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BY STEVEN M. DERKS, MDA PRESIDENT AND CEO

The Heart of Our Mission

The Muscular Dystrophy Association is the world's leading nonprofit health agency dedicated to saving and improving the lives of people with muscular dystrophy, ALS (amyotrophic lateral sclerosis) and other life-threatening neuromuscular diseases. It does so by funding worldwide research to find treatments and cures; by providing comprehensive health care services and support to MDA families nationwide; and by rallying communities to fight back through advocacy, fundraising and local engagement.

EACH DAY ACROSS AMERICA, the children and adults we serve and represent demonstrate inspiring can-do spirit and strength, often defying remarkable odds through their actions — from unforgettable events like walking a daughter down the aisle, skydiving or hiking the Grand Canyon, to everyday moments like starting kindergarten, going to college or sharing a laugh with a loved one.

MDA families are — and always have been — at the heart of MDA's lifesaving mission. We were started by families, for families, and we are 100 percent dedicated to freeing families from the harmful effects of muscle-debilitating diseases. Along the way, we work hard to help families have more of these unforgettable and everyday moments with fewer barriers.

With the families we serve in the forefront, I am encouraged by the partnership and collaboration of the neuromuscular disease community to achieve progress. We know it will take all of us working together to achieve our shared mission. In turn, MDA has been working to expand its collaborative relationships with researchers, clinicians, and both nonprofit and for-profit partners. We are excited to be involved in new partnerships with CureDuchenne, the Friedreich's Ataxia Research Alliance and numerous ALS groups, and we continue to move ahead and make progress through ongoing partnerships with the American Association of Neuromuscular & Electrodiagnostic Medicine and Genzyme. These relationships will enable us to align resources and focus efforts for faster impact.



Certainly, we have an urgent focus to accelerate treatments and cures. Since our last issue of *Quest*, MDA has awarded \$10 million in new research grants to the best and brightest researchers across the globe. These new grants are supporting studies seeking to advance our understanding and discovery across the full spectrum of neuromuscular diseases. Therapy development is getting a greater percentage of these grants, given the great progress that has occurred in that field during many decades of past MDA basic science investments.

We are seeing exciting, promising advances and continue to develop new ways to provide the best care to kids and adults fighting these devastating diseases, including:

- Expanding clinical trials through enhanced partnerships with medical institutions and drug developers.
- Using new channels to connect families and caregivers to more and better resources.
- Refreshing the MDA brand, including rolling out a new MDA website with advanced capabilities early next year.
- Establishing a new corporate engagement and recognition program to give our generous partners enhanced avenues through which to support families and fuel progress.

As you'll see in the subsequent pages and at MDA.org, our families' compelling stories of strength, determination and resilience inspire us and help raise awareness for the diseases we fight. With the holidays and family gatherings fast approaching, I want to thank all of you who proudly support MDA's lifesaving mission. Your continued support helps translate this hope into strength that is yielding answers.

Steven M. Derks
President and CEO
Muscular Dystrophy Association

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COVER PHOTO BY ROB GOULDING

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web exclusives

Changing Directions

Writer Chris Anselmo, who lives with Miyoshi myopathy, finds a new job for the summer in a new city and learns the value of embracing the unexpected.

Born with a Superpower

Writer Sarah Manuel, who lives with type 2 spinal muscular atrophy, finds strength in herself because of her disability, not despite it.

Click on the *Quest* cover at quest.mda.org to read these and other stories.

Quest



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Fill the Boot

In their jobs, fire fighters save lives every day. But they also personify strength and courage off the clock. Consider the Fill the Boot (FTB) campaign, the long-standing fund-raising partnership between MDA and the International Association of Fire Fighters (IAFF). Each year, members line street corners to collect money — in their boots — from neighbors and passersby. Just as past donations have translated into life-changing research and support for MDA families, so too are today's FTB collections fueling future progress in the fight against muscular dystrophy, ALS and related life-threatening diseases.

162,000

Number of fire fighters participating in FTB in 2015 (includes union-IAFF and nonunion fire fighters)

61

Years of the MDA-IAFF partnership

50

Number of states represented with FTB

\$558 million

Total money raised since the partnership began

3,200

Number of FTB events expected to take place in 2015

“With MDA, the IAFF’s support is not going to come to an end until the need for MDA doesn’t exist.”

— Harold A. Schaitberger, IAFF General President



For more on Fill the Boot and MDA’s relationship with IAFF, visit mda.org/sponsor/international-association-fire-fighters and read “Giving Muscle Disease the Boot” in the *Quest* archives at quest.mda.org. To get involved in future FTB events, contact your local MDA office at **(800) 572-1717**.

MARATHON

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Marathon Pharmaceuticals was founded to develop treatments for people living with rare neurological and movement disorders.

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To learn about Marathon Pharmaceuticals, LLC, visit:
www.marathonpharma.com



Image does not represent an actual patient
DMD-15-005

Toward Treatments and Cures

New grants pave the way to progress

As part of an effort to refocus its research grants program, MDA began funding 36 new research and development grants, totaling nearly \$10 million, on Aug. 1, 2015. The awards were made to researchers seeking to increase understanding of and find promising treatments and cures for muscular dystrophy, ALS and related neuromuscular diseases that limit strength and mobility. Making up the summer 2015 grant cycle, the awards reflect an increase in spending within MDA's research program and underscore MDA's commitment to investing in research across diseases — with the expectation that increased understanding in one disease area will inform progress and lead to research and medical breakthroughs in other disease spaces.

MDA's Medical and Scientific Advisory Committees, comprising some 40 of the world's leading clinicians and scientists who serve in these volunteer roles for MDA, recommended the top grants out of 350 applications to MDA's Board of Directors. Board approvals were made in July.

Among the research projects funded by the new grants:

- Scientists at the University of Kentucky in Lexington will study a new drug target for **ALS** and test a potential therapy.
- Scientists at Biosciences Institute, University of São Paulo (Brazil), will study how a protein called Jagged1 prevents the symptoms of **Duchenne muscular dystrophy (DMD)** in

animal models bearing the mutation that causes the disease.

- Scientists at the University of Oklahoma Health Sciences Center will work to find the optimal HDAC inhibitor (and dose) for increasing production of the frataxin protein, which is deficient in **Friedreich's ataxia (FA)** and, in parallel, test an alternative HDAC inhibitor that may prove more effective than others tested to date.
- Scientists at Columbia University in New York will study the underlying mechanisms that drive a recently discovered form of **congenital muscular dystrophy (CMD)** and test three possible treatment paradigms.
- Scientists at Johns Hopkins University School of Medicine in Baltimore will test a gene therapy strategy to increase SMN protein levels in **spinal muscular atrophy (SMA)**. This project

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

is co-funded by the American Association of Neuromuscular & Electrodiagnostic Medicine (AANEM), as MDA and AANEM continue to build on a partnership aimed at providing neuromuscular disease education and supporting research related to muscle and nerve disorders.

The new grants complement a wide range of ongoing neuromuscular disease research MDA is supporting worldwide.

ALS (amyotrophic lateral sclerosis)

Q on the Move

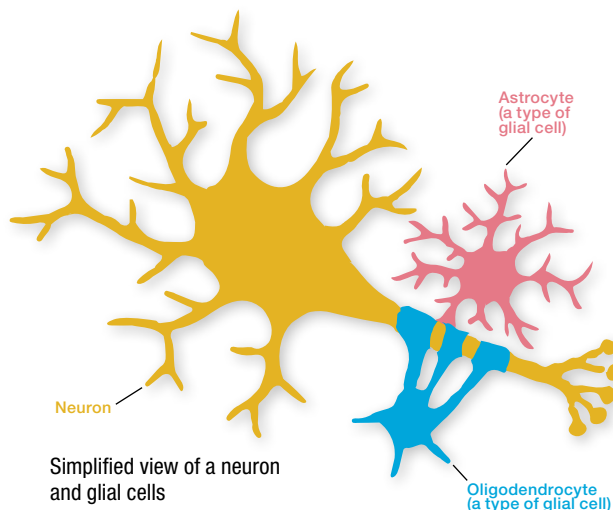
FDA clears way for clinical trial to test ALS cellular therapy

Q Therapeutics announced in June that the U.S. Food and Drug Administration (FDA) has cleared its Investigational New Drug Application (IND) for the initiation of phase 1-2a clinical trials of its experimental cellular therapy, Q-Cells, in people with **ALS (amyotrophic lateral sclerosis)**.

