

Quest

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FALL 2016

MDA[®]

For Strength,
Independence & Life

EMPOWERING FAMILIES WITH
INFORMATION AND INSPIRATION

INDEPENDENT PRO
A young professional
lands her dream job

SOCIAL SUPPORT
How to build a strong
support network

the heart of care

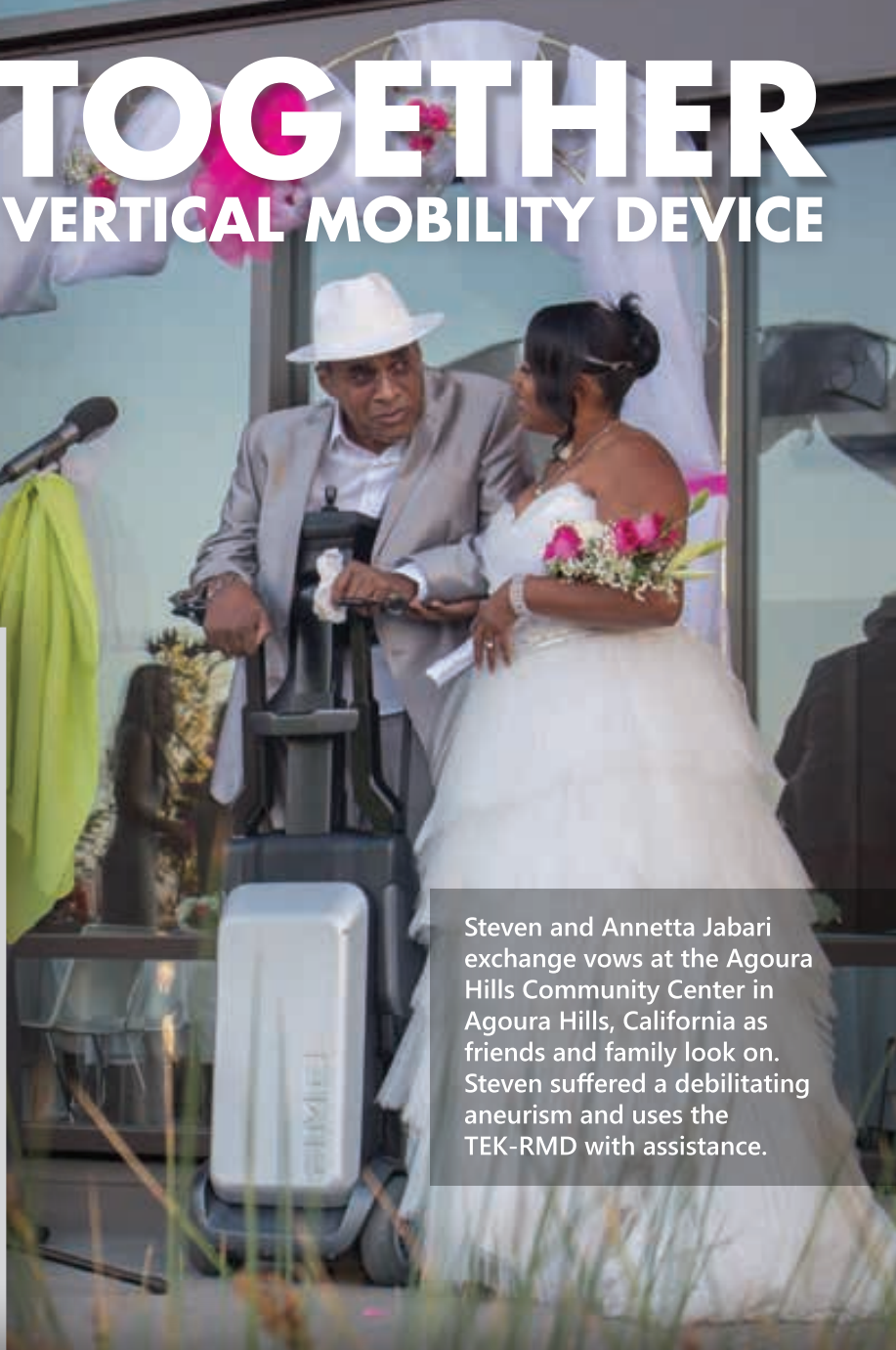
Couples keep romance alive when one
is the primary caregiver for the other



MOVING FORWARD
Celebrating the
accelerated approval of
eteplirsen (Exondys 51)

STAND TOGETHER

WITH THE TEK-RMD VERTICAL MOBILITY DEVICE



Steven and Annetta Jabari exchange vows at the Agoura Hills Community Center in Agoura Hills, California as friends and family look on. Steven suffered a debilitating aneurism and uses the TEK-RMD with assistance.

TAKE YOUR RELATIONSHIP TO A WHOLE NEW LEVEL

Sometimes, life's biggest moments demand that you stand. With the TEK-RMD, individuals with mobility limitations can stand tall and move gracefully when the moment matters most. The TEK allows you to traverse narrow spaces no regular wheelchair can access, and provides all the health benefits of standing in a simple, functional platform. With the TEK-RMD, you can drive with one hand while the other is free, and both hands are free when you are standing still, allowing you to cook, reach into cabinets, and even walk down the aisle.

With its incredibly narrow width, unassisted use, and exceptional versatility, the TEK makes your world more available. Call, visit us online, like us on Facebook, or watch us on YouTube to learn more about the revolutionary TEK-RMD, the award-winning Wijit lever drive and braking system for manual wheelchairs, the award-winning lineup of WHILL personal mobility devices and power wheelchairs, the easy-to-use ERGOtrans safe transfer harness, or any of the other extraordinary mobility and accessibility products available from Innovations Health.

MDA is leading the fight to free individuals – and the families who love them – from the harm of muscular dystrophy, ALS and related life-threatening diseases that take away physical strength, independence and life. We use our collective strength to help kids and adults live longer and grow stronger by finding research breakthroughs across diseases; caring for individuals from day one; and empowering families with services and support in hometowns across America.

Learn how you can fund cures, find care and champion the cause at mda.org.

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Landmark Decision Brings Hope for Continued Progress, Momentum in 2017

An important win for all individuals and families living with muscular dystrophy, ALS or one of the related neuromuscular diseases that



MDA fights occurred Sept. 19, 2016.

The U.S. Food & Drug Administration (FDA) granted accelerated approval to eteplirsen (brand name

Exondys 51) for the treatment of some forms of Duchenne muscular dystrophy. This is an outcome MDA dreamed of decades ago when we first invested in the research to identify the cause of DMD and then took the risk to be the first to invest in a breakthrough exon skipping research

project that led to development of this drug (see Progress Now, page 9). It is the first of what we know will be many disease-modifying drugs for DMD.

MDA is honored to have stood shoulder-to-shoulder with everyone who has fought to make this therapy a reality. Where would we be if not for years and years of telethons, fire fighter Fill the Boot campaigns, backyard carnivals, Lock-Ups and the countless generous individuals, sponsors and celebrities supporting our work — none more impactful or visible over the years than Jerry Lewis, of course? Jerry was thrilled with this news, and he encouraged us to keep fighting for our families. Even more heartwarming was the outpouring of support, love and encouragement we received from many of you.

Why is eteplirsen such a big deal, even for non-DMD families? We believe this success will encourage more researchers, biotech companies, pharmaceutical

companies and other industry partners to invest their time and resources in the development of drugs for neuromuscular diseases, leading to an expansion of the number of therapeutic options in the pipeline.

And yes, we are encouraged that the exon skipping strategy behind Exondys 51 may prove effective for numerous diseases, as evidenced by work underway for spinal muscular atrophy (SMA), late-onset Pompe disease (AMD), facioscapulohumeral dystrophy (FSHD) and others.

MDA has charted a bold course to double our spending on research toward drug development and clinical trials. Nearly 200 clinical trials are underway for diseases in our program, testing doz-

“By the next time I write, we hope to invest another \$6 million in new research projects pending the outcome of a strong fourth-quarter fundraising season.”

ens of promising therapies. We will continue to help pave the way and move other promising therapies toward the finish line. By the next

time I write, we hope to invest another \$6 million in new research projects pending the outcome of a strong fourth-quarter fundraising season.

As we continue to work together for strength, independence and life, MDA's sense of urgency has never been greater. It is with that sense of urgency that I continue to ask for your partnership, volunteerism and financial support to make our shared progress possible.

Best wishes for a wonderful holiday season from our MDA family to yours.

Steven M. Derks
President and CEO
Muscular Dystrophy Association

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fall 2016

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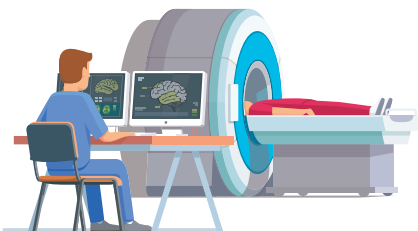
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MORE ONLINE

Read stories from around the MDA community – and share your own – on Strongly, the MDA blog. Visit strongly.mda.org to find personal stories from people living with neuromuscular diseases, research news, fun videos and more. If you're interested in sharing your story on Strongly, contact us at strongly@mdausa.org.

On the cover: Sarah Lidstrom and Scott Thomas



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Finishing Strong at MDA Muscle Walk

Every year in hometowns across America, MDA Muscle Walk participants of all ages and abilities make their way through a 1- to 3-mile wheelchair friendly course for a good cause. But this is more than a fundraising walk. Here are excerpts

from the Strongly blog about why people participate in this life-changing event.



“I took [Miguel] out of his wheelchair at the end of the course so he could walk over that finish line and to prove that we will get him through anything. To hear everyone cheering and clapping his great accomplishment was wonderful for him.”

– Stacy Santiago, mom of Miguel who has Duchenne muscular dystrophy

Join us at your local MDA Muscle Walk to bring strength to life. Visit mdamusclewalk.org to find a walk near you and register today.



“It’s just a really fun time. Getting to be at an event where you know that it’s helping you and others. It gives you such a good feeling.”

– Dana Parrot, who has limb-girdle muscular dystrophy



“Muscle Walk is a time for families affected by muscle-debilitating diseases to come together and unite in the fight for freedom and independence from our daily challenges.”

– MDA National Goodwill Ambassador Joe Akmakjian, who has spinal muscular atrophy



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A background image showing various pieces of laboratory glassware, including beakers and flasks, some containing colored liquids. The colors range from blue and purple to red and yellow. The glassware is slightly out of focus, creating a sense of depth and scientific activity.

progress now

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

Big-Picture Perspective

New MDA grants go to work

In August, MDA awarded nearly \$7 million in new research grants, supporting 25 new research projects around the world to accelerate treatments and cures. With 41 grants awarded earlier this year, MDA's investment in new neuromuscular disease research projects totals more than \$17 million for 2016.

The new research projects underway are expected to build learnings and create positive outcomes that cross disease borders and impact the greater neuromuscular disease landscape.

MDA's summer 2016 research grants reflect the organization's continued commitment to

double research spending on drug development and clinical trials by 2020.

