's when I realized, I was the only person discounting myself.

Following this realization, I started to put myself out there, gained confidence – and then during law school, I joined a band. While I have had difficulty putting on weight due to SMA, I decided to embrace my "skinny rocker vibe" look. We released a couple of albums and had the aspiration to perform professionally, but much to my disappointment, the band eventually separated. For me, performing was a passion and an energizer that I was not willing to give up, so I began to perform solo. I became a songwriter, singer, multi-instrumentalist, and actor, and continue to pursue this passion today. But getting to that point wasn't easy.

In my early thirties, I noticed my physical ability was declining – climbing stairs had always been difficult, but it became increasingly challenging. I was also struggling to lift my arms and play my guitar. Playing music is such a big part of my identity and I felt I would be losing myself if I could no longer play. On several occasions, I fell on stage when performing. Falling in front of a crowd was mortifying, but I decided to tell my audience why I had fallen in the hopes of helping to raise awareness of SMA. It was around that time that I found out about SPINRAZA® (nusinersen) on Facebook. There was a lot of information out there, but I didn't think much of it until I heard about it again at the Cure SMA Gala of Hope, and decided to see a neurologist shortly after. My neurologist told me that SPINRAZA could be an option for me and that we should start the process of getting me on treatment right away. My goal for starting SPINRAZA was to hopefully not lose my ability to walk. So far, not only have I maintained my ability to walk, I've also been able to maintain my ability to perform my music. Keep in mind that this is my personal experience and yours may be different.

*Individual results may vary based on several factors, including severity of disease, initiation of treatment, and duration of therapy.*

While SMA is supposed to take away a person's physical strength, it's given me the strength to not be afraid of standing out from the crowd and have the courage to share my diagnosis. And finally, it's given me the strength and ability to see that I should never count myself out.

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**INDICATION**

SPINRAZA® (nusinersen) is a prescription medicine used to treat spinal muscular atrophy (SMA) in pediatric and adult patients.

**IMPORTANT SAFETY INFORMATION**

**Increased risk of bleeding complications** has been observed after administration of similar medicines. Your healthcare provider should perform blood tests before you start treatment with SPINRAZA and before each dose to monitor for signs of these risks. Seek medical attention if unexpected bleeding occurs.

**Increased risk of kidney damage, including potentially fatal acute inflammation of the kidney,** has been observed after administration of similar medicines. Your healthcare provider should perform urine testing before you start treatment with SPINRAZA and before each dose to monitor for signs of this risk.

**The most common side effects of SPINRAZA include** lower respiratory infection, fever, constipation, headache, vomiting, back pain, and post-lumbar puncture syndrome.

These are not all of the possible side effects of SPINRAZA. Call your healthcare provider for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

**Before taking SPINRAZA,** tell your healthcare provider if you are pregnant or plan to become pregnant.

**For additional Important Safety Information, please see brief summary of full Prescribing Information on the next page.**

This information is not intended to replace discussions with your healthcare provider.

SPINRAZA® (nusinersen) injection 12 mg/5 mL

**Biogen is compensating Ian for sharing his story. This content has been reviewed for compliance with FDA guidelines. Please keep in mind these are Ian's experiences with SMA Type 3 and SPINRAZA, and others may have different experiences. For more information about SPINRAZA, visit SPINRAZA.com.**

# IMPORTANT FACTS ABOUT SPINRAZA® (nusinersen)

## USES
SPINRAZA is a prescription medicine used to treat spinal muscular atrophy (SMA) in pediatric and adult patients.

## WARNINGS
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SPINRAZA® (nusinersen) injection 12 mg/5 mL

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*Ray Sears (front left) at an Annapolis Fill the Boot kick-off event in 2018*

# Brother. Caregiver. Fundraiser.

## A Maryland Papa John's owner puts his pizzas to good use

Ray Sears wears many hats. He is the operating partner of 10 Papa John's franchises in Southern Maryland. He is a Grand Knight with the Annapolis Knights of Columbus council, and a caregiver to his brother Robert, 52, who lives with spinal muscular atrophy (SMA). Every night, Ray helps Robert with household tasks, meals and getting him in and out of bed.

Robert worked as a software technical specialist for the state of Maryland for 16 years. He retired in March because the disease had progressed to the point where he lost most of the strength in his upper body and extremities.

Seeing the toll muscle disease has taken on his brother, Ray, a lifelong MDA supporter, wanted to do more to help MDA find a cure.

So starting last year, Ray put his pizzas to good use in support of local Fill the Boot drives. He donated pies to the volunteering fire fighters and threw a pizza party for the department that raised the most funds. He also offered a Papa John's promo code to drive sales and donated a portion of those sales to MDA. Last year, Ray's efforts raised $1,006 for the Annapolis Fill the Boot campaign. These vital funds support MDA's innovations in science and care, which includes MDA Summer Camp.

"Little by little, we're plugging away to raise money for research," Ray says. "And if we can bring a summer smile to a kid going to camp, it's certainly worth it."

### Get Involved

From pizza parties to athletic events, there are many ways to raise money to help kids and adults living with neuromuscular disease. Learn more at **mda.org/yourway**.



# NALC Goes to Summer Camp

*Top fundraising representatives from the National Association of Letter Carriers (NALC) visited MDA's Camp Aranzazu in Rockport, Texas, in July. MDA is NALC's official charity, and, in addition to fundraising for MDA, letter carriers volunteer at MDA Summer Camps across the country.*

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# More Than a Game

*A woman with CMD finds community and purpose through gaming*

BY ERIN HAWLEY

*Erin Hawley (second from left) was on a panel at the 2018 Games for Change Festival.*

A typical weekend day for me starts off with my partner or caregiver feeding me breakfast (Cheerios with milk on the side) and ends with me falling asleep to an episode of "Cutthroat Kitchen." In between these moments, I game with friends from all over the country.