Q-Cells are human glial-restricted progenitor cells (GRPs) that give rise to two types of specialized glial cells — astrocytes and oligodendrocytes — which are critical for normal motor neuron function.

The trial, which is set to begin in October, will test the safety, tolerability and early efficacy of Q-Cells transplantation in 12 participants with ALS. The trial is not yet open for enrollment.

The FDA has previously granted Q-Cells orphan drug status — a designation intended to advance the evaluation and development of therapeutics that demonstrate promise for the treatment of rare diseases — for ALS.



Simplified view of a neuron and glial cells

+ For more about Q Therapeutics' development of Q-Cells to treat ALS, visit qthera.com. To find out more about this trial, search for "NCT02478450" at ClinicalTrials.gov.

Duchenne muscular dystrophy (DMD)

MOVING FORWARD

Corticosteroid treatment deflazacort advances toward approval in the U.S.



Marathon Pharmaceuticals has begun the New Drug Application (NDA) process for deflazacort as a treatment for **Duchenne muscular dystrophy (DMD)**.

Deflazacort, a corticosteroid, works as an anti-inflammatory and immunosuppressant. It has been shown to slow the loss of muscle strength and function, preserve cardiac and respiratory function, and reduce the incidence of scoliosis (curvature of the spine) in people with DMD. Importantly, the unwanted side effects often experienced with corticosteroids, such as weight gain, loss of bone mass, glucose intolerance (diabetes) and behavioral issues, may be less severe with deflazacort as compared to other steroids.

Marathon expects to submit the NDA to the FDA in the first quarter of 2016. If the application is approved, the company could make the drug commercially available in the United States in the first quarter of 2017.

+ To learn more about deflazacort in DMD, read “Charting a Corticosteroid Course: The Benefits and Side Effects of Prednisone and Deflazacort” at quest.mda.org.

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Duchenne muscular dystrophy (DMD)

Trial Seeks Participants

CAT-1004 to be tested in boys with DMD, ages 4 to 7



A phase 1-2 clinical trial called MoveDMD is open for recruitment at Shriners Hospitals for Children in Portland, Ore., and the University of Florida in Gainesville, Fla., with a third site expected to open soon in Philadelphia.

The two-part trial will test the experimental therapy CAT-1004 in boys with DMD ages 4 to 7 years old. Investigators are looking for a total of 18 boys to participate in the first part of the trial, which will examine how the drug moves through the body and collect data on physical function and muscle strength of the lower and upper legs to help understand potential benefits of the drug in DMD.

CAT-1004, in development by Catabasis Pharmaceuticals, is an anti-inflammatory compound that is designed

to act similarly to corticosteroid medications (like prednisone and prednisolone) but with fewer unwanted side effects. Preclinical data suggest that CAT-1004 may reduce muscle damage, stress and inflammation.

Boys who participate in the first part of the trial and who meet study criteria will be asked to participate in the second part of the trial, which will further evaluate safety and efficacy of the experimental therapy.

+ To learn more about this trial, including inclusion and exclusion criteria, search for “NCT02439216” at ClinicalTrials.gov.

TREATMENTS ON THE HORIZON

Two exon-skipping drugs to treat DMD in a subset of boys diagnosed is poised to enter U.S. market

BioMarin announced June 29 that the FDA has accepted its New Drug Application (NDA) for drisapersen, and Sarepta announced Aug. 25 that the FDA has accepted its NDA submission for eteplirsen. Both “exon-skipping” drugs target exon 51, and may help up to 13 percent of DMD patients. Exon skipping is a treatment strategy in which sections of genetic code are “skipped,” allowing the creation of partially functional dystrophin, the muscle protein missing in DMD. Exon skipping is not a cure for DMD, but it could lessen the severity of muscle weakness and atrophy that are the hallmark of this disease. Both companies hope to have an FDA decision by early 2016.

+ For more about BioMarin’s development of drisapersen to treat DMD, visit BMRN.com. For more about Sarepta’s development of eteplirsen to treat DMD, visit sarepta.com.

STRONG PROSPECTS

Early results for HT-100 in DMD are encouraging

The experimental DMD drug HT-100, in development by Massachusetts-based Akashi Therapeutics with support from MDA, has shown encouraging preliminary results in an ongoing phase 1b-2a clinical study in boys with DMD. Data

from this ongoing clinical trial shows that boys with DMD who were treated with HT-100 demonstrated improvements in muscle strength. In addition, the drug continues to be safe and well-tolerated by trial participants.

MDA has contributed more than \$1 million toward the development of HT-100 as a therapy for DMD, supporting early-stage work that informed the laboratory development of the drug and providing funding for the ongoing trial as well.

HT-100 is an oral compound that is intended to reduce inflammation and scar tissue formation and promote

regeneration in DMD-affected muscles. It does not target specific mutations in the dystrophin gene, making it potentially useful for all patients with DMD.

+ For more on this trial, read “DMD: Multicenter Trial to Test Drug that Fights Muscle Scarring, Inflammation” at quest.mda.org.



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Duchenne muscular dystrophy (DMD)

Fighting Fibrosis

FG-3019 to be tested in DMD

FibroGen announced in July that it has received FDA clearance to begin clinical testing of FG-3019 in DMD. The company plans to initiate a multisite phase 2 trial in late 2015 to test the therapy in nonambulatory patients.

In DMD muscle cells, an absence of the dystrophin protein required for normal muscle fiber function leads to muscle damage, inflammation, fibrosis (scarring), and progressive dysfunction and weakness. In preclinical studies, FG-3019 reduced muscle fibrosis and significantly improved muscle function. Separately, in clinical trials for another fibrotic condition, idiopathic pulmonary fibrosis, FG-3019 was shown to reverse fibrosis in

a significant proportion of patients.

If testing is successful, this therapy could help improve the lives of people with DMD by delaying loss of muscle function.

+ For more about FibroGen's development of FG-3019 to treat DMD, visit fibrogen.com.



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Facioscapulohumeral muscular dystrophy (FSHD)

QUALITY OF CARE

Experts publish first-ever evidence-based guideline for FSHD diagnosis and care

The American Academy of Neurology (AAN) and American Association of Neuromuscular & Electrodiagnostic Medicine (AANEM) have released a guideline for the diagnosis and care of people with **facioscapulohumeral muscular dystrophy (FSHD)**, a genetic muscle disorder in which muscles of the face, shoulder blades and upper arms are among the most affected.

Based on the best available scientific studies of FSHD, this is the first-ever evidence-based guideline to help physicians best manage the care of people with FSHD. It includes key recommendations on:

- genetic testing to confirm diagnosis; and
- management of FSHD-associated complications, including those related to lung and breathing function, shoulder problems, hearing loss and pain.

Goals for the new guideline are to increase awareness that although cures are not currently available, disease management is important and valuable; raise awareness of the strong need for further FSHD research; and increase awareness of the importance of genetic testing for accurate diagnosis.

+ Read the guideline in the Guidelines section on aan.com, or view a family-friendly version on the site at aan.com/Guidelines/Home/GetGuidelineContent/702. To learn about pain management, read "Medication, Meditation and Movement" at quest.mda.org.

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Endorsed by Rob Roozeboom (above), member of MDA's National Task Force on Public Awareness

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Hereditary inclusion-body myopathy (HIBM)

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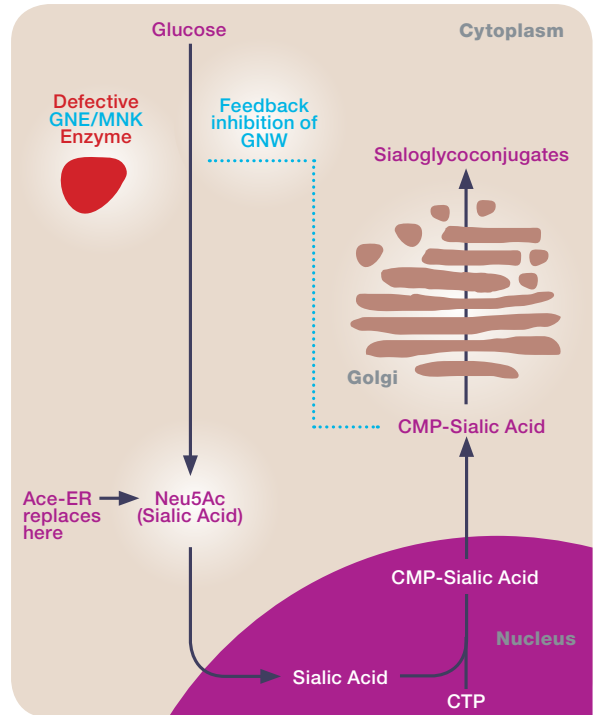
Extended release aceneuramic acid in HIBM is being tested in a phase 3 trial

Ultragenyx Pharmaceutical has initiated a phase 3 study of aceneuramic acid extended release (Ace-ER) tablets for the treatment of GNE myopathy, also known as **hereditary inclusion-body myopathy (HIBM)** and **Nonaka distal myopathy**.