Among the new research projects MDA is funding, highlights include the following:

- Scientists at the University of Florida in Gainesville will work to develop an antibody therapy as a potential treatment for ALS.
- Scientists at Sanford Burnham Prebys Medical Discovery Institute in La Jolla, Calif., will work to increase understanding about the complexity of cellular interactions that underlie disease progression in Duchenne muscular dystrophy.
- Scientists at the University of Washington in Seattle will work to increase understanding of the role of DUX4 protein in facioscapulohumeral muscular dystrophy.
- In collaboration with RaNA Therapeutics, scientists at the University of Alabama at Birmingham will investigate a new approach to treat Friedreich's ataxia.
- Scientists at Emory University in Atlanta will continue groundbreaking work to identify and characterize new gene defects that can cause limb-girdle muscular dystrophy.
- Scientists at Cornell University in New York will examine changes in metabolism that occur in mitochondrial myopathies and may be used as biomarkers of disease progression.
- Scientists at the University of Edinburgh, Scotland, will investigate how the mechanisms underlying spinal muscular atrophy influence how therapies work at different stages of the disease.

MDA currently is funding about 150 research projects worldwide.

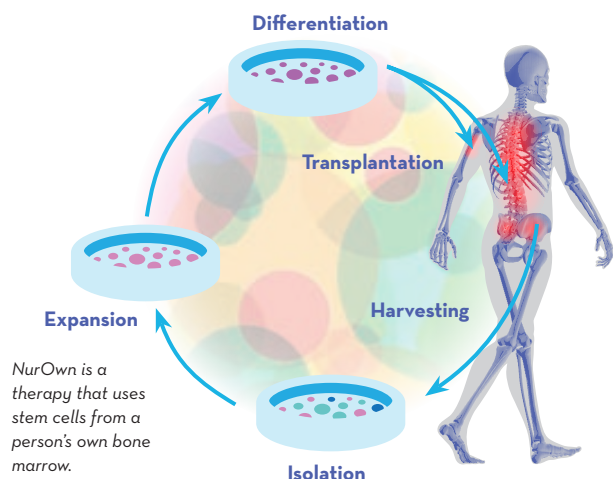


Read more about MDA grants at mda.org/gaag.

ALS (amyotrophic lateral sclerosis)

Stem Cells Show Positive Effects in ALS

Results suggest the treatment is safe and could possibly enhance overall function in people with ALS



Results from a phase 2 trial of NurOwn cell therapy conducted in the United States demonstrated that the experimental treatment was safe and could possibly slow decline in muscle and respiratory function in people with ALS.

NurOwn, under development by BrainStorm Cell Therapeutics, is a stem cell technology in which stem cells derived from the bone marrow of an individual with ALS are modified to secrete nerve-nourishing proteins before being transplanted back into the person's spinal cord and muscle.

Trial investigators found that transplantation of the cells was associated with improvements in the ALS Functional Rating Scale Revised (ALS-FRS-R), a measure of overall function for people with ALS. Participants who seemed to respond to the treatment also showed increases in nourishing factors after the treatment as well as decreases in inflammatory markers.

BrainStorm currently is planning a larger trial to evaluate repeat dosing. From 2010 to 2013, MDA supported Daniel Offen's work to develop stem cells engineered to produce neurotrophic factors as a therapy for ALS. Offen currently serves as chief scientific advisor at BrainStorm and is a co-inventor named in the NurOwn patent.

 For more about Brainstorm Cell Therapeutics' development of NurOwn to treat ALS, visit brainstorm-cell.com.



Natural History Study Sheds Light on Familial ALS

Information about the natural course of disease helps investigators accurately plan future trials

Results from an MDA-supported natural history study for familial (inherited) ALS caused by a mutation in the SOD1 gene have confirmed that a variation of the mutation called SOD1 A4V is associated with a more aggressive disease course when compared to non A4V SOD1 ALS.

Study investigators evaluated the medical records of 175 individuals with SOD1 gene mutations to characterize how SOD1 ALS progresses.

Although age of disease onset was approximately 50 years for all participants, results confirmed that the SOD1 A4V mutation predicts a rapid ALS disease course. Survival time in people with A4V SOD1 mutations averaged a little over one year, a significantly shorter amount of time than the nearly seven years associated with participants who had non A4V SOD1 mutations. The A4V variant also was associated with more rapid decline in ALS-FRS scores and measures of respiratory function.

The SOD1-specific data from the study is expected to help design and implement future clinical trials to test investigational therapies that aim to combat toxicity caused by the mutated SOD1 protein.

 To learn more about current ALS research efforts, visit mda.org/disease/ALS/research.

Charcot-Marie-Tooth disease (CMT)

MDA Partners with CMT Association

Alliance aims to make greater impact in the lives of individuals and families with CMT

In July, MDA and the Charcot-Marie-Tooth Association (CMTA) announced a partnership aimed at advancing CMT research, therapy development and clinical care. The partnership also aims to increase awareness and understanding about the disease by improving education for kids and adults affected by CMT, medical professionals and the public.

Goals include working to accelerate the search for new therapies and treatments for CMT, improving communication throughout the CMT community, and increasing collaboration among research and clinical investigators. MDA and CMTA plan to co-fund research and training grants; collaborate on infrastructure initiatives; coordinate advocacy efforts at the national, state and local levels; and work together on communication outreach to CMT families.

The alliance represents a key step in MDA's commitment to form strategic alliances with for-profit and nonprofit organizations.



 To read about MDA's CMT research efforts, see mda.org/five-questions-with-cmt-researchers-robert-burgess-and-scott-harper.

Dermatomyositis

PIONEER Trial

Participants sought for test of dermatomyositis drug



Investigators are seeking people to participate in PIONEER, a phase 2 clinical trial sponsored by Idera Pharmaceuticals, to evaluate the effect of the experimental drug IMO-8400 on skin lesions and muscle weakness in dermatomyositis.

While the cause of dermatomyositis is unclear in most cases,

it is known that ongoing inflammation triggered by the disease leads to destruction of muscle, skin and other tissues. IMO-8400 is designed to block the activation of specific Toll-like receptors (TLRs), which researchers hope might interrupt any potential


contribution these proteins may make to the harmful cycle of inflammation and tissue damage.

Trial participants will be randomly assigned to receive either placebo, or one of two doses of IMO-8400, delivered once weekly by subcutaneous (under-the-skin) injection for the duration of the study.

Participants must be 18-75 years old, have a diagnosis of dermatomyositis, have both active disease-related skin and muscle disease, and meet other eligibility criteria.

The study will be conducted at approximately 25 trial sites

in the United States, United Kingdom and Europe. Study participants may be able to receive support for travel associated with clinic appointments.

 For more information about this trial, including complete inclusion and exclusion criteria, please see ideraclinicaltrials.com, or visit ClinicalTrials.gov and enter NCT02612857 in the search box. For information on any individual trial site, please contact Kate Tighe, manager, patient advocacy, at patientinfo@iderapharma.com or 617-679-5541.

Duchenne muscular dystrophy (DMD)

FDA campus
in Maryland



Eteplirsen Granted Accelerated Approval

Landmark FDA decision makes eteplirsen the first drug approved to treat the underlying cause of DMD

Sarepta Therapeutics announced in September that the U.S. Food and Drug Administration (FDA) has granted accelerated approval of eteplirsen (brand name Exondys 51) to treat some forms of DMD.

Eteplirsen is an exon skipping drug designed to slow disease progression in DMD. Administered by intravenous infusion, it targets a section of genetic code called exon 51 in the dystrophin gene. It's estimated that 13 percent of boys with DMD could benefit from the treatment.

Eteplirsen was approved under the accelerated approval pathway, which provides earlier patient access to promising new drugs while the company conducts clinical trials to verify the predicted efficacy.

The FDA is requiring Sarepta to conduct a clinical trial to confirm eteplirsen's clinical benefit. The required study

will assess whether eteplirsen improves motor function in individuals with DMD. If it fails to verify clinical benefit, the FDA may withdraw approval of the drug.

MDA has been central to development of the exon skipping approach from the beginning in the 1990s, having funded foundational work upon which the strategy was built and extensive research into the strategy since that time. MDA supported the early development of eteplirsen via funding to Steve Wilton, currently at Murdoch University in Perth, Australia.



For the latest updates on eteplirsen, be sure to check mda.org/eteplirsen. For questions on whether you or your child may be eligible for treatment with eteplirsen and how to obtain the drug, please contact your physician.

Deflazacort Under Review

Approval would make deflazacort among the first FDA-approved treatments for DMD

The U.S. Food and Drug Administration (FDA) has accepted Marathon Pharmaceuticals' new drug applications (NDAs) for deflazacort for the treatment of DMD and granted priority review. A decision on the drug is anticipated in February 2017.

Deflazacort, a glucocorticoid, works as an anti-inflammatory and immunosuppressant. In a pivotal study involving 196 participants with DMD, results showed that treatment with the drug improved muscle strength.

FDA approval of the two NDAs, one for an immediate-release tablet form of the experimental treatment and one for an oral suspension formulation, would allow widespread access to deflazacort for kids and adults with DMD across the United States and make the drug among the first FDA-approved treatments for DMD. (Eteplirsen, approved by the FDA in September, is the first disease-modifying treatment approved to treat some forms of DMD.)



In the United States, the drug is considered an investigational therapy, as it has not been approved by the FDA for any disorder. Versions of deflazacort are approved in other countries, but not for DMD.

During the FDA review process, Marathon is making deflazacort available to people with DMD in the United States – at no cost – through Access DMD, an expanded access program operating under FDA authorization.

MDA has a long history of supporting research and clinical study into the effects of corticosteroids, including deflazacort, in DMD, with studies to determine the drug's mechanism of action, drug effects, side effects and best dosing regimen.



Learn more about AccessDMD, including a list of clinical sites participating in the program, by visiting AccessDMD.com or calling 844-800-4363.

Duchenne muscular dystrophy (DMD)

ImagingDMD Trial

DMD natural history study seeks participants



MRI is an imaging technique that allows doctors to look inside the body using a scanner that sends out a strong magnetic field and radio waves. The procedure is painless, noninvasive and does not use radiation.

Researchers are looking for boys 5–18 years old to participate in ImagingDMD, a natural history study designed to assess the potential of imaging techniques to monitor disease progression and serve as an outcome measure for clinical trials in DMD. Both healthy volunteers and individuals with DMD are invited to participate.

Study investigators will compare the muscles of boys with DMD to the muscles of healthy children of the same age and will monitor the progression of the disease in those with DMD. They will examine the relationship between muscle function and muscle composition by comparing muscle function testing results with images taken by MRI.

Trial participants will visit a study site yearly or in six-month intervals over a period of five to 10 years. Each clinical visit will include MRI and muscle function testing.

Trial sites are located in Florida, Pennsylvania and Oregon. Travel and hotel accommodation costs associated with clinic visits will be covered.