Gaming has been part of my life since the 1980s. I grew up playing on all the major gaming systems and had a constant slew of competitors in my brother and neighborhood friends. But as congenital muscular dystrophy (CMD) affected my body over the years, and as game controllers became more complex, I started having trouble keeping up with my peers. My hands grew weaker and my wrists contracted, making it harder to hold the controllers and reach certain buttons. My first gaming system, the Atari 2600, had one button to press and one joystick. With every new system that came out, developers added more buttons and combinations. Console gaming was totally cut off to me by the time the PlayStation 3 rolled out in the early 2000s with more than 14 buttons and two joysticks.

I switched to gaming on a PC using a keyboard and mouse; its customization makes it more accessible. But I still found that many games include inaccessible content, like having to press buttons quickly or hold multiple buttons down at once.

*"Console gaming was totally cut off to me by the time the PlayStation 3 rolled out in the early 2000s with more than 14 buttons and two joysticks."*

## SHARED FRUSTRATIONS

In 2012, I started a blog called The Geeky Gimp (**geekygimp.com**) to tackle these issues, as well as disability representation in other media. I also expressed myself on Twitter and Facebook, and discovered there were other disabled nerds going through the same frustrations.

Until that point, I didn't know many disabled people, let alone disabled people in gaming. I was mainstreamed in all my schooling and had little connection with disability organizations growing up. But with my blog, I was able to uncover this community that I belonged to and that I could lean on for support. I learned how game inaccessibility impacts individuals with disabilities other than my own.

Through my journey in PC gaming, I discovered Twitch, a streaming platform that allows users to share their gameplay with an audience and interact with viewers. I started watching other disabled gamers there, chatting with them about the latest titles, or just discussing our lives. I started my own channel on Twitch to create a safer place for anyone to come in, relax and enjoy the company. A lot of people outside of gaming think it's

silly and pointless to watch other people play games, but it's not about the games. It's about the communities we build around them, no different than a bunch of friends getting together to watch football.

## POSITIVE COMMUNITIES

Online communities are especially important for disabled people who may not be able to leave the house easily because they can't drive and public transportation is inaccessible. Or for people who have trouble being social when they have a mental illness. Or for those of us who deal with chronic pain and fatigue and find chatting from bed is easier than going out.

Online socializing gets lambasted in the media, and sometimes that critique is warranted; bullying, hate speech and other negatives are easily shareable on social media. But what many fail to consider are the exceptional, supportive and positive communities fostered by disabled people and their abled friends. If it wasn't for the friends I've made through gaming, I would lead a more isolated life.

The original goal of my blog and Twitch channel was to educate the gaming industry on accessibility and disability representation. That goal has been met tenfold because it led to my work consulting with tech leaders to make their products more accessible and inclusive, including playtesting and promoting the new Xbox Adaptive Controller. What I did not expect from my work was the true friendships I've built online. I did not expect to be playing on console again with my brother, and now his daughter.

Gaming and, more importantly, disability in gaming, has transformed my life for the better. Q

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*Erin Hawley, 35, lives in Keyport, New Jersey. She is a writer, accessibility consultant, sensitivity reader and digital content producer. She enjoys video and tabletop gaming, "Star Trek," and obsessing over Mariah Carey. You can find her @geekygimp on Twitter, @thegeekygimp on Instagram and vlogging as The Geeky Gimp on YouTube.*

*Erin Hawley uses the Xbox Adaptive Controller.*

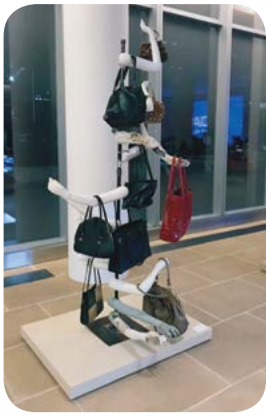
# Worth a Thousand Words

*An artist honors his mother's life and battle with ALS*

Karen Condron modeling, circa 1965

A display of Karen's shoes, representing the progression of the disease

An art installation featuring Karen's clothing



"Birds suddenly appear," showcasing Karen's purses

**Baltimore–based artist Jim Condron Jr.** was able to both celebrate his mother's life and promote MDA's mission to find a cure for amyotrophic lateral sclerosis (ALS) through his art exhibit "Close to you" at MDA's Wings Over Wall Street event in May in lower Manhattan.

The site of his art show was the same place his mother, Karen Condron, was honored with MDA's prestigious Wings Over Wall Street Spirit Award in 2015, recognizing her passion for life while battling ALS. Initially given three years to live when she was diagnosed with bulbar-onset ALS in 2010, she defied odds and lived until July 2018.

Condron created 10 art installations that showcased his mother's zest for life and put a face to the debilitating disease.

"She ran a business in Connecticut, she had six kids, she ran an average of five miles every day and she was a concert-level violinist. She also painted in watercolor and donated some of her paintings to MDA's silent auctions," says Condron, a professor at Towson University in Maryland.

One installation features a series of her shoes, beginning with high heels and gradually progressing to flats and then sneakers, showing the disease's evolution and the very tangible way it affected his mother.

In another, sweaters, jackets and purses stuffed with straw and yarrow represent her love of fashion. Other pieces feature family photos and memorabilia, like Condron's childhood cradle.

Condron hopes that the next stop for the exhibit will be an academic museum, where Karen's memory and MDA's mission can inspire others.

"I think my mom would have appreciated all the effort and work," Condron says. "I think she would have been really happy." Q

### Get Involved

There are many ways to support the MDA community. Learn more at **mda.org/get-involved**.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