The new placebo-controlled clinical study, conducted in approximately 80 participants, will assess the efficacy and safety of six grams per day of Ace-ER over 48 weeks. The primary endpoint of the study is a composite of upper extremity muscle strength as measured by handheld dynamometry (HHD). Key secondary endpoints include the GNE Myopathy-functional activity scale (GNEM-FAS), a disease-specific, patient-reported outcome that measures mobility, upper-extremity function and other indicators of lower-extremity muscle strength.

+ To learn more about this trial, including inclusion and exclusion criteria, search for “NCT02377921” at ClinicalTrials.gov.

Ace-ER is designed to replace sialic acid, which is deficient in patients with this disease. It is expected that the muscles will pick up increased amounts of sialic acid from the serum and incorporate it into proteins and fats, potentially improving muscle strength and function over time.



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Limb-girdle muscular dystrophy (LGMD)

DIAGNOSTIC ACCURACY

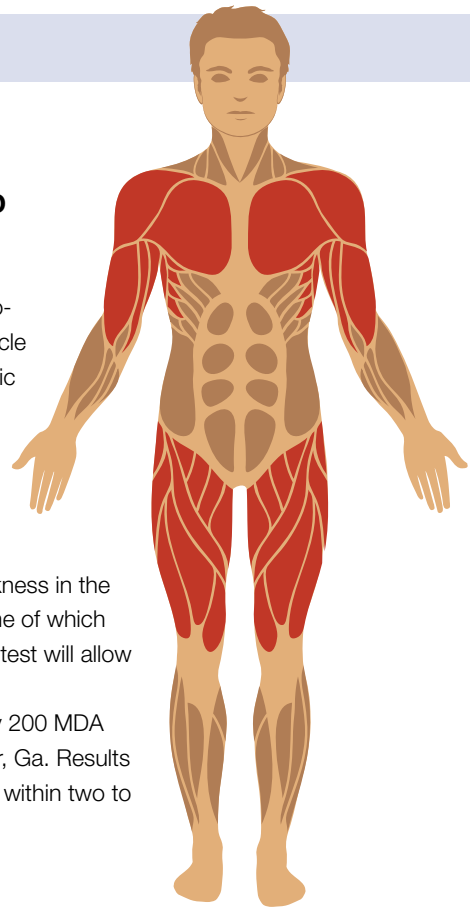
Additional funding will expand access to genetic testing for LGMD

Genzyme has provided \$700,000 in additional funding to continue a **limb-girdle muscular dystrophy (LGMD)** genetic testing program launched in March. The program, which allows MDA clinics to offer genetic testing to people experiencing muscle weakness suspected to be caused by LGMD and who do not already have a genetic diagnosis, has received an overwhelming response. This additional funding will allow more people living with LGMD, and their clinicians, to find the most accurate diagnosis and treatment paths available.

Since the partnership began in March, more than 600 tests have been administered in MDA clinics across the country.

LGMD is a disorder with more than 30 genetic subtypes, all of which cause weakness in the limb-girdle muscles — the muscles surrounding the shoulders and hips — and some of which are much more likely than others to involve the cardiac or respiratory muscles. This test will allow clinicians to provide accurate diagnosis and treatment.

The test requires a saliva sample, which can be collected at any one of the nearly 200 MDA clinics across the country and is then sent to Emory Genetics Laboratory in Decatur, Ga. Results are returned to the clinic physician and communicated to patients and their families within two to three months.



+ To find an MDA clinic near you and to learn more, visit mda.org/locate.



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Periodic paralysis

UNRESTRICTED MOVEMENT

FDA approves first medication for hyperkalemic and hypokalemic periodic paralysis

Taro Pharmaceuticals announced in August that the FDA has approved Keveyis for the treatment of **hyperkalemic** and **hypokalemic periodic paralysis**. Keveyis is the first medication to be approved for the treatment of this group of hereditary disorders that causes episodes of muscle weakness or paralysis. Keveyis is expected to be available for patients in late 2015, and Taro is working to make sure people diagnosed with periodic paralysis can receive treatment as soon as possible.

MDA provided support for the development of Keveyis beginning in the early 1990s, including contributions toward the early-stage development of the drug and funding support for a phase 3 human clinical trial — all of which has helped make this new treatment possible.

+ To be notified when the drug becomes available, enroll at keveyis.com.

Spinal muscular atrophy (SMA)

POSITIVE PROGRESS

Gene transfer therapy chariSMA demonstrates encouraging results

Biotechnology company AveXis announced promising interim results in June for its ongoing phase 1 SMA gene therapy trial in infants with type 1 **spinal muscular atrophy (SMA)**.

AveXis reported that all three infants being treated with a low dose of the company's SMA drug, known as chariSMA, have surpassed the age at which untreated babies with type 1 SMA lose their battle with the disease. The three currently range in age from 15 to 18 months.

All nine infants enrolled in the trial remain alive, and none have progressed to the need for permanent ventilation (defined as 16 hours/day or greater use of ventilation assistance).

SMA is caused by a mutation in the SMN1 gene, which results in a deficiency of SMN protein.

ChariSMA contains the gene for the full-length SMN protein, encased in the shell of a type 9 adeno-associated virus (AAV9 “vector”) that serves as a “gene transfer” or “gene therapy” delivery vehicle.

The phase 1 trial of intravenous SMA gene therapy is being conducted at Nationwide Children's Hospital in Columbus, Ohio, under the direction of neurologist Jerry Mendell, a long-time MDA research grantee and co-director of the MDA clinic at his institution. (MDA is not, however, funding this trial.)



+ For more information on chariSMA, search for “NCT02122952” at ClinicalTrials.gov.

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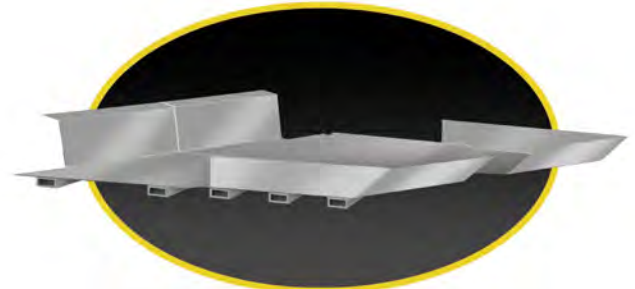
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Spinal muscular atrophy (SMA)

Embracing Opportunity

A multicenter phase 2 study of ISIS-SMNRx seeks participants

Biogen, in partnership with Isis Pharmaceuticals, has initiated a new phase 2 clinical trial, called EMBRACE. This multicenter phase 2 clinical study aims to test the safety and exploratory efficacy of the investigational drug ISIS-SMNRx in approximately 20 patients with infantile or childhood-onset SMA over a 14-month period.

ISIS-SMNRx is being developed by Isis Pharmaceuticals in collaboration with Biogen. Developed using a gene-modifying strategy known as antisense, it targets the SMN2 gene and encourages production of the full-length SMN protein that is needed but deficient in SMA-affected cells.

EMBRACE is the fourth ongoing trial testing the same drug (ISIS-SMNRx) for SMA patients. The other trials are ENDEAR, CHERISH and NURTURE. The purpose of the new trial is to capture patients who do not meet the age requirements or other criteria used in the other trials.



+ To learn more about how to participate in this trial, search for "NCT02462759" at [ClinicalTrials.gov](https://clinicaltrials.gov).

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Holiday Cheer

Helpful tips to minimize end-of-year stress and maximize the spirit of the season

BY BARBARA AND JIM TWARDOWSKI, RN

AS THE AUTUMN LEAVES start to fall, the holiday season is upon us. And between the hustle and bustle of decorating, shopping, food preparation and social gatherings, the holidays can be a source of increased stress. Anyone can feel overwhelmed this festive time of year, but for people living with neuromuscular diseases, managing the additional activities and expenses is crucial to maintaining mental, physical and financial health. Consider the following tips to help you plan ahead.