MDA has contributed previously to foundational work in the use of MRI in DMD and is supporting the ImagingDMD trial in part through a development grant to Rebecca Willcocks, adjunct research assistant scientist at the University of Florida.

For more information about this trial, including complete inclusion and exclusion criteria, please visit ClinicalTrials.gov and enter NCT01484678. If you or someone you know may be interested in participating in this trial, contact Dr. Claudia Senesac at 352-273-6453 or csenesac@phhp.ufl.edu.

Mitochondrial myopathy (MM)

MMPOWER Trial

Encouraging results for elamipretide in mitochondrial disease

Stealth BioTherapeutics reported encouraging results from the completed phase 2 MMPOWER clinical trial to test elamipretide in people with mitochondrial disease.

Elamipretide is an experimental drug designed to modify disease by helping to restore normal energy production in mitochondria (cellular energy factories) and decrease oxidative stress.

The MMPOWER trial, conducted in 36 individuals with genetically confirmed mitochondrial disease, ages 16–65, was designed to evaluate the safety, tolerability and efficacy of elamipretide to improve function in primary mitochondrial myopathy. Results showed that trial participants treated with the drug showed improvements on the six-minute walk test (6MWT), which measures the distance a person can walk on a flat, hard surface in a period of six minutes.

MDA has supported research investigating the role of oxidative stress and mitochondrial energy production in mitochondrial myopathies, as well as other potential therapies targeting these mechanisms.

Read more at strongly.mda.org. Search for “mitochondrial myopathy treatment.”

Myasthenia gravis (MG)

REGAIN Trial

Mixed results for MG drug


Results from the phase 3 REGAIN trial to test eculizumab (brand name Soliris) in people with refractory generalized myasthenia gravis (MG) show that the drug did not have a statistically significant effect on the primary endpoint – the Myasthenia Gravis Activities of Daily Living profile (MG-ADL), a patient-reported index of daily living assessment.

Alexion Pharmaceuticals, which is developing the drug for MG, reported that although the primary endpoint was missed, clinically meaningful improvements in several secondary measures were observed in those treated with eculizumab compared with those who received a placebo. ➤

Eculizumab, a terminal complement inhibitor, is thought to work in MG by inhibiting the complement pathway to prevent destruction of the neuromuscular junction.

Alexion reported that it is continuing to analyze the data and plans to discuss the results with regulatory agencies in the United States and Europe. Eculizumab is approved in the United States, European Union, Japan and other countries for the treatment of other conditions.

Although MDA was not involved in this trial, it has invested in previous research into complement inhibition as a therapeutic strategy for MG.

 **Read more at strongly.mda.org. Search for “myasthenia gravis drug.”**

Pompe disease (acid maltase deficiency, AMD)

Pompe Drug Program Discontinued

Participants can contact their physicians about treatment options

BioMarin Pharmaceutical has discontinued development of BMN 701, an enzyme replacement therapy (ERT) for the treatment of Pompe disease, or AMD.


In Pompe, a deficiency of an enzyme called acid alpha-glucosidase helps break down sugar stored in cells (glycogen). Without this enzyme, glycogen accumulates in cells and impairs their ability to function normally. The experimental drug BMN 701 (revelglucosidase alfa), a synthetic form of the enzyme, is designed to help cells metabolize accumulated glycogen.

BioMarin said it greatly appreciates the many individuals, families and physicians who have participated in its Pompe program and noted that the discontinuation is not based on

any concerns for participants' safety or drug effectiveness. Current trial participants are encouraged to talk with their physicians about potential treatment options going forward.

There are FDA-approved treatments available for Pompe, as well as ongoing clinical trials for other experimental drugs under development to treat the disease.

MDA had no direct involvement in the development of BMN 701 but has a long history of supporting ERT research in Pompe disease, including some of the earliest ERT feasibility studies and clinical trials.

 **Read more at strongly.mda.org. Search for “pompe drug.”**

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Spinal muscular atrophy (SMA)



AVXS-101 Receives Breakthrough Therapy Designation

Gene replacement therapy is designed to treat the cause of SMA

Gene therapy company AveXis announced in July that the FDA granted breakthrough therapy designation to AVXS-101 for the treatment of spinal muscular atrophy (SMA).

AVXS-101 is a clinical-stage gene replacement therapy designed to be a one-time treatment for type 1 SMA. Via a viral delivery method, the therapy delivers the needed SMN1 gene, which is defective in SMA. The therapy currently is being tested in infants with type 1 SMA in a phase 1 clinical trial.

Breakthrough therapy designation is granted by the FDA to experimental therapies for which preliminary clinical evidence indicates the drug may demonstrate substantial improvement over existing therapies for a serious, life-threatening condition. FDA expedites the development and review of drugs with the breakthrough therapy designation.

MDA has supported foundational research to develop gene therapy as a treatment strategy for SMA and related neuromuscular diseases.

 To learn more about the development of AVXS-101 to treat SMA, visit avexis.com.

Nusinersen Shows Promise

SMA drug to be made available to eligible babies with infantile-onset SMA



Biogen plans to open a global expanded access program for babies with infantile-onset SMA in the coming months. The program will allow eligible infants to receive treatment with nusinersen prior to approval of the drug by the FDA.

Encouraging early results from a late-stage clinical trial for nusinersen, under development by Biogen and Ionis Pharmaceuticals to treat SMA, prompted investigators to stop the trial and transition all participants into an open-label trial called SHINE, in which all infants will receive the drug.

Nusinersen is a disease-modifying antisense drug designed to target genetic instructions in RNA (a chemical cousin to DNA). It aims to increase production of the needed SMN protein.

Interim analysis from the phase 3 ENDEAR trial, conducted in 120 infants with type 1 SMA, found that infants receiving nusinersen experienced a statistically significant improvement in the achievement of motor milestones – such as being able to sit or roll over unassisted – compared to those who did not receive treatment.

In September, Biogen filed for regulatory approval for nusinersen as a treatment for SMA in the United States. If approved, nusinersen would be the first disease-modifying drug approved for the treatment of SMA.

MDA has funded foundational work in SMA and supported Adrian Krainer at Cold Spring Harbor Laboratory in New York for early-stage development of nusinersen.

 Read more about nusinersen at strongly.mda.org. Search for “nusinersen trial.”

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Living Social

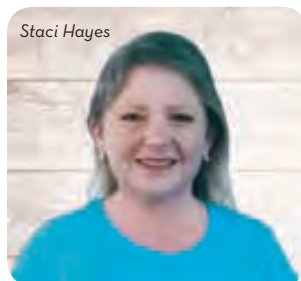
Building and maintaining a strong social support system helps you live well with neuromuscular disease

BY BARBARA & JIM TWARDOWSKI, RN



“When you have a diagnosis, you feel like you’re the only one going through something.”
— Staci Hayes

Months after Staci Hayes gave birth to her daughter, she began to suspect her extreme exhaustion was not normal. “How I felt went beyond [the idea that] I have a newborn and I’m tired,” says Hayes. After



consulting with doctors and undergoing a range of testing, Hayes received a diagnosis of myasthenia gravis (MG). At the age of 40, every aspect of her life changed. Her 20-year career as a nurse ended, her marriage fell apart, and she became a single parent with a 3-year-old child.

Unable to maintain an active life, the only places Hayes went were to the pharmacy and doctors’ offices. Her lack of energy made it difficult to venture away from home for activities. She couldn’t make plans because she never knew

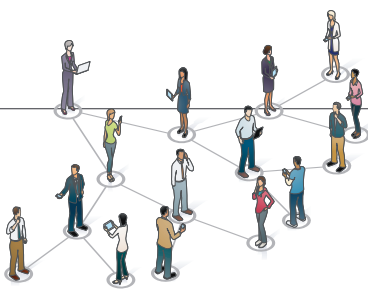
if it would be a good day or a bad day. Her friends became frustrated. They didn’t know what they could do to help, so they just stopped calling.

Everything Hayes dealt with was made more difficult by her social isolation.

THE IMPORTANCE OF SOCIAL SUPPORT

“Innumerable studies show one of the major factors in psychological well-being and physical health is that social support systems make a huge difference in a person’s life,” says Jay LeBow, Ph.D., a psychologist at The Family Institute at Northwestern University and a clinical professor at Northwestern University. A social network gives one a sense of belonging. This informal collection of friends, family, neighbors and co-workers are the people you turn to when you need a sounding board or a helping hand.

People who are living with a chronic condition such as a neuromuscular disease are likely to experience higher stress levels. Building and maintaining an informal social network is a valuable life skill that can reduce stress. Some people are naturally more outgoing and easily engage with new acquaintances while others have to make



GROW YOUR NETWORK

Connecting with others is one of the best ways to handle stress and avoid social isolation. Here are tips for building and maintaining a robust social support system.

Join in. Organizations that are welcoming to newcomers include places of worship, senior centers, libraries and community centers.

Volunteer. Giving will make you feel happier. Working with others, even for a short amount of time, can help you connect with your community and meet like-minded individuals.

Make an effort. Relationships need to be nurtured. Schedule time for get-togethers and chats – even if they are on the phone or online – to maintain friendships. Be an active listener.

Take a class. Learn a foreign language, make jewelry or write your memoir. Following an interest will help you meet others. Give friendships time to develop.

Open your home. If it is difficult for you to get out of the house, invite others to your home. Host the knitting group, book club or neighborhood watch meeting.

Cast a wide net. Be open to meeting all kinds of people.

Show appreciation. Nurture the relationships in your life by expressing gratitude. Take the time to say “thank you.” Be supportive of others when they need you. Successful relationships are reciprocal.

Find a support group. Look for a local or online group. If there isn’t one for your disease, consider one that is similar. For example, those with myasthenia gravis (MG) might find a group for people with autoimmune diseases or rheumatoid arthritis. MDA offers disease-specific support groups, as well as groups for caregivers, parents, young adults and more.

a conscious effort to forge relationships that provide emotional support.

“With many illnesses, social support can help the person feel better and even affect how the disease progresses,” says Dr. LeBow. Having a strong social support network can positively influence a person’s mood and ward off depression.

SEARCHING FOR HELP

Stress makes the need for a social network more acute, but it also can make it harder to build and maintain that network.

While a social support network is informal, a support group is an organized gathering of people with a common concern. The group may include people with a specific

disease or challenge, as well as family members, caregivers and a facilitator who leads group discussions. There is a sense of fellowship as individuals share information and experiences.

When chronic conditions make it hard to be social, finding support groups can be a way to build that essential social support system. That’s what Hayes discovered.

“The internet was a life-saver,” says Hayes, who found chronic disease online support groups. Chatting with others who had the same condition enabled her to cope. “When you have a diagnosis, you feel like you’re the only one going through something.”

Hayes did not want other people who had MG to feel alone, so she created the Inner Strength Myasthenia Gravis Support Group in central Alabama. “I may not be able to nurse anymore, but I can still care for and help people,” she says. The support group joined forces with MDA, and Hayes facilitates the monthly meetings, which often include talks from experts in the field.