Diet and food prep

If you are cooking a holiday meal, review the recipes you intend to include weeks before they are needed. Determine which dishes can be made ahead of time and frozen. To prevent fatigue, pace yourself by cooking only one recipe per day or every other day. For cooks who can't lift a huge turkey or use a hot oven, order the meat from a local restaurant or grocery store, and stick to easier-to-prepare side dishes.

And when in doubt, delegate tasks to family and friends. When entertaining, divide the work by throwing a potluck party. Use paper products instead of the fine china to make cleanup a snap. Don't overexert yourself before the party even begins; ask a friend or two to arrive early to help with the preparation.

Holiday dishes are notoriously high in fat. "If you eat well 80 to 90 percent of the time and you're not having tons of snacks and treats, then in many cases you should be allowed to go to a party and eat what you want," says Elana Sussman, registered dietitian at Mattel Children's Hospital UCLA, site of an MDA clinic. But for individuals with special diets or food restrictions related to a diagnosis, consult a dietitian and care provider on specific options and goals, she adds.

To enjoy the holidays without sabotaging your waistline, Sussman offers these tips as well:

- **Don't skip meals.** Going to a holiday function starving will invariably lead to overeating.

- **Bring healthy dishes to holiday events.** If cooking is a chore, pick up a fruit or vegetable tray at the market. For people who have difficulty swallowing, bring a smoothie and let the hostess pour into small cups to share with other guests.
- **Load half your plate with vegetables, fruit and lean protein.** Take only a bite or two of food with creams or sauces.
- **Alcohol inhibits your ability to make good food choices.** If you are allowed alcohol, limit consumption — a conservative serving is four ounces. Calorically, red wine is always a better choice than mixed drinks.
- **Drink plenty of water to stay hydrated and feel full.** Holding a glass of water also keeps your hands occupied so you aren't munching on the appetizers.

Families and friends

Holidays sometimes create unwanted drama. The uncle who overindulges in the eggnog and the sisters who always fight are emotionally draining on those around them. People who have chronic health conditions need to set parameters regarding visitors, whether family or friends, advises Sheilah Storch, a licensed clinical social worker at the MDA Clinic at Texas Neurology in Dallas.

Before the holidays, it's a good idea to communicate with your potential visitors. Schedule visits when you typically feel the strongest. For instance, many people with neuromuscular diseases have more energy in the morning and tend to tire in the afternoon, Storch notes. Crowds can be overwhelming for some individuals, especially those experiencing sensory loss or communication difficulties. Limit the number of visitors to two or three. If you are able to join friends and family outside your home, then it is easier to exit when your stamina is waning.

If you are traveling to see relatives, consider staying at a hotel. An all-suite property is typically more spacious. The added square footage is worth the expense for people who are traveling with bulky medical equipment. Book reservations early, as hotels have a limited number of accessible rooms. If you are entertaining houseguests, set a limit to the number of days they can visit or ask them to spend some of their visit in a hotel.

Gifts and other expenses

Overspending on gifts, party outfits, decorations and all the other activities surrounding the holidays also can cause stress. Planning ahead for purchases and setting a realistic budget is



A LITTLE HOLIDAY HELP

Many people seek out volunteer opportunities during the holidays, and some high schools even require students to fulfill service hours. Reach out to local schools and community groups, like scouting troops and religious organizations, to inquire about assistance with holiday baking, decorating or gift-wrapping. Or contact your local MDA office at (800) 572-1717 (select option 2) or mda.org/locate for help finding such leads.

paramount to avoiding the buyer's remorse that accompanies an inflated credit card bill. Consider these tips to get started:

- Make a list of every person who will receive a gift and determine the total budget. Before heading to the mall, compare prices online. To avoid dealing with excess crowds and traffic, purchase gifts throughout the year, as items go on sale – or shop online.
- Instead of giving to every person, suggest that family members, perhaps adult siblings, draw a name and exchange a gift with a predetermined price cap.
- Keep it simple and make donations on friends' behalf to a favorite charity, like MDA.

- Save money and buy decorating supplies at the dollar store. For people who have difficulty cutting wrapping paper or manipulating tape, stock up on gift bags and tissue paper. The addition of a \$1 ornament can turn a solid-colored bag into a holiday-themed package.
- Handmade items are all the rage. Save money by rolling up your sleeves and creating one-of-a kind gifts. Some examples, like building a bird house or painting a set of notecards, can even be made with the help of children. Look for inspiration on Pinterest.
- Scan the newspaper for free concerts and performances. Check with the Convention and Visitor Bureaus where you might travel for affordable events, parades and holiday markets.
- Many local MDA offices sponsor a holiday party for families, as well.

Above all else, don't feel pressured to be perfect during the holidays. It's OK to scale back. Be picky about how you spend your time to ensure you're rested for the events you do attend. In other words, during this hectic time of year, remember that it's OK to say, "No."

So take a deep breath, relax and enjoy the season. **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 Mandeville, La., and writes about accessible travel, assistive technology and related issues.

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A pink sticky note is placed on a dark wooden desk. In the background, there is a spiral-bound notebook with a black pen resting on it. To the right, a portion of a white laptop is visible.

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WORKFORCE

FOR MANY INDIVIDUALS WITH NEUROMUSCULAR DISEASES, **FINDING A MEANINGFUL CAREER** CAN MAKE FOR A RICHER, MORE INDEPENDENT LIFE

ALL IN A

day's w



oving from being a high school or college student to an independent, working adult can be challenging for almost anyone.

But for individuals who live with neuromuscular diseases, the transition into the workforce can be especially overwhelming.

But young adults in the MDA community who have successfully made this employment leap agree that the rewards of meaningful work are worth the challenges they've faced en route to establishing thriving careers.



ork

BY DONNA ALBRECHT



Take Dan Dorszynski. “It feels great to be able to keep working and participating in society in the way most people do,” says Dorszynski, a computer programmer and entrepreneur who received a diagnosis of **Becker muscular dystrophy (BMD)** when he was 13 years old. “Although I have days where I feel like my plate is overloaded, I know that having work in front of me is better than

not having anything to do. Plus, I like what I do, so it’s fun and challenging.”

Here, Dorszynski and others in the MDA community share stories and tips from their own career journeys thus far.

The job hunt

According to Amanda Papp, MDA’s health care service coordinator in Columbus, Ohio, who works with many young adults

as they explore their future, the path of pursuing and landing a job and succeeding in a work environment is by no means out of reach for young adults with neuromuscular disease. In fact, she says, in some respects such individuals are well-suited for this challenge because they’ve always had to test their limits to get as far as they have in life.

“A sense of hope is present because many of these young adults are ready to tackle that challenge and take on the world,” Papp emphasizes.

That sense of hope, and youthful idealism and enthusiasm, is especially potent in high school and college students. Perhaps that’s part of the reason why Papp and others suggest that the best time to begin looking for a full-time job is when one is still in school. Internships in particular are

“INDEPENDENCE does not mean being able to do every single task. It means being able to direct how things are done. A big aspect of independence is directing your own life through education and then employment.”

— Katrina Gossett



HEALTH CARE HOW-TO

As an independent adult, you may qualify for continued coverage under your parents’ insurance plan; Medicaid (exact qualifications and benefits vary by state); the Affordable Care Act (ACA); or through your employer’s plan. For example, Dan Dorszynski says, “I’m really happy the Affordable Care Act passed, because it allowed me to buy my own insurance as a self-employed person with a pre-existing condition.” Katrina Gossett and Dan Sedgeman on the other hand, have health insurance through their employers.

Kristin Stephenson, MDA vice president of Policy & Advocacy, offers these notes and tips to help you evaluate the best health plan for you:

- **Medicaid** is different in every state, and there are wide variations among policies and coverage details.
- Similarly, health insurance is not a one-size-fits-all proposition — determining the right coverage option requires taking a deep dive into the fine print of a policy. For example, if you move from one plan to another, will your current providers still be covered as “in-network”? And what is the network? Who is part of it?

Other questions to consider:

- **Co-pays:** What’s included? Doctor/medical appointments, medications, etc.
- **Deductibles:** How much per use? Is there a cap?
- **Preauthorization requirements:** Does the plan require a gatekeeper (like many HMO plans) or can a patient self-refer to a specialist?
- **Cost calculations:** How do your known costs (or most likely costs based on experience) compare to the annual out-of-pocket maximums?
- **Coverage considerations:** What’s included in terms of durable medical equipment, physical therapy (PT), occupational therapy (OT), vision, dental, etc.?

a great way for individuals to test the waters in a certain field or role, and find where their skills and passions align — or don’t.

“My first job was from a word-of-mouth lead,” says Dorszynski. “I interviewed for an intern position I heard about from a friend’s mom and got it.” Then, after he graduated from Stanford with a B.S. in civil and environmental engineering in 1997, that internship “led to a regular full-time job at the same place,” he adds.

Although this was just the first stop in a career that has since taken him into a new and unexpected field, Dorszynski’s story is an example of the importance of networking when it comes to job search and career

recruiting. It’s a lesson shared by Katrina Gossett, as well.

Gossett, who lives with **spinal muscular atrophy (SMA)**, knew she wanted to move closer to family in Indiana when she graduated from the University of Chicago Law School. So during her years in Chicago, she made a special effort to make contacts with individuals working at law firms back in Indianapolis. That networking groundwork helped Gossett land consecutive summer internships at Indianapolis’ Faegre Baker Daniels LLP. Like Dorszynski, she then graduated with a job offer in hand, and today, Gossett is living her dream as an associate in the firm’s business litigation group.

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Back to School

This article is part two of a two-part series on MDA Transitions. The first article, "A Bright Future," focused on how young adults with neuromuscular diseases have found success and a steppingstone to independence in college. Find it in the *Quest* archives at quest.mda.org.

Embrace change, find balance

Between company mergers and acquisitions, downsizing, relocations, and promotions, change has become a regular part of how we work in this day and age. Of course, voluntary change — taking a new job or jumping into an entirely new role or field — is equally common. All of which is to say that even though modern work life is not always smooth, with patience and persistence, you can build a career.

Sometimes, it's just not the one you originally planned.

After Dan Dorszynski's post-college internship, he felt driven to continue along a logical career trajectory, moving on to graduate school, which he did, before finding a niche working in civil engineering. But during that process, he discovered computer programming, and that changed everything. In programming, Dorszynski discovered not just a growing, in-demand field that appealed to him intellectually; he also found a profession that he feels is far more compatible with the progression of his BMD.

"Once I changed paths and started programming, I was less concerned [about being able to find and perform work effectively] because the job is 95 percent being in front of a computer. So whether I was walking or using a wheelchair, I could still do it," he says.

Today, Dorszynski works as a Web developer and programmer and runs a computer-based company with a friend.

Adapting a career path to the realities of a diagnosis is something that Dan Sedgeman knows well. With a B.S. in physical education from Southwest Minnesota State University and a master's in clinical exercise physiology/cardiac rehabilitation from Minnesota State University, Mankato, Sedgeman wanted to become a certified personal trainer — even if he knew that path might not be possible.

"Because I have **Duchenne muscular dystrophy (DMD)**, I didn't think I'd be working after college," he admits. "I picked a major I thought was interesting, and it gave me the opportunity to study muscles and muscle fibers."

As expected, Sedgeman's DMD eventually made him too weak to physically train clients. But he remained passionate about fitness, so he slightly reframed his outlook, as well as his career goals. Now, he works as a health coach, helping novices establish fitness programs by reviewing screening results for their health programs, as well as counseling them on developing their health and fitness goals.

Sedgeman has also found time to translate his academic and



"I PICKED A MAJOR I thought was interesting, and it gave me the opportunity to study muscles and muscle fibers."

— Dan Sedgeman

professional expertise into a personal passion project: Expressive Movements. This integrated dance program, open to those with and without disabilities, blends fitness, therapeutic benefits and social interaction. (Find out more at vsamn.org/expressive-movements-new-integrated-dance-program.)

For Sedgeman, who also regularly plays power hockey, an adaptation of the game in which participants use power wheelchairs, these recreational pursuits have provided a great way to indulge the specific passions he wasn't able to focus on in his career. Plus, he says, it helps make him more well-rounded and have better work-life balance.

Similarly, Dorszynski leverages the fact that he's self-employed to carve out time for his other passions, like wheelchair tennis and travel.

In her case, Gossett adds balance to her working life as an attorney by competing in ComedySportz, an improv comedy show/competition that has programs in many major cities. Before she found the law as a calling, Gossett was a theater major in college. So even though improv is by

RESOURCES + MILESTONES

October is National Disability Employment Awareness Month (NDEAM). This year, NDEAM celebrates its 70th anniversary. To find related disability employment resources, learn about the history of NDEAM and see how you can support its cause, visit dol.gov/odep/topics/ndeam.

This year also marks the 25th anniversary of the Americans with Disabilities Act (ADA). To learn more about the ADA and its impact, visit MDA's ADA Information Center at cqrengage.com/mda/ADAinfo and read an ADA-related Q&A with MDA Vice President of Policy and Advocacy Kristen Stephenson at quest.mda.org/article/ada-25th-anniversary.

A woman with long dark hair, wearing a blue long-sleeved top and patterned leggings, is sitting in a black and blue motorized wheelchair. She is smiling broadly and looking towards the camera. The wheelchair has 'F3' on the side. To her left, another woman is laughing and blowing streamers. The background is a party with colorful balloons (red, blue, yellow) and people. A large blue balloon with the text 'DON'T HOLD BACK' is in the foreground on the right.

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BEEN THERE - DONE THAT!

When it comes to getting information about some aspect of life you're about to embark on, there's nothing better than learning from the experiences of those who have already been there and succeeded. The MDA Transitions Center includes a blog written by other young adults who have successfully overcome many challenges associated with life with neuromuscular disease. Among the bloggers are some of the individuals mentioned in this article: Dan Dorszynski, Scott Drotar and Katrina Gossett. The Transitions Center also has a "Career Center" page with links to useful job search and career resources. To check out the Center's offerings, read a blog or submit your own story, visit transitions.mda.org today.

its nature spontaneous, not scripted, she says these classes have helped her "feed the theater bug" that still resides inside her. While Gossett admits she was initially terrified and thought she couldn't be funny consistently, she has come to thoroughly enjoy the classes — and even acknowledges that studying improv has benefited her in her day job.

"I would have loved to be an actress professionally," she explains. "I knew it was a very hard career to get into and hard to support myself. But I was a lawyer in mock trial in high school and enjoyed the trial drama. There's a little bit of theater in the law, at least when you're in the courtroom."

Blaze your own trail

Even if you're able to establish a healthy work-life balance, not everyone is interested in becoming an employee in the traditional sense. One reason could be that such a person has an entrepreneurial spirit and loves the freedom to follow his or her own muse. Another could be that this person has a neuromuscular disease that limits strength and stamina, making a standard eight-hour work day difficult or impossible. As it happens, in some cases it's a combination of the two.

Scott Drotar was diagnosed with SMA at 18 months old. He earned a mathematics degree at the University of Notre Dame and a master's in quantitative psychology from

the University of Kansas. After grad school, he started doing statistical analysis work, but he wasn't building the life he wanted. On a whim, he started a personal Web page and blog (ScottDrotar.com) and began writing about his life. Before he knew it, he had a growing following and invitations to speak to groups and earn a living by sharing his story.

Drotar has embraced this new path as well as the flexibility it affords him. Now, living in Kansas City, Kan., he has created a work schedule based on his personal needs, instead of those of an employer. While he still supplements work related to his website with statistical analysis projects on a freelance basis, his work day invariably begins about 1 p.m.

"With my medications and such, my body is more cooperative at that time," he says. "I usually spend the afternoon doing bookkeeping for my speaking business. Workplace stuff is important, but not the stuff I enjoy doing. When that's done, I write new blog posts, a new talk or work for one of my statistical analysis clients."

Drotar stresses that finding a schedule that best balances health and wellness with what's satisfying about a given job, while factoring in income needs and financial considerations, is key.

"It took me a while to know how to budget my finances for ups and downs and to structure my time," he says. "I am in charge of when I work and how much. If I have a day when my body is not working well, I can work less and not worry about upsetting a boss. I can take Tuesday and Wednesday off to feel better and work more hours on Thursday and Friday to get the work done, but not have to pay for it with my body."

Finding your independence

For those living with a neuromuscular disease, the transition into

the workforce often accompanies a larger transition from youth and dependence to adulthood and independence. Both types of transitions are a process that are unique to each individual. For Scott Drotar, the process of transitioning to independent living was intentionally gradual, and college marked a pivotal point along the way.

"It was great that I was able to gradually ease in to being a completely independent adult, and I am not sure I would have been this successful if I had jumped straight to living on my own," he says. "By first living in the dorms and learning how to function as a disabled adult in an able-bodied world, while still having the added support of the college setting, I was able to develop some of the necessary skills to live independently."