“Starting the support group gave me an MDA family and a whole new social support system,” says Hayes. [Q](#)

Barbara Twardowski has Charcot-Marie-Tooth (CMT) disease and uses a power wheelchair. Jim, her husband, is a registered nurse. The couple lives in Louisiana and writes about accessible travel, assistive technology and related issues.

You’re Not Alone in Your Journey

MDA is here every step of the way, ready to provide resources and guidance, support groups and ways to connect with others online. Contact your local MDA office to find support groups and other events in your community at mda.org. (Type your ZIP code into the “Find MDA in Your Community” box.) Be sure to connect with MDA on Facebook (MDANational) and Twitter (@MDAnews).

THE

WEART



When one partner is the primary caregiver for the other, a little creativity and a lot of communication help keep the fire burning

of

CARE

BY DONNA SHRYER



When a couple vows to share their lives — whether or not they express that commitment before an authorized officiant — there’s a traditional phrase that holds particular pertinence when one partner is both mate and primary caregiver for the other. It’s the line about loving one another for better, for worse, for richer, for poorer, in sickness and in health.

When caring for a partner with a neuromuscular disease, those words are often put to the test, especially in terms of intimacy. On the other hand, if both partners use the challenges to spark a little more laughter, sincere communication and resourceful creativity, these challenges can ignite and sustain a deeply intimate love.



“I had so many insecurities about what I could no longer do. For me, the solution was to stop focusing on what everyone calls a ‘normal romantic relationship’ and focus instead on what works for my partnership with Sarah.”

— Scott Thomas

THE BIG PICTURE

The first step, suggests Rebecca Axline, a supervisory licensed clinical social worker at Houston Methodist Neurological Institute, site of an MDA ALS Care Center, is for both partners to realize that they’re much like any other couple. “When one partner has a neuromuscular disease and the other partner is the caregiver, there will be role changes,” she says. “But that doesn’t make you different; that makes you normal.”

Couples deal with role changes all the time. The birth of a child, caring for an elderly parent, clashing work hours — all these situations change each partner’s role in a relationship and often leave little time for romantic intimacy. “I encourage individuals to recognize this and look at role changes as something that’s normal in every relationship,” Axline says.

“I’m not minimizing any aspect of neuromuscular disease, but understanding how ‘normal’ you and your partner are can be very freeing,” she adds. “Once you realize that

everyone gets the rug pulled out from under them at some point or another, both partners are in a much better position to find creative, out-of-the-box solutions that can bring back the intimacy they crave.”

THE NEW NORMAL

The second step, particularly when one partner assumes the role of primary caregiver, is to redefine “normal” intimacy.

Scott Thomas, 27, received a diagnosis of amyotrophic lateral sclerosis (ALS) in 2012. Thomas and his 24-year-old fiancée, Sarah Lidstrom, who is Thomas’ primary caregiver, see their situation as a “new normal.”

“Feeling romantic was very difficult early on,” Thomas says. “I had so many insecurities about what I could no longer do. For me, the solution was to stop focusing on what everyone calls a ‘normal romantic relationship’ and focus instead on what works for my partnership with Sarah. Once I did that, I learned that what Sarah needs most is to feel loved and cared for. That I can do!”

Expanding on Thomas' outlook, Lidstrom emphasizes how important it is to accept that things will be different — but that doesn't have to be negative. "The best thing we did for our relationship was to accept the fact that it was not going to be like when we first met or like the relationships we see around us. That doesn't make us better or worse, just different," she says.

"We are here for each other through anything life throws at us, and we are able to see the good that has come from all of this. We developed a passion for traveling and adventure; we make each other laugh like no one else can; and we realize that this hurdle has transformed us into the

people we want to be. I don't think we would be able to say all this had it not been for this disease process."

As for finding intimacy in a relationship, Lidstrom adds, "If close contact is an important piece of intimacy for you, then find time for that during transfers or spend time lying next to each other in bed. If kissing is important, then kiss. It may be a kiss on the cheek, but that kiss can mean more than any other kiss you've ever had in your life. If sex is an important part of your relationship, be creative and figure



Dianne & Rickie

out how to have sex. Just know that intimate experiences will be different. That doesn't make the experiences less important. They may be more important, because you have to be a little more clever to get what you both need."

“After 41 years, we're very comfortable with each other, and we're best friends.”

– Dianne Bauer

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“We have a level of communication that goes beyond anything any disease can throw at us.”

— Ronald Thompson

CARE FOR THE CAREGIVER

When you're the primary caregiver for a loved one, there's no denying that the experience ranks right up there with life's most meaningful, giving and often uplifting acts. At the same time, though, caregiving can be physically exhausting and emotionally draining.

Here are some tips on staying happy and healthy that benefit everyone involved.

1. Laugh it up

When caring for a loved one with neuromuscular disease, a little levity may help the caregiver get through what might otherwise be a tense situation. Studies suggest that laughing may turn on the brain's endorphins — the same pain-relieving chemicals released in response to exercise, excitement and love, among others. Research also indicates that a hearty laugh may relieve physical tension

and stress for up to 45 minutes after the laughter ends.

Find ways to exercise your sense of humor both with and without your loved one — watch comedies, share jokes with friends, or practice laughter yoga, which combines deep breathing

and calming movement with interactive laughter exercises.

2. Indulge in self-indulgence

Ronald Thompson, the primary caregiver for his partner Brian Fender, who has ALS, brings up a strong argument in favor of caregivers tending to their own physical, mental and emotional needs. “It's tough to feel intimate when I've barely slept and put off getting a haircut, bathing or even getting dressed. And I'm not talking about sex. Cuddling, kissing, holding hands or resting in bed next to Brian — these are intimate moments, too. And it helps when I feel attractive.”

So as a caregiver, Thompson's advice is to take care of your physical health, but never underestimate the importance of “feeling pretty.”

3. Find outside help

“When I talk to someone caring for a partner with neuromuscular disease, I stress trying to hire private help as early as possible and often as possible,” explains Rebecca Axline, a supervisory licensed clinical social worker at Houston Methodist Neurological Institute. To accomplish the feat, which can admittedly be challenging, Axline advises asking your MDA Care Center team, including the social worker, neurologist, nurse and family support and clinical care coordinator, about what resources are available. Another resource is your local MDA office. To help cover out-of-pocket expenses, some couples put together GoFundMe accounts or ask their place of worship to host a fundraiser.



DEFINING INTIMACY

A key to taking Lidstrom's advice is to look at the true meaning of intimacy. “In our culture, we often equate intimacy with sex,” says Axline. “In reality, aspects of intimacy include

intellectual and emotional connections.”

Axline adds, “When I talk to couples, I explain how intimacy uses all your senses — touch, smell and vision — as well as emotions, like caring and warmth. Intimacy is so

much bigger than what we often see it as.”

Intimacy can be a gentle touch: Rickie, 62, and Dianne Bauer, 59, have been happily married for 41 years. After Rickie was diagnosed with ALS in 2012, the couple

learned that intimacy is much bigger than they originally thought. "I think we're luckier than some couples," Dianne says. "After 41 years, we're very comfortable with each other, and we're best friends. So, yes, our intimate relationship has changed, but we naturally started communicating our love in new ways — warm hugs, holding hands, laughter and lots of little pecks on the cheek."

Intimacy can be a knowing glance: Some of life's most intimate moments follow a deep, meaningful connection, whether verbal or nonverbal. If anyone understands that idea, it's Ronald Thompson, 52, the primary caregiver for his partner Brian Fender, 48, who was diagnosed with ALS

in 2011. Today, Fender has a tracheostomy to breathe, and he can no longer talk, walk, sit up, type or eat on his own. "We've been together for 24 years — 19 years before Brian's diagnosis — so we have a level of communication that goes beyond anything any disease can throw at us," Thompson says.

Without the power of speech, Thompson has learned to read Fender's expressions. "He has a certain smile when he's feeling frisky. It's a glimmer in his eyes that's impossible to resist. He's just adorable. And his expressions tell me how he feels about household decisions, too, which he is always part of. He's never excluded. That's what keeps

"When I talk to couples, I explain how intimacy uses all your senses — touch, smell and vision — as well as emotions, like caring and warmth. Intimacy is so much bigger than what we often see it as."

— Rebecca Axline

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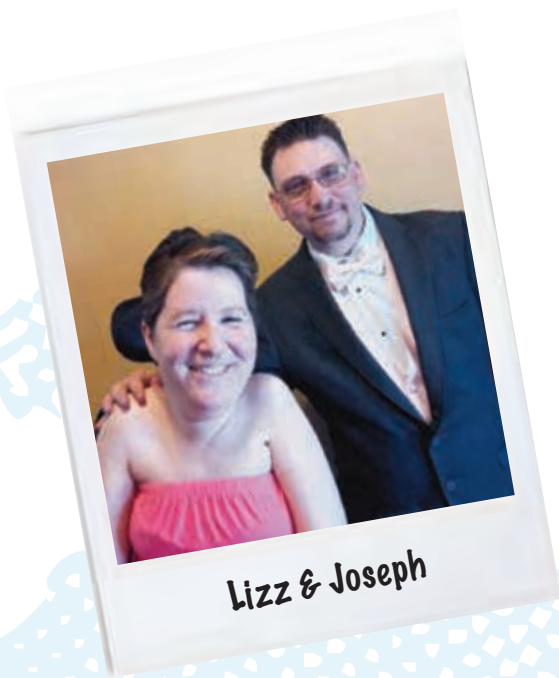
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“I can get angry and depressed, or I can laugh it off and move on. ... Overall, we think it’s a waste of time to spend life being angry and fighting.”

– Lizz Barna

our relationship genuine and intimate. He’s not my patient; he’s an intimate member of our family,” Thompson says.

Intimacy can be open, heartfelt honesty: According to Lizz Barna and Joseph McCabe, honest communication — no holds barred — is a powerful element of intimacy. “Being able to completely trust someone is amazingly intimate,” Barna says.

Barna has lived with spinal muscular atrophy (SMA) for nearly 33 years, since she was diagnosed at 18 months old.

Her partner and primary caregiver is Joseph McCabe, now 44, and as he puts it, “I’m more in love with Lizz than ever.”

A profound part of that love stems from the fact that Barna was always honest about her disease. “I told Joe everything from day one,” she says. Both agree that knowing the facts allowed McCabe to make a conscious decision. Was he up to caring for this woman through better and worse, sickness and health? McCabe chose to stay by Barna’s side, which was, to say the least, an intimate choice, and it continues to drive their close, loving relationship. In fact, today the couple has a wonderful 11-year-old son.

Intimacy can be spontaneous giggles and grins: “Laughter is very important; it keeps us strong,” Dianne says, adding that Rickie likes to set up moments of levity by doing something unexpected, like making a fuss about the clothing his wife sets out for him in the morning. Rickie is not and has never been a fashionista, so this prompts Dianne to reply, “Are you kidding me?” And of course, he is kidding, which is apparent as Dianne watches Rickie sit there “smiling and giggling.”

Barna and McCabe couldn’t agree more. “You have to have a sense of humor — keep things light,” Barna insists. Whether it’s a major issue, such as when a piece of essential equipment goes awry, or a general inconvenience, like when Barna can’t reach her much-loved Snapple, she says there are two choices. “I can get angry

Thank You, Caregivers!

MDA would like to recognize all the family caregivers whose tireless support and devotion help empower people living with neuromuscular diseases and ensure they receive the care they need. From MDA, we say thank you.

MDA is here to provide help, support and hope to families whenever, wherever they need us. To find caregiver resources, visit mda.org/services/caregiver-resources.

and depressed, or I can laugh it off and move on. Obviously, there are times when Joe and I have to discuss serious matters, but overall, we think it’s a waste of time to spend life being angry and fighting.”

MAKING IT WORK

It turns out that neuromuscular disease cannot delete intimacy between two lovers. It may take another form, but it’s always there if both partners are willing to work at it. As Dianne says, “Rickie and I worked on our marriage every day prior to his diagnosis, and we still work at it — we just work at it differently.”

Facing role changes is a normal part of every couple’s union. And when done with love, patience and creativity, it may very well turn out to be one of the better, richer, healthier times. **Q**

Donna Shryer is a freelance writer in Chicago.



LEG WEAKNESS

TROUBLE BREATHING

TROUBLE CLIMBING STAIRS

EXCESSIVE FATIGUE

TROUBLE WALKING

DAYTIME SLEEPINESS

DIFFICULTY SWALLOWING

DIFFICULTY REACHING OVERHEAD

MORNING HEADACHES

DIFFICULTY GETTING UP OUT OF A CHAIR

FALL EASILY

IT'S NOT IN YOUR HEAD

IT'S IN YOUR MUSCLES

If these symptoms seem familiar, you may want to learn about Pompe disease¹⁻³

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References: **1.** Kishnani PS, Steiner RD, Bali D, et al. Pompe disease diagnosis and management guideline. *Genet Med.* 2006;8(5):267-288. **2.** American Association of Neuromuscular & Electrodagnostic Medicine. Diagnostic criteria for late-onset (childhood and adult) Pompe disease. *Muscle Nerve.* 2009;40(1):149-160. **3.** van der Beek NA, de Vries JM, Hagemans MLC, et al. Clinical features and predictors for disease natural progression in adults with Pompe disease: a nationwide prospective observational study. *Orphanet J Rare Dis.* 2012;7:88.

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INDEPENDENT

*Lauren Carter
loves life
as a young
professional*

BY CHARMAINE DYMOND

Like many of us, 25-year-old Lauren Carter's path to her chosen career took a few turns along the way.

"I was definitely one of those students all throughout high school who was constantly changing my mind about what I wanted to do with my life," Carter says. She considered becoming a doctor or a lawyer, as well as a cook, even though she admits to being terrible at cooking.

As she entered her last year of high school, broadcast journalism appeared on her radar. Carter, who was diagnosed with spinal muscular atrophy (SMA) as a baby, served as MDA National Goodwill Ambassador when she was 8 years old. This experience included a lot of time in front of cameras — perfect practice for a career in broadcasting. "After everything that I had done with MDA, I knew how to be on camera. I had experience with it, and it was fun when I was younger."





Above, Lauren and her mom on a trip to Paris in 2009. Right, Lauren and co-worker Delia Krimmel at a holiday party in 2015.



Turns out, being in front of the camera as an adult wasn't as much fun for Carter. A media technology course during high school helped her confirm that she wanted to work in communications, so she switched her focus to public relations. After graduating from the University of Houston in 2013, she landed a job in her field, which she describes as "a dream come true."

FINDING HER VOICE

Carter always knew she wanted to pursue higher education, but she admits she was concerned about how her disability would affect her in college, especially when she decided to attend without the personal care assistant who

had been with her throughout high school.

"I had the most wonderful aide," Carter says. "Gail made sure I had everything I needed, and then she let me be a regular teenager. She was kind of like my school mom."

But when it came time for college, Carter decided to go it alone. Her university offered disability services, so she knew she would be able to seek assistance if she needed it. "I decided that I wanted to try it independently and see how it went. It was great. I didn't use an aide the entire time."

Going to college without a personal care assistant meant that Carter had to learn to be assertive and deal with any problems herself. "Practically from birth until I graduated high school, I had always had a great support system. They did the majority of the work for me," she says. "It was finally in college that I was able to be independent and do things on my own. It was where I grew up and where I was able to find my voice and learn how much power I have as an independent person."

FINANCIALLY SMART

While in college, Carter lived at home to keep costs down. This decision, along with the scholarships she received, meant she was able to graduate without debt.

Now that she's working full time, the idea of moving out is on her mind. "We want to be young professionals living in our cute little over-priced apartments," says Carter, adding that she's itching to decorate her own place.

CITGO: 30 YEARS OF MAKING A DIFFERENCE FOR MDA FAMILIES

Since 1986, CITGO Petroleum Corp. and its employees have supported MDA's mission to help kids and adults with neuromuscular diseases in hometowns across America live longer and grow stronger. Based in Houston, CITGO is a refiner, transporter and marketer of transportation fuels, lubricants, petrochemicals and other industrial products.

CITGO's 30-year commitment to MDA's lifesaving cause has helped improve the lives of countless children and adults living with neuromuscular diseases. As MDA's largest corporate sponsor, CITGO has set the standard in fueling the fight to find treatments and cures for neuromuscular diseases.

"Working with MDA is a great way for our nearly 5,300 locally owned stations to get involved in their communities, and on a corporate level, our employees can rally around and support this important cause," says Rick Esser, CITGO's vice president of supply and marketing.

Together with CITGO, MDA is working to provide families with much-needed hope, answers and support. Because of CITGO's unparalleled support, MDA is bringing strength, independence and life to families by finding research breakthroughs across diseases; caring for individuals and families from day one; and empowering families with services and support, including equipment assistance, support groups, MDA Summer Camp for kids and more.

A SNAPSHOT OF CITGO'S SUPPORT FOR MDA FAMILIES

- **\$200+ million** raised since 1986
- **\$15 million** raised in 2016
- **1.6 million** MDA Shamrocks sold this year at 5,300 locally owned CITGO stations across the country
- **60 special** MDA fundraising events sponsored each year by CITGO

But Carter is determined to be financially smart about her future. "I'm going to need some type of personal care assistant or nurse to help me with the big things, like helping me transfer from my bed to my wheelchair. That has a cost associated with it," she says. "Because of my disability, I really can't make impulsive decisions. I thought about it on my own, and then I discussed it with my parents. They allowed me the opportunity to stay at home so that I could save for my future."

FULL CIRCLE

Carter has worked for CITGO's corporate communications team since May 2015. She says the experience has been fantastic because of CITGO's longtime support of MDA families across the country. "It's very strange because I first met people with CITGO when I was National Goodwill Ambassador back in 2000. I don't know if it's full circle or if it's just an interesting interconnection, but it's great."

Understanding that not all graduates find employment in their chosen field, Carter says she feels very fortunate to have a job where she can apply what she learned in college. "It sounds very corny saying it's a dream come true, but it kind of is."

This dream is not without its challenges, and for Carter, the biggest is fatigue. "I do get tired a lot because of my disability. It may take me longer to do things, so I just have to be conscious of time management and making

sure that I'm not overloading myself."

Her manager and co-workers are very supportive and understanding, she says. "I think that's another amazing thing about working here [at CITGO] is that I work with people who do understand what I'm going through. If there is something that I need help with, I can go to them and they'll understand."

Carter has big plans for the future, including a mile-long travel wish list and dreams of pursuing her MBA. "There are so many places I want to go, causes I want to support and things I want to learn. I hope that I can continue sharing my story and showing people that a physical

disability does not define you and encouraging them to never give up on what they want to achieve. A physical disability doesn't mean, "No, you can't do something," it just means you find a different way to do it. **Q**

Charmaine Dymond is a freelance writer based in Halifax, Canada.

Follow Along

Be sure to follow the MDA Strongly blog at strongly.mda.org for more stories of individuals and families with neuromuscular diseases who are defying their limits every day and challenging the nation to live unlimited.

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Should I RUN

*Is participating in a clinical trial
right for me?*



Clinical trials are research studies conducted to determine whether a medical strategy, treatment or device is safe and effective for use in humans. They can test medical products including drugs and devices, help discover more information about a disease, or evaluate procedures or behavioral changes such as diet or exercise.

While a clinical trial is the cornerstone of research, it is important for individuals to carefully consider participation in any trial prior to making a commitment.

PREPARATION TIPS

What questions should I ask myself before deciding to participate in a clinical trial?

- Why do I want to participate in the clinical trial?
- What are my goals and expectations if I were to be selected?
- How could this impact me if I do participate in the trial? How will this impact me if I do not participate?
- Have I weighed the benefits versus the risks?

What questions should I ask my doctor or study coordinator before deciding to participate in a trial?

About the trial:

- What is being studied?
- Why do researchers believe the intervention being tested might be effective? Why might it not be effective? Has it been tested before?
- What are the possible interventions that I might receive during the trial?
- How will it be determined which interventions I receive (for example, by chance)?
- How will I know which intervention I receive during the trial? Will I know? Will members of the research team know?
- Will I get a placebo?
- Can my doctor or I pick what group I am a part of?
- Will I know which group I am in?
- Are there risks?
- How do the possible risks, side effects and benefits

of this trial compare with those of my current treatment?

- What are my options if I am injured during the study?
- Who will oversee my medical care while I am in the trial?
- Will my information be kept confidential?

About my role:

- What will I have to do?
- Will I have to stop taking any of my current medications or supplements to participate in this trial?
- How often will I have to visit the hospital or Care Center?
- What tests and procedures are involved?
- Will hospitalization be required?
- How long will the study last?
- Will I be allowed to participate in another clinical trial if another one comes up during this trial? Do you recommend another clinical trial for me over this one?
- Who will pay for my participation?
- Will I be reimbursed for other expenses?

About the post-trial period:

- What type of long-term follow-up care is part of this trial?
- If I benefit from the intervention, will I be allowed to continue receiving it after the trial ends?
- Will results of the study be provided to me?

WHY ARE TRIALS IMPORTANT? WHY DO WE NEED THEM?

Clinical trials play a key role in the development of new treatments for diseases. They may be used to detect, diagnose and reduce the risk of disease. Clinical trials provide important treatment information, including what works well and what might cause harm.

WHO CAN PARTICIPATE IN A CLINICAL TRIAL?

People of all ages, races, ethnicities and genders can participate in trials. Each study has its own inclusion and exclusion criteria to determine who is an appropriate candidate.

WHY PARTICIPATE IN A CLINICAL TRIAL?

Participating in a trial helps advance research. It can inspire feelings of empowerment for the participant, who may see it as an option to fight back against a disease.

HOW DOES PARTICIPATING IN A CLINICAL TRIAL HELP ME?