Just like the journey one goes on to find a meaningful career, becoming an independent adult is not always easy, but in the end, the rewards of doing so are worthwhile. So it's important to keep that perspective in mind and remember that sometimes what holds a person back isn't their actual limitations, physical or otherwise; it's their own natural fear of what lies ahead or even the fears of loved ones who don't want to see them get hurt.

"Once you set your goal," says Drotar, "don't let others — parents, siblings, friends, caregivers — tell you what they believe you can do. Everyone in my life told me, 'You'll never live on a campus in North Dakota,' but I refused to listen. I decided I've got this one life. It's mine to live. It's going to be shorter than most, so I've got to make the most of the time I've got. Even if I fail, I'm back where I started. Live your life for you — not everybody else. Dream big and go for it." **Q**

Donna Albrecht is a freelance writer and speaker in Northern California.

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Sarah Coglianesse (left), who lives with ALS, receives caregiver support from her sister, Liz, who lives around the corner.



MY BROTHER'S

(Or Sister's)

KEEPER

EXPLORING THE UNIQUE DYNAMIC — ITS BLESSINGS
AND OCCASIONAL HURDLES — OF ONE SIBLING CARING
FOR ANOTHER WITH NEUROMUSCULAR DISEASE

BY DONNA SHRYER

In

a small Detroit apartment, Sam Barash, 32, lives with and cares for his 20-year-old, soon-to-be 21, sister, Angela Barash, who is living with **myotonic muscular dystrophy** and **myasthenia gravis**. For a year now, Sam's been his sister's primary caregiver, waking her every four hours for medication, fixing her meals, helping her get dressed and doing everything possible to make Angela comfortable.

It wasn't Sam's original plan — but as he puts it, "Things happen." By that, among other things Sam is referring to the loss of his mother to cancer in 2014. Before passing, Mrs. Barash asked Sam to always be there for his sister. "I said, 'yes,'" Sam recalls. "I knew I'd have to give up a lot — like my 60-hour-a-week job and a pretty active social life — but when I agreed, it came so naturally. I even surprised myself at how much I want to be here for my sister."

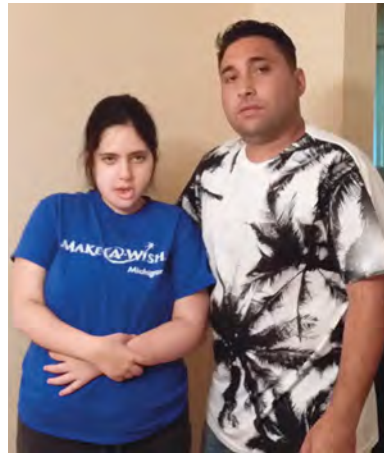
Up for the challenge

Sam Barash's life-altering decision is an increasingly common topic among American families with an adult child with a disability.

"Caring for a Sibling with a Disability," a study conducted by the market research firm Ipsos, represents a small but telling survey of adults who do not experience a disability but have a brother or sister who does. Of those surveyed, 24 percent currently serve as the primary caregiver for their brother or sister with a disability, and one in three plan to eventually become the primary caregiver for their sibling with a disability.

Like Barash, those surveyed see the responsibility as a positive move, with a resounding 83 percent feeling "comfortable" taking over as their sibling's caregiver and a strong 60 percent feeling "emotionally prepared."

Such positive responses may overlap with certain additional survey findings, including the fact that six in 10 respondents feel that caring for a sibling positively impacts their quality of life — helping them to develop



Angela Barash says she's able to best express herself with her brother, Sam, who serves as her primary caregiver.

patience, understanding, compassion and providing perspective.

Cheryl Ruhle, 54, recently became the full-time caregiver for her identical twin sister, Sharon Corosanite, who received a diagnosis of **ALS (amyotrophic lateral sclerosis)**. Together, the sisters live in a small one-bedroom central New Jersey apartment, sleeping in the same bedroom, just as they did as children.

Ruhle admits that every day is different, and many days bring new

challenges, but overall she recognizes that caring for her sister stimulated her own emotional growth.

"Caring for Sharon has taught me to live in the moment," Ruhle explains. "With ALS, there could be 10 more years of tomorrows or maybe only two years. So you can't say, 'Let's do this in three months.' You have to live for today. This perspective has helped me find so much joy in life — instead of focusing on loss."

Blessings and hurdles

Living in the moment, Ruhle insists, doesn't require exhaustive planning. Sometimes the most precious moments just come.

"Sharon brings the family together — that's such a blessing. And we're a loud, happy Italian family, so when we get together, fun follows," Ruhle says, with a smile in her voice as she names the crew. In addition to Cheryl and Sharon, there's Elizabeth (Libby) Hertkorn, Kelly Henry and Charles Henry. The siblings care for Sharon at various levels, each taking on what he or she can handle, although the family unit is always up for a playful gathering.

"A family picnic or dinner together aren't huge events, but they make Sharon smile," Ruhle continues. "Someday her smile won't be there, but for today — when we can watch Sharon's face light up — it's a blessing. It's such a beautiful smile."

For sisters Cheryl and Sharon, when there's a smile, laughter is always close behind. "Caring for my sister has given me a new appreciation for laughter," Ruhle says. "It's the best medicine! Not just for Sharon, but for me, too!"

While Barash and Ruhle find that caring for their respective sibling positively impacts their quality of life, they also admit that being a primary caregiver comes with hurdles.

"Many caregivers feel frustrated from time to time, and I can't say it



A HELPING HAND

To help caregivers embrace the joys and jump the hurdles when it comes to caring for a loved one, MDA provides a rich pool of resources, including caregiver-related support groups across the county. Another valuable resource is MDA's online caregiver section, which provides helpful information and resources to help support family caregivers. Learn more at mda.org/caregivers. To find a local MDA office and other resources in your area, visit mda.org/locate.

Additional organizations and programs to consult for caregiver support and services include:

- Administration on Aging National Family Caregiver Support Program
- Caring Connections
- Share the Care
- Sibling Support Project (Program core is Sibshops, which celebrates the many contributions made by brothers and sisters of kids with special needs)
- Family Caregiver Alliance
- Strength for Caregiving (AARP-supported; primarily for seniors)

EVERYTHING OLD IS NEW AGAIN

Caring for a sibling comes with a unique dynamic, explains MDA Health Care Service Coordinator Marissa Lozano. “Adult siblings argue from time to time — just like they did 20 years ago in the sandbox. It’s a fact that has nothing to do with whether or not one sibling has a disability.”

Many siblings experience firsthand this “spirited” sibling dynamic with every family Thanksgiving dinner or birthday gathering. Experts suggest that when adult siblings get together,

it’s natural to slip into old roles. They may assume labels long outgrown, like the “good” one, the “troublemaker” or the “micro-manager.” That can pull old feelings to the surface, because, as Lozano explains, “You let your guard down around family. That can make the good times even better, but you’re also likely to have feisty times, too.”

There is no simple solution to address the latter, although, as Lozano suggests, it helps to recognize these tendencies for what they are: yesterday’s news.

Instead, both siblings should focus on the gift they’re giving each other today. And if this feels like an insurmountable goal, it may be time to seek professional counseling.

“Everybody needs help from time to time,” Lozano says. “It shows great strength when you seek help. After all, if you don’t take care of yourself, you can’t take care of your sibling. If you’re the one being cared for, you risk losing so much shared happiness with someone who loves you so much.”

enough: That’s OK, and it’s perfectly natural,” explains MDA Health Care Service Coordinator Marissa Lozano, who’s based near Philadelphia. “It’s important to have a support group, a friend, a therapist or someone you can talk to, and a safe place to vent.”

In Ruhle’s case, she attends an MDA support group led by Lozano. “It gives me a chance to express feelings that I can’t explain to anyone else. In the support group, everyone understands right away.” It is, Ruhle adds, like entering a room overflowing with camaraderie — but never sympathy, which Ruhle stresses she does not want or need.

In addition to emotional hurdles, a sibling caring for a brother or sister with chronic or progressive conditions may hit financial obstacles. For Barash, who needed to quit his job to care for Angela, the solution was to become his sister’s paid caregiver. He reached out to LifeLong Advocacy, an organization created to help individuals with developmental disabilities. Sam attended classes through LifeLong Advocacy, and after earning certification as a qualified caregiver, he now cares for his sister and also earns a salary. (In many states, Medicaid and Medicaid-related state programs offer options for individuals

to earn compensation to supplement or replace other income when working as a caregiver for a loved one. Local MDA offices may be able to help locate additional resources.)