Participation in a clinical trial may accelerate the development of treatments, increase knowledge of a particular disease or even establish a cure. Results from these studies can improve patient care by providing information about the benefits and risks of products or interventions. Some trials may provide participants with direct medical benefits, while others do not.

WHAT ARE MY RIGHTS AS A CLINICAL TRIAL PARTICIPANT?

The trial specifics and what

Tell study investigators about any possible side effects, as well as any hospital, ER and outpatient clinic visits. Do not conduct your own experiments, as they may invalidate study results.

will be expected of a participant will be explained to you prior to providing consent. You will have every opportunity to ask questions even after you have consented to participate.

WHAT ARE MY RESPONSIBILITIES AS A PARTICIPANT?

Comply with the study protocol (visits, treatment, keeping notes, etc.). Tell study investigators about any possible side effects, as well as any hospital, ER and outpatient clinic visits. Do not conduct your own experiments, as they may invalidate study results. It's also important to maintain confidentiality. Sharing impressions of study experiences can bias study investigators and participants and compromise the integrity of the study.

WHAT CAN I TALK ABOUT?

You may discuss any questions or concerns with the study coordinators or principal

investigator. The study is obligated to maintain your confidentiality and the confidentiality of other participants.

WHAT CAN'T I TALK ABOUT?

You should not talk about whether you think you have placebo or drug. However, if you have a concern about the treatment you are receiving, you should always contact the study coordinator or principal investigator. Both are excellent resources for any questions you may have.

WHAT IF I ENROLLED, BUT NOW I DON'T WANT TO OR CAN'T CONTINUE?

You can withdraw or decide not to participate at any time.

If you withdraw from the study it is very important to contact your study coordinator and inform them of your decision. If you were taking a study medication, the study team will request a final visit with you to ensure you are OK. Your health and well-being come first in research.

WHAT IS INFORMED CONSENT?

Informed consent is permission provided by the participant after they have received all important information about a trial. This includes potential benefits, risks and consequences.

WHO CAN GIVE CONSENT FOR MY CHILD?

If the clinical trial is indicated

for children, the parents or guardian must decide if they want their child to participate. If the parents give permission, older children are usually asked if they wish to participate. This process is called assent. In most cases, a child can refuse to participate, even if the parents are willing to permit it.

HOW DO I FIND A TRIAL?

Talk with your doctor, and reach out to your MDA Care Center team for additional guidance. Search for trials at **ClinicalTrials.gov** or contact local universities. Also, MDA is launching a new clinical trial finder tool in early 2017 on **mda.org** to better connect families with trials for which they are eligible. MDA is working collaboratively with

Learn More

To learn more about current clinical trials relating to neuromuscular diseases, visit mda.org/research/clinical-studies-and-trials.

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TYPES OF CLINICAL TRIALS

When a new product or approach is being studied, it is not usually known whether it will be helpful, harmful or no different than available alternatives. Investigators try to determine the safety and efficacy of the intervention by measuring certain outcomes in the participants.

Phase 0 – Also known as “exploratory studies,” these trials test a small dose of a new drug in a few people as researchers explore how the drug may work in humans and ensure that it is safe. Trial participants may or may not have the disease the drug has been developed to treat.

Phase 1 – In these trials, or “safety studies,” researchers focus on whether an experimental treatment is safe while determining how best to administer the treatment and in what dose. Placebos are not used, and these studies typically include a small number of participants.

Phase 2 – If an experimental treatment proves safe in phase 1 clinical trials, it may then be tested in phase 2 “efficacy” trials. These studies are designed to assess whether an experimental treatment works. The trial may involve healthy volunteers or people with the targeted condition, and some participants may be randomly assigned to different groups that receive a different dose or treatment protocol.

Phase 3 – In this “confirmatory” phase, the number of participants is increased and the experimental drug often is tested against a placebo in order to prove that the drug is both effective and better than any existing treatments.

Phase 4 – Approved drugs are monitored over a period of time in phase 4 studies. Although these treatments are available for doctors to prescribe outside the phase 4 study, participation in these trials helps researchers continue to learn more about the treatment.

In an **observational study**, investigators assess health outcomes in groups of participants according to a protocol or research plan. Participants may receive interventions, which can include medical products or procedures as part of their routine medical care, but participants are not assigned to specific interventions by the investigator.

In a **natural history study**, investigators follow a group of people over time who have, or are at risk of developing, a specific medical condition or disease. These studies collect information to determine how the medical condition or disease develops and progresses. Information collected may be used later to develop and test interventions.

our Care Centers to host more clinical trials during the next few years.

WHY IS GENETIC TESTING IMPORTANT WITH REGARD TO CLINICAL TRIALS?

Some studies require genetic confirmation of a disease before a person can participate in a clinical trial. Genetic testing can pinpoint an individual’s disease-causing mutation to ensure all people enrolled in the study are similar.

WHAT IS A CLINICAL TRIAL TEAM?

Clinical studies are led by a principal investigator, who often is a medical doctor. In addition, there often is a research team that may include doctors, nurses, study coordinators, social workers and other health care professionals.

WILL INSURANCE PAY FOR TREATMENT ASSOCIATED WITH A CLINICAL TRIAL?

Some trials do bill insurance. In many cases, treatment

received in connection with a clinical trial may be covered. This should be disclosed in the study consent form. [Q](#)

This article was written with contributions from Amy Madsen, marketing communications manager for MDA’s research program, and in consultation with Amy Bartlett, NeuroNEXT clinical research manager at The Ohio State University Wexner Medical Center’s Department of Neurology.

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Smart Assistive Mobility Products

Accessories, devices and equipment that hold big promise

BY SHAILA WUNDERLICH



Diestco cup holder

Keeping pace with the latest assistive mobility products is a tall order. The marketplace is so dynamic, so innovative, so accelerated; it can be impossible to stay on top of what's new, what's different, what's covered by insurance or what will enhance your mobility and independence. This edition of "Innovation" catches up with some of the smartest assistive mobility products on the market now, as well as tips for selecting the right types to meet your needs.

convenience accessories are considered "extra" by insurance companies. Wheelchair cup and cellphone holders are two of the most in-demand accessories according to Danielle Lewis, an occupational therapist in the MDA Care Center at Children's Medical Center of Dallas. "They do so much to foster independence," Lewis says. But because their cost usually falls to the patient, "parents end up rigging a DIY version or buying a used or out-of-date one."

Diestco's cup holders include a version for power chairs with padded armrests (\$25, diestco.com) and a choice of horizontal, vertical or front grip. Their cellphone holders (\$20) feature a polyester wrap that attaches to a chair's arm for horizontal grips.

Blue Sky Designs' Mount n' Mover (\$1,595 to \$1,695, blueskydesigns.us) satisfies the most simple of needs: to hold in place the everyday things people can't hold for themselves. The

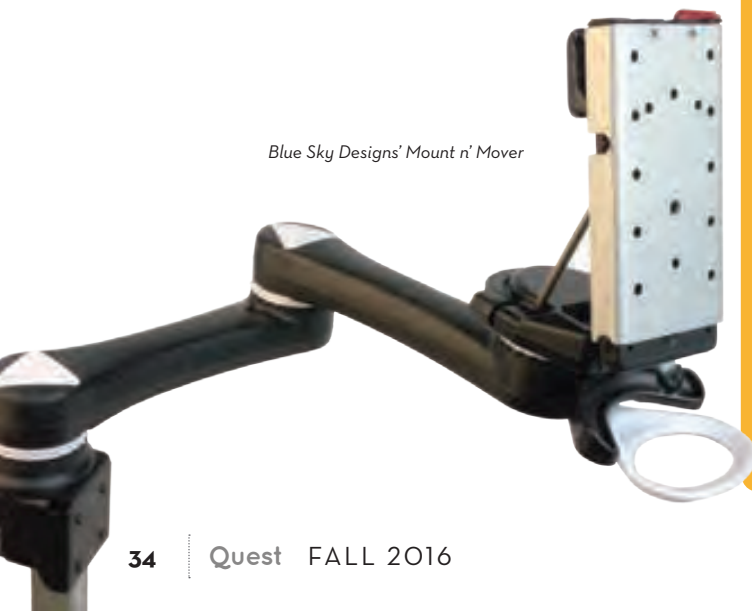
THE MUST-HAVES

Often, it's the simplest things that hold the biggest promise for impacting quality of life. And yet, many everyday

KNOWLEDGE SOURCE TOP THREE TIPS FOR SELECTING PRODUCTS

1. To navigate the question of insurance coverage, start with your doctor. If your prescription is denied, contact the manufacturer. Manufacturers have a good idea who usually covers the cost for their products (insurance companies vs. individuals) and may be able to guide you to sources for coupons, discounts or samples.
2. Therapy and seating sessions, when your child will be 'trying on' equipment and accessory options, make ideal opportunities for learning what's out there, as well as what your child likes. "I've had friends or loved ones pull me aside during appointments to ask product questions," says Kelli Reiling, an occupational therapist at the MDA Care Center at University of Kansas Medical Center.
3. Many products are customized to meet the individual's needs, requiring extensive measurements and information just to place an order. Most manufacturers will guide purchasers through the types of specs needed, as well as creative ways to collect them. "We've had customers email us pictures of wheelchairs, which fills in a lot of the holes," says Mary Kay Walch of Blue Sky Designs.

Blue Sky Designs' Mount n' Mover





The Ergo 3D

Ezpz's Happy Bowls (top) and Happy Mats

KNOWLEDGE SOURCE TOYS FOR EVERY ABILITY

Does your holiday gift list include kids with special needs? Consider looking for toys that do double duty as playthings and developmental aids for children who have physical, cognitive or developmental disabilities with the Toys "R" Us Toy Guide for Differently-Abled Kids. The online guide features specially selected toys that are clearly labeled with the skills they help build, such as language, fine motor or social skills. Find it at toysrus.com/differentlyabled.

long adjustable arm and flat mounting base attach to wheelchairs or bedside stands. Mount 'n Mover was revamped in September 2016 to offer twice the number of possible positions. Most use the mount to hold iPads and food trays, but anecdotes of less common uses show the applications are limitless. "We know of a sixth-grader who loves horns but doesn't have the muscle tone to hold an instrument to his mouth," says Mary Kay Walch of Blue Sky Designs. "He uses his mount plate to perch and play the trumpet in a band."

THE COOL FACTOR

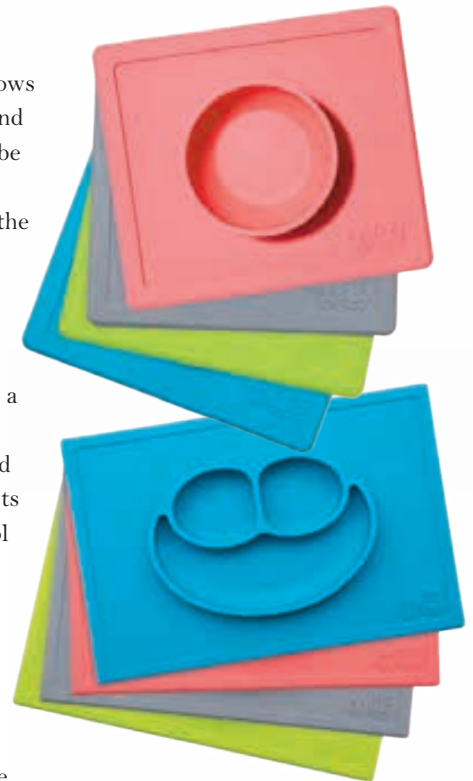
Looking "cool" is crucial to kids of all ages, regardless of their abilities. Some assistive mobility products that look cool actually go much farther than that by also helping kids with neuromuscular diseases stay connected to their peers. "Whenever it's possible to make a product that's both clinically sound and non-medical looking, it's our goal to make that happen," says Matt Lawrence, vice president at Drive Medical. His company's P-Pod (\$1,595 to \$1,995, columbiamedical.com) is a bean-bag style activity

positioning chair that allows children to sit in places and positions that might not be feasible otherwise.

Lewis is intrigued by the chair's design and specs. "This could be a great way to get a smaller child down on the floor with other kids, maybe in a school setting," she says.

Several new eating and drinking assistive products are managing to look cool and help facilitate everyday activities. The Ergo 3D (\$50.92, eurodib.com), a spoon made of malleable silicone, can be twisted to an individuals' most effective ergonomic position. Suddenly, a person who needed feeding assistance may be able to feed themselves. "With the wrist weakness and decreased range of motion that occurs in some individuals with neuromuscular disease, utensils sometimes pair well with a universal cuff or wrist splint," says Teri Krassen, an occupational therapist with the MDA ALS Care Center at Good Shepherd Rehabilitation Hospital in Allentown, Pa.

The Stoneware Dignity Mug (\$40, grannyjopproducts.com) also promotes independence at mealtime. This handcrafted version of the company's classic two-handled mug is easier to grip and lift than traditional drink ware. Ezpz's Happy Mats (\$24.99, ezpzfun.com) and Happy Bowls (\$19.99) are a perfect partner to assistive eating utensils. These tabletop accessories are made in a non-slip silicone that significantly reduces messy, frustrating spills. They come in bright colors, and Happy Mats feature a smiley-face shape to invite fun plating. Q



Drive Medical's P-Pod



The Stoneware Dignity Mug

Shailla Wunderlich is a freelance journalist in St. Louis who has worked for a variety of magazines, journals and newspapers for nearly 20 years.



access MDA

Your guide to the MDA community, from news briefs to inspiring profiles

School Spirit

Sarah Bellish works hard and advocates for more accessibility at school

Between working as a resident assistant, being the philanthropy coordinator for her sorority Alpha Gamma Delta, acting as the 2016 Greek Sing Director, planning a study abroad trip to Australia and volunteering for other organizations, it's hard to believe that Sarah Bellish, a sophomore at Baldwin Wallace University (BWU) who has spinal muscular atrophy (SMA), still has the time to advocate for improved accessibility on her college campus.

"It's been a battle," Bellish says, "but after I came back

after the summer, it was amazing to see all of the changes they can make."

She first noticed the major accessibility issues during freshman welcome week, when she tried to attend a school-sponsored block party with some of her classmates and found that almost all the activities weren't accessible to individuals in wheelchairs.

"All they had on the grounds that was accessible was a photo booth," she says. "All my friends wanted to play the games, so I said 'Yeah, go ahead,' and I just went to my room and started bawling."



Sarah Bellish, a sophomore at Baldwin Wallace University, is active with her sorority, Alpha Gamma Delta.

She was so upset by the situation that she considered transferring schools, but talking with her mom helped her calm down. Bellish's mom suggested joining a sorority, and Bellish found one that was a good fit for her. Sorority membership also provided a stronger support system.

"My sorority sisters give me a lot of support," she says. "They wrote letters to the school, went to meetings with me and things like that. Disability services mostly focus on academic accommodations, which is great, but that's not necessarily everything that I need."

For example, when Bellish initially joined Alpha Gamma Delta, most of the floors and the chapter rooms were not accessible. Her sisters and the sorority's headquarters helped Bellish push for more accessibility, and now the sorority has moved to a first-level building.

"We also changed the way we do recruitment, because I couldn't attend half of the events," Bellish says. "All of that is accessible now, too."

Beyond her sorority house, Bellish has spoken with the university president and other campus leaders to address accessibility issues on campus. For example, Bellish says she got stuck in a lift in BWU's student union dozens of times throughout her freshman year. This year, the university installed a new lift, including a climate-controlled space where students can wait for it.

While it's clear Bellish is making BWU a more accessible place for herself



Sarah Bellish and her sorority sisters do a "throw what you know" hand gesture for their sorority Alpha Gamma Delta in front of Coe Lake, near their university.

and other students with disabilities, it's far from her only activity at the university. Beyond her studies in business administration, Bellish loves singing and hopes to sing professionally in the future. She even recorded a song in Nashville – as part of a Make-A-Wish trip – about her experience at MDA Summer Camp. The song, called "Let Me Go Back," reflects on her 11 years at summer camp

and all the great memories she made during that time.

"I think MDA Summer Camp has been the most helpful thing for me with forming my thoughts about accessibility and everything," she says. "It makes you realize you're not just fighting for yourself, you're fighting for all your friends. We have that week of the year to recognize that there are all these other people who feel the same

things and go through the same things, and forming relationships with them has been the most helpful thing ever."

One particular memory that stands out for Bellish is when she performed "Let Me Go Back" for her fellow campers.

"Performing at camp has been my favorite time singing or performing in my life because I'm singing about the people I care about and the things that matter to them," she says.

 **For more information about MDA Summer Camp, visit mda.org/services/mda-summer-camp. Listen to Sarah Bellish's song "Let Me Go Back" at bit.ly/2cpwJvU.**



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Ava Illingworth poses with her running partner, Candace.

Running for a Reason

Ava Illingworth turned her daily stress reducer into a way to fundraise for MDA

Ava Illingworth picked up running two years ago as an outlet to burn off some of the stress from her job installing software and interfaces for health care organizations. It wasn't the most likely stress reducer for her, considering she has been living with Charcot-Marie-Tooth disease since she received the diagnosis in 2009 at the age of 35.

"All the doctors were back and forth on whether I should exercise," Illingworth says. "I tried Pilates, but for me it hurt. And I'm the kind of person

who needs to sweat to feel like I had a good workout."

So Illingworth started running, at first just for her own exercise, but soon she started doing organized races as well. After participating in several 5K and 10K races, Illingworth went looking for a way to support the fight against muscular dystrophy through her running. This brought her to MDA Team Momentum, and her decision to take on the 10K at the 2016 Marine Corps Marathon & 10K in Washington, D.C., Oct. 30. ▶

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“By the time I was on the Team Momentum website, I already had around 25 5Ks under my belt and two 10Ks,” she says. “I was eager to join a team that was strictly for muscular dystrophy, and they seemed like an open, supportive group to be with.”

And Illingworth has certainly felt the support of her MDA Team Momentum teammates. While living in Delaware made it difficult to attend all of the D.C.-based Team Momentum group practices, Illingworth did make the trip for the kickoff practice.

“It was four hours of driving for an hour-and-a-half run, but it was worth it,” she says. “It was awesome to see everyone face to face and to hear their connection [to the cause].”



Ava Illingworth with her husband Matthew and son Lincoln.

The support continued past that trip on social media and video chat. Illingworth and her teammates posted encouragement for each other often. So much so that Illingworth said her dog, Candace, who runs with her and is often a part of her posts, has become an unofficial mascot for the group.

Heading into October’s home stretch, Illingworth had raised almost \$2,000 for MDA Team Momentum, exceeding her goal of \$1,600.

 To find out how you can join MDA Team Momentum and run to support the fight against muscular dystrophy, visit mdateam.org. Upcoming races include the Dallas Marathon and Half-Marathon (Dec. 11) and the Miami Marathon and Half-Marathon (Jan. 29).

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Calling the Shots

Jermia White brings her passion for sports to work every day

Sports have always been a big part of Jermia White's life. White, a 24-year-old sports statistician from Dallas who has spinal muscular atrophy (SMA), remembers watching her father coach basketball when she was 9 years old, and she went on to be a cheerleader, a manager for her high school basketball team and sports editor for her school's newspaper. Before she went off to college, she knew she wanted to be involved with sports, but it wasn't until she got to Dallas Baptist University that she figured out the right fit.

"Initially I wanted to be a sports journalist," White says. "But at [my college] they had sports broadcasting, and it turned out I like broadcasting more than writing about sports."

Soon she was not only studying broadcasting, but also helping produce the broadcasts of her school's basketball games, producing and directing an awards dinner, volunteering around campus, participating in honors



Jermia White interviewed professional basketball player Rodrigue Beaubois while he was with the Dallas Mavericks.



societies and even interning for the NBC affiliate in Dallas.

"I like the technical side of things," she says. "Everyone wanted me to be the in-front-of-the-camera person, but for me, personally, I'm not that outgoing," she says. ➤

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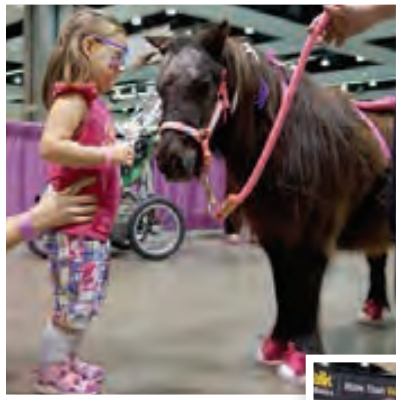
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“I like to be behind the scenes, making sure everything is going the way it should.”

While White’s current job with the NBA Development League’s Texas Legends and the WNBA’s Dallas Wings involves tracking and recording stats, she believes producing is still in her future.

“I’m working part-time on the stats crew, but should I get a full-time producing job, which I plan to, I still want to be involved with the Dallas Wings and the Texas Legends,” she says. “Next year, I’m hoping to work my way up to working with the Dallas Mavericks.”



Watch CBS Sports’ Jimmy Valdes live life unlimited at bit.ly/2cxu1Ss. His inspiring story has gone viral, with more than 1 million views over all social media platforms.



Moving Your Muscles

Members of the MDA community across the country have come together to walk a staggering amount in the name of neuromuscular disease research this year. In 2016 alone, there were:

- 145 Muscle Walk finish lines crossed**
- More than 280 miles of Muscle Walk routes**
- 48 participating states**
- 363 hours of Muscle Walk events**

All of these finish lines crossed, miles walked and hours spent add up and translate into helping MDA fund research breakthroughs, provide best-in-class, comprehensive care from day one, and empower families with support, services and resources.



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Flying High

For Joe Feidt, disc golf is more than a diversion



Joe Feidt's 40-year love affair with the sport of disc golf began as a happy accident. Feidt, a 66-year-old writer and editor for *DiscGolfer* magazine who has inclusion-body myositis (IBM), first discovered the sport in 1976 at a Frisbee tournament in Minneapolis. As it happened, one of his best friends from college was already on the pro Frisbee tour, and he encouraged Feidt to get involved.