Ruhle, who is on family leave while caring full-time for Corosanite, hopes

her sister will soon qualify for a daytime nurse so she can return to work and continue caring for her sister during evenings and weekends.

“Some days, I sit and make phone call after phone call. It’s been a struggle,” Ruhle admits, “But I’ve had a lot



Sharon Corosanite poses with her daughter, Lindsey.



Family Caregivers

This November, as we celebrate National Family Caregivers Month, it's important to recognize these loved ones and the vital roles they play in the MDA community. For more tips for family caregivers, read "Caring for the Caregivers" in the *Quest* archives at quest.mda.org/article/caring-caregivers.

A CHANGING WORLD

According to a study conducted within the Department of Disability and Human Development at the University of Illinois at Chicago, an estimated 76 percent of individuals with developmental disabilities live at home with their parents. In 25 percent of these households, the caregiver is age 60 or older, and the average age of the adult child with a disability is 38 years.

It's important to note that because "developmental disabilities" are a group of conditions due to an impairment in physical, learning, language or behavior areas, this study covers a population far broader than the MDA community. But its findings do shed light on common family dynamics between caregivers and care recipients.

That said, two other facts impact the historically traditional path whereby parents care for an adult child with disabilities:

- New therapies, emerging research and advances in medical management are leading to longer lives for those diagnosed with neuromuscular diseases, which often extends that person's need for a caregiver.
- Many parental caregivers are today hitting their 60s or 70s. So it won't be long before these aging Baby Boomer parents will be unable, or at least less able, to care for an adult child with a disability. In fact, it's possible today for many older adults with disabilities to outlive their parents.

Combine these two realities, and the response is an upswing in other family members, often siblings, assuming the role of primary caregiver for a loved one with a disability.

her sister. Talking to her counselor, in a safe place, defuses her anger, so she can compassionately talk to Liz.

"I tell her that I feel bad about not being able to help out more," Sarah says, "and that I can see her frustration with all the work she has to do. It doesn't change anything, but I do think it helps when Liz knows how much I appreciate her."

Lozano strongly agrees that seeking outside, objective counseling can help those in similar sibling-caregiver relationships.

"It gives the individual a better perspective as to what the caregiver is feeling, which can tighten the bond between the caregiver and that person," Lozano notes. "That's why we recently launched two support groups, one for caregivers and one for the individuals living with a neuromuscular diagnosis. For those in the latter who are no longer able to speak, we offer support through an online community, since many of these individuals can share their thoughts by typing out their feelings and frustrations."

For both the caregivers in this dynamic and their siblings, life at times may be frustrating, a struggle or simply a joy. So finding ways to cope and vent about challenges in a healthy way, and truly cherish the good moments, is the key to maintaining individual peace of mind and a positive sibling relationship, they say. Aside from that worthy goal, there's just one thing that tends to annoy Sam Barash.

"When I talk to anyone about my choice to care for Angela, they always end the conversation with the same thing. They say, 'Good luck,'" he notes. "But luck has nothing to do with my choice. We make it through each day because we love each other. We're family." **Q**

Donna Shryer is a freelance writer in Chicago.

of help from Sharon's neurologist, and MDA and Marissa [Lozano], of course. She's been wonderful."

Care for caregivers

Open, honest communication is also a hot topic for Sarah Coglianese, 36, who received a diagnosis of

ALS in 2012. Sarah lives in the San Francisco area with Scarlett, her 5-year old daughter; her husband, Rob Goulding; and the family's new puppy, Otto. Liz Coglianese, Sarah's 32-year-old sister, lives around the corner with her husband and toddler son. Even though Sarah has a daily assistant, Liz plays a major role in her sister's care.

While Liz is there 100 percent for her sister — and Sarah knows this in her heart — rough patches occasionally bubble up. "I sometimes feel guilty, because I know this isn't how Liz and I expected things to play out," Sarah explains. "I'm not mad at my sister; I'm mad at the situation. I fear that she'll get sick of taking care of me. I know that is not true, but it's a pretty real feeling."

The solution, Sarah adds, comes down to a few from-the-heart discussions with her counselor and also with

For both the caregivers in this dynamic and their siblings, life at times may be frustrating, a struggle or simply a joy.



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● FOLLOW THE *biomarker*

IMAGINE BEING IN A STRANGE CITY. You're driving to the airport to catch a flight. Now suppose every street sign and traffic light you see is covered in paint. Which way do you turn? You look at your dashboard. All the indicators are covered in paint, too. Is the tank full or empty? Is the engine hot? Should you go faster? Slower? Or stop altogether?

The truth is you simply don't have the information you need to proceed safely and get to your destination.

That's a little like trying to help kids and adults affected by neuromuscular diseases without the benefit of very important tools called biomarkers. Biomarkers — including measurements such as blood pressure, heart rate or the presence of particular proteins in the blood or urine — are biological indicators or measurements that tell us about someone's health or the progression of a disease.

Biomarkers can provide crucial information for researchers conducting clinical trials, helping them determine at the earliest possible point in the process whether a treatment is effective. As such, MDA is strongly committed to advancing research to find and utilize specialized biomarkers that will accelerate progress for the families MDA serves.



kerns

BY PHIL IVORY

HOW IDENTIFYING THE
BODY'S KEY SIGNS AND
MEASUREMENTS SPURS
LIFESAVING PROGRESS IN
RESEARCH, CLINICAL TRIALS
AND THERAPY DEVELOPMENT



To learn more about all the new MDA research grants, check out MDA's Grants at a Glance slideshow at mda.org/gaag. And be sure to read the "Strength in Numbers" blog post about the new grants by MDA Senior VP and Scientific Program Director Grace Pavlath.

"MDA's research program places a strong strategic focus on the importance of identifying biomarkers linked to muscular dystrophy, ALS and related neuromuscular diseases," says Grace Pavlath, Ph.D., MDA senior vice president and scientific program director. In August MDA awarded four new grants, totaling just over \$1 million, to fund projects aimed at identifying biomarkers in **Duchenne muscular dystrophy (DMD)**. If successful, this research will lead to biomarkers that could help track disease progression, increase clinical trial preparedness, lead to improved outcome measures in clinical trials and facilitate trials that could accommodate nonambulatory patients.

Spinal muscular atrophy

In 2014, MDA awarded Stephen J. Kolb, M.D., Ph.D., an assistant professor in the departments of neurology and biological chemistry and pharmacology at Ohio State University, a human clinical trial grant as supplemental funding for an NIH study comparing infants with and without **spinal muscular atrophy (SMA)**.

"We are studying how SMA progresses during the first two years

of life and comparing that to how healthy infants develop," says Kolb. "In addition to measuring motor function, we are measuring potentially useful biomarkers that may accelerate and improve clinical trials in SMA."

Although the study focuses on infants, Kolb believes it may yield information applicable to older children and adults with SMA.

"With our data, investigators who are running and designing clinical trials in infants with SMA may be able to shorten the time it takes to determine if a therapy is really working or not," says Kolb. The study is expected to finish in spring 2016.

Facioscapulohumeral muscular dystrophy

Research funded by MDA has shown that abnormal production of a protein called DUX4 is a major molecular cause of **facioscapulohumeral muscular dystrophy (FSHD)**.

In 2011, MDA awarded a development grant to Fedik Rahimov, Ph.D., a postdoctoral research fellow at the program in genomics at Harvard Medical School and Boston Children's Hospital, to support his work seeking to further illuminate

the molecular mechanisms underlying FSHD. That included looking at destruction that occurs in FSHD that may be due to activity of *other* genes set in motion by DUX4.

"Genes that are induced by DUX4 are those that likely play a role in disease initiation and progression in FSHD," says Rahimov. "These genes could serve as disease biomarkers as well as targets to develop new therapies."

Using muscle samples collected from more than 20 families, the study identified

several genes that can be used as muscle biomarkers for FSHD.

"Genes that are either significantly up- or downregulated in affected muscles relative to control muscles could serve as potential disease biomarkers," Rahimov adds. "Such biomarkers tend to be more accurate and can be used to develop therapies and monitor the success of these therapies in preclinical and clinical trials."

ALS

Michael Benatar, MBChB, M.S., DPhil, chief of the neuromuscular division and professor of neurology at the University of Miami, Miller School of Medicine, has received past funding support from

MDA for his research seeking biomarkers in ALS. His work focuses on collecting critical data from individuals who are at risk for ALS due to genetic factors but who have not yet shown clinical symptoms. The

ongoing study uses comprehensive testing ranging from blood samples to magnetic resonance imaging.