"I just happened to get involved right as it was really starting up," Feidt says. "Disc golf then wasn't even really a sport; it was one ➤

Joe Feidt (right) plays disc golf with his friend Chris Enstad.

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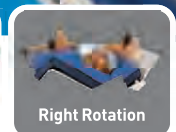
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event in competitive Frisbee tournaments.”

Feidt proceeded to fall in love with disc golf (a game in which players throw a flying disc at a series of targets) and soon set up his own course at a park in Minneapolis. In 1977, he created the first disc golf tournament in Minnesota and, to his surprise, a lot of people showed up to play. That tournament is still going today – the 39th annual Kenwood Klassic was held this year.

Since his start in 1976, Feidt has stayed engaged in the disc golf community, playing professionally and for fun, designing courses, hosting tournaments, writing a newsletter and helping to produce

DiscGolfer, the official magazine of the Professional Disc Golf Association (PDGA). For all the work he’s done, he was inducted into the Disc Golf Hall of Fame in 2011 and is considered “the father of disc golf in Minnesota.”

Being active in the disc golf community keeps Feidt in good health, so when he began to notice certain small issues around 2013 – jars were harder to open, he lost his grip while waterskiing, and he was having occasional issues with swallowing – he didn’t think much of it. During a routine checkup in 2014, Feidt’s wife, Susan, encouraged him to ask the doctor about his swallowing problem. This started a series

of tests and appointments that led to an IBM diagnosis. (Inclusion-body myositis causes progressive weakness of the muscles of the wrists and fingers, the muscles of the front of the thigh and the muscles that lift the front of the foot.)

“It was a surprise,” Feidt says. “I had always been very healthy. As a matter of fact, earlier that year I had applied for life insurance and been given the best rating because I’m so healthy.”


The uncertainty of waiting for a diagnosis while being shuffled through the health care system was overwhelming for Feidt, but learning more about the disease through MDA and other

resources helped him put things in perspective.

“It’s kind of a nasty disease,” Feidt says. “But it’s one of those things that just happens. And I’m lucky that in my particular case, the disease is deciding to progress slowly. When I went to my first MDA support group, I was the only one without a cane or walker or wheelchair.”

And for now, Feidt can still get out on the disc golf course.

“I can still play with my friends, and I really enjoy that,” he says. “I’m hoping I can keep on doing it.”

 **Find more stories of people living unlimited at strongly.mda.org. Learn more about disc golf at PDGA.com.**

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11/15

Walking Strong

Tia Blankenship crosses the finish line with the support of friends and family

When Tia Blankenship was diagnosed with Charcot-Marie-Tooth disease (CMT) in 2002 at the age of 23, she was told she wouldn't be able to walk by the time she turned 35. This past April, at the age of 37, Blankenship walked both the 5K walk and the 1-mile walk at the MDA Muscle Walk of Tampa Bay (Fla.).

"It was emotional," Blankenship says. "One of my husband's employees and his wife and two kids stayed behind us, and my husband and my daughter each had one arm of mine supporting

me. [At the end,] my daughter took off running and had my wheelchair waiting. I sort of collapsed at the line."

The walk was particularly special for Blankenship because it came on the heels of four major surgeries she underwent that caused her to miss the 2015 MDA Muscle Walk. After physical therapy for her last surgery was completed, she started working with a personal trainer to prepare for this year's Muscle Walk. Blankenship and her personal trainer created a plan, based on her doctors'




Tia Blankenship enjoys a well-deserved rest at MDA Muscle Walk of Tampa Bay.

"It helped me not focus on what's happening to my body and to look forward to something in the community," she says. "MDA has been a blessing. Before MDA, I was seeing doctors who didn't know anything about my disease, and it took years to get a good diagnosis. I don't know what we would have done without them."

recommendations and additional research, to prepare Blankenship for the walk.

Blankenship's team, CMT Standing Strong, exceeded their \$7,000 fundraising goal by about \$700. However, for Blankenship, the MDA Muscle Walk was about more than just the money raised.

 **For neuromuscular diseases, early diagnosis and specialized care often lead to the best outcomes. To learn about MDA Care Centers, visit mda.org/services/your-mda-care-center. To find and register for your local MDA Muscle Walk, check out mdamusclewalk.org.**

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Right now, you can make twice the impact – every donation (up to \$100,000) can be matched before Dec. 31. Consider asking family and friends to give to support MDA's efforts to help children and adults live longer and grow stronger.

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From Self-Denial to Self-Acceptance

Learning to accept help from others in daily life is freeing, not debilitating

BY JACQUELINE JOHNSON



“In the first five years of counseling, my therapist taught me how to ask for help and feel OK with receiving that help.”

— Jacqueline Johnson

I was diagnosed with spinal muscular atrophy (SMA) at 18 months. I was pretty fortunate that my doctors said that I looked strong, and the quality of my life would be determined by every milestone I surpassed. However, they couldn't foresee how I'd react to my own disability throughout life.

When I was young, all I wanted was to be what I considered “normal” or “typical.” I wanted to be like everyone else. With that said, I was blessed with two parents and other special people in my life who always told me I was beautiful, I could be anyone I wanted to be, and

my disability never had to define me because I could define it. But my own voice said differently.

Consequently, I kept my distance from people with disabilities most of my life. In addition, I was incredibly stubborn. I wanted to accomplish everything on my own, whatever the task, even if it was painful, really challenging or almost impossible.

So, at what point did I go from self-denial to a place of self-acceptance? This may sound shocking, but it didn't happen fully until my mid-to-late 30s. I faced horrible bouts of depression living in a state of self-denial until that point.

Things started changing in my late 20s, when my depression, stress and anxiety became so severe that I could barely get out of bed. I even had suicidal thoughts. It wasn't the first time I felt this way, though, this time, I knew I needed intensive help. I reached out to the county mental health department and sought the guidance of a therapist who could hold me accountable and help reprogram my thinking.

It took me 10 years to fully accept myself. As you can see, things didn't get fixed overnight, but I had a proper support system and people in place who helped empower,

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support and encourage me along the way. In the first five years of counseling, my therapist taught me how to ask for help and feel OK with receiving that help. This applied to physical and emotional help. He helped me understand that receiving kindness and love from others wasn't a sign of weakness but a sign of strength.

In the last five years, my therapist and an influential teacher told me that my ego was keeping me stuck in that mode of self-denial. I took the courageous steps necessary to love myself and acknowledge the greatness of my life. Conquering the voice of the ego and facing my deepest fears propelled me into living a more fulfilling life. I was able to interact with people with disabilities and see them as inspirational. I was even able to see myself as inspirational.

At the same time, I realized that I could take more risks, which could help create liberation and independence. I had lived on my own before, and I was sure I could do it again. I'm currently living

with my parents and receiving caregiving services. In the past year, I started really noticing my parents aging. I knew I needed to find a solution for all of us. That's when I decided to speak with a social worker at Marianjoy Rehabilitation Hospital about supportive living facility locations.

At first, I was scared to look at this option. I knew I didn't need a nursing home, and I didn't know much about supportive living facilities. After a bit of trepidation, I made an appointment at one facility about 45 minutes from our house.

It was amazing! All of the necessary services are provided in the building and each person has their own apartment. Wow, that was exactly what I needed. The application process was pretty simple. You need to be a Medicaid recipient. Check. You need a state rehabilitation services evaluation. Check. Of course, there are also doctors' notes and financial records that need to be provided. Check. Check.

I'm now on the waiting list. I'll either be provided a studio or one-bedroom apartment. I was able to see a studio apartment. It was really spacious. There is 24/7 caregiving, laundry twice a week, three meals a day, and different medical doctors come at different times during the month. As a bonus, this facility also has extra amenities, such as a basketball court, bowling alley, greenhouse and access to the Fox River, which is beautiful.

Imagine having all the things that you need and having your own individual apartment and privacy. Doesn't that sound amazing? I was told most states have these supportive living facilities for younger adults, but few people know about them. I'm envisioning a whole new life for myself. I'll be able to have personal relationships, if I'd like them. I'm definitely going to start up oil painting again. I'll be able to come and go as I please. Freedom bound! I can't wait!

I hope you'll take more risks and reach for your dreams. I promise it will be worth it. [Q](#)

Jacqueline Johnson, 38, was diagnosed with SMA at 18 months. She's currently working as an online English teacher working with international students all over the world. She's also a professional coach and aspires to be a motivational speaker. You can reach out to her at [linkedin.com/in/jackiejohnson16](https://www.linkedin.com/in/jackiejohnson16).

Running Wild

Abel Alejandrino goes above and beyond for his daughter, Ava



Abel Alejandrino and Ava



Abel Alejandrino poses with Emily Gaul, Sharon Coburn and his daughter, Ava, at the Colfax Marathon in Denver, Colo.

By the end of this summer, Abel Alejandrino had already run two marathons and an ultramarathon in support of MDA. He had covered more than 100 miles — and he was just getting started. Alejandrino intends to run at least two more marathons this year as part of MDA Team Momentum, including the Bank of America Chicago Marathon in October and the Dallas Marathon in December.

Why does he run? Alejandrino’s 7-year-old daughter, Ava, has nemaline myopathy, a neuromuscular disorder that causes weakness and poor muscle tone (hypotonia) in the face, neck and upper limbs, and often affects the respiratory muscles (those that control breathing).

“I only really started running in 2012, and I’ve always been active, but nothing like this,” Alejandrino, a program specialist for San Francisco County, says.

While he says doing so much running has been physically challenging, that hasn’t affected his resolve.

“A lot of people think I’m doing all the work,” he says, “but this is what people like Ava who have muscular dystrophy are doing every day.”

When Ava was first diagnosed with nemaline myopathy in late 2013, it came as a surprise to Alejandrino and his wife, Cynthia. They weren’t sure how their friends and family would deal with the news, so some found out about Ava’s diagnosis for the first time through Alejandrino’s MDA Team Momentum fundraising campaign. He says the response has been great, and he’s seen many people reach out to provide support, including his fellow MDA Team Momentum runners.

“The first Team Momentum event I did was out of state,” says Alejandrino. “I arrived and I was actually wearing the MDA Team Momentum shirt they sent me. As soon as [the other Team Momentum participants] saw me, they brought me into their group and it was so welcoming and such a rewarding experience, not only for me, but for my daughter as well. It was like we knew everyone already.”

After Alejandrino finishes his whirlwind year of running marathons, he plans to share his passion with Ava at the 2017 Kids Marathon Mile at Legoland in January 2017.

“She’s preparing,” he says. “We have to strategize, because even though Legoland is only a mile, it’s like a marathon for her. We’re going to hold a track session and bring the community in and get people involved. She’s happy and excited about it.” **Q**

Get Involved

Join Abel and hundreds like him, and cross the most meaningful finish line of your life. Upcoming events include races in Dallas and Miami. Learn how you can get involved with MDA Team Momentum at mdateam.org.

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