Finding subjects for the study who are at risk for ALS but not yet symptomatic required Benatar and colleagues to seek out individuals with the familial (inherited) form of the disease. However, Benatar believes his work will have implications for the larger ALS population, both those with the familial and sporadic (noninherited) forms of the disease. He's seeking biomarkers that can both predict disease onset and monitor progression.

Benatar says: "We've been interested in studying people before they develop the disease, to understand



ALS Biomarkers

For more on this topic, enter "Looking for a Sign: Biomarkers in ALS" in the search box on mda.org.

Fedik Rahimov is working to identify biomarkers that may help speed therapy development in FSHD.



such things as: When does that disease begin? How does it unfold? How does it progress? And are there opportunities to intervene early?"

"If we can figure out ways to identify early disease or people at risk for disease, I think that offers a really unique opportunity for early therapeutic intervention or even disease prevention," says Benatar, who also co-directs the Kessenich Family MDA/ALS Center at the University of Miami.

Collaboration, commitment

The FDA issued a report in June titled "Targeted Drug Development: Why are Many Diseases Lagging Behind?" which emphasized that, for many rare diseases, the molecular and genetic underpinnings are not sufficiently understood to allow effective therapeutic intervention. Part of this challenge, the report said,

involves the need for more and better biomarkers that can help make clinical trials more efficient and successful.


As part of MDA's national advocacy effort, and in response to an FDA request posted in the Federal Register, MDA collaborated with the Friedreich's Ataxia Research Alliance (FARA) and Cure SMA to provide the FDA with a report on the current state of research pertaining to biomarkers in neuromuscular diseases.

"The FDA appreciates that biomarkers are critical and is seeking information about which biomarkers could be good candidates for validation, which we're glad to provide," says Kristin Stephenson, MDA vice president of policy and advocacy.

"We are deeply committed to working with government agencies and our friends at other nonprofit organizations to optimize awareness of the crucial importance of biomarkers

across the full spectrum of neuromuscular diseases — and we appreciate that the FDA has invited the community to weigh in on this important topic," Stephenson continues.

In addition to the four new MDA research grants aimed at discovering biomarkers in DMD, promising research into biomarkers is also taking place in **Friedreich's ataxia (FA)**, **inclusion-body myositis (IBM)** and other diseases across the neuromuscular disease spectrum.

As MDA's Pavlath notes: "Biomarkers hold huge potential, from enabling earlier detection and better treatment to streamlining the drug discovery process so that lifesaving therapies will be found more quickly for the families we serve." 

Phil Ivory is MDA's senior writer for the Communications and Marketing team.



What's a Biomarker?

Learn the answer to this important question in the **Quest** archives at quest.mda.org/article/whats-biomarker.

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Rest Easy

Products and tips to improve your sleep and maximize energy and comfort during waking hours

BY BETH HOWARD

IT'S A CATCH-22: Getting a good night's sleep is an important way for people living with neuromuscular diseases to help maximize their health and well-being. But the physical symptoms of many of these diseases can themselves limit the restfulness of sleep.

"We tend to overlook the importance of sleep; it's so basic," says Sharon Greenberg, an occupational therapist in the MDA Clinic at Seattle Children's Hospital. "But getting good rest can be a real challenge for people with these diseases."

Immobilized joints tend to get achy and uncomfortable. And although most people change positions or stretch frequently during sleep without even being aware of it, those with limited mobility can't easily move during the night to relieve the pain and numbness that result from staying in the same spot hour after hour.

"It's difficult for people with neuromuscular disease and difficult for caregivers who have to get up frequently to help them change position," says Jacqueline Montes, a physical therapist and assistant professor of clinical rehabilitation and regenerative medicine in neurology at Columbia University (site of an MDA

clinic) in New York City.

People who stay in the same position also risk developing pressure sores.

"Sometimes patients are in a deep sleep and don't notice the pressure, and body parts can become numb when you take away circulation for any length of time," adds Greenberg. "Some patients don't have a lot of padding on bony [areas], and the skin can break down pretty quickly."

Improving sleep is usually a process of trial and error, often starting with low-tech aids, such as mattress overlays made of convoluted foam, gel or air pockets that help to distribute pressure evenly during sleep to relieve pressure or make turning easier for those with some mobility. "There are some simple foam overlays from places like Bed, Bath and Beyond that can be helpful," says Greenberg.

The Invacare Gel Foam Mattress Overlay (invacare.com) is a step up in price (\$545) and sophistication. Constructed of 24 horizontal "bladders" filled with a non-toxic gel, it's secured on

Invacare Gel Foam Mattress Overlay



RESOURCE CORNER

OTHER SLEEP-FRIENDLY SOLUTIONS

PROBED FREEDOM BED

The Freedom Bed from ProBed Medical Technologies uses programmable computer controls to turn the user through a range of stable sleeping positions. The angles and times of the turns are determined in consultation with the user's caregiver and health care team. The bed also features an air-powered head- and leg-raising system that allows the user to be raised into the sitting position.

More: pro-bed.com

How much: Contact vendor for details



SLEEPSAFE SAFETY BED

PT Jacqueline Montes of Columbia University often recommends the SleepSafe Bed for children. The beds prevent a user from becoming entrapped between the mattress and frame, as there is practically no space between the mattress and the side rails, headboard and footboard. The beds use CertiPUR-US mattresses made of memory foam infused with gel polymers, all designed to reduce the incidence of pressure sores. Customers choose from fixed, articulated or hi-lo frames.

More: sleepsafefed.com

How much: \$5,000-\$13,000



top of any mattress to prevent pain and pressure sores. Likewise, the Stimulite On Top Wellness Mattress Cover from Supracor (supracor.com) is made of a unique bioengineered “honeycomb” material that distributes weight uniformly across the surface. The unit’s cellular matrix consists of alternating thick- and thin-walled cells that flex when compressed to relieve pressure. Prices range from \$1,400 to \$2,000.

For those with greater mobility limitations, there are the Volkner Turning System mattress overlays from James Consolidated (volkner.com). The units sport a double row of air cells divided in the middle to provide relief from pressure along the central axis of the body. Air pumped automatically through hoses alternately inflates and deflates the two sides, turning the sleeper every four to six minutes.


Other options come from the wider consumer market. “A portion of our patients like Tempur-Pedic beds,” says Montes. The memory-foam mattresses (tempurpedic.com), which range from \$1,700 to \$7,500 in price, are designed to reduce pressure points. Adding the company’s Tempur-Ergo Adjustable Base (\$1,400–\$4,900) lets users or their caregivers select from an infinite number of rest positions using a wireless remote.

“A lot of folks are going to Sleep Number beds,” (sleepnumber.com), adds Pam G. Glazener, a senior occupational therapist at the MDA/ALS Center at Houston Methodist Hospital. She offers

this tip: “You can adjust them to the firmest setting. That makes it easier for people who can roll to move into a comfortable position for sleep. Then they can adjust the bed to a softer setting for sleep.”

On the upper end of the scale, the Neuropedic Electric Adjustable Bed (neuropedic.com) offers easy adjustment of head and foot positions with the touch of a button. It enhances circulation and reduces tension in sore muscles with a gentle vibration system. The Neuropedic CC 2000 Series Sleep Set pressure reduction mattress, made of a special foam that reduces the likelihood of pressure sores, completes the setup.

If price is a concern, comfortable air mattresses and beds can sometimes be found in community-based equipment assistance programs, such as MDA’s equipment inventory program, or at local resale stores. Coverage through Medicare, Medicaid and some forms of private insurance require that medical necessity be well-documented.

Unfortunately, Medicare doesn’t usually cover the costs of beds that help pressure ulcers unless a user already has two active stage wounds. If you can get a doctor’s prescription, however, in many cases taxes can be waived. Says Glazener, “Every little bit helps.” 

Beth Howard is a freelance writer in Charlotte, N.C.

RESEARCH CAN MAKE A DIFFERENCE



Since its conception, the FSH Society has committed to act as a driving force in the development of research directed toward treatment and a cure of facioscapulohumeral muscular dystrophy (FSHD). Learn more at FSHsociety.org.

FSHSOCIETY.ORG

YOUR RESOURCE FOR FACIOSCAPULOHUMERAL MUSCULAR DYSTROPHY

Bryce Madsen's Team Bryce at the 2015 Twin Cities Muscle Walk.



+ This year, approximately 150 Muscle Walk events will take place across the country, featuring more than 12,000 teams and 40,000 registered participants coming together to walk and raise money for MDA. To get involved in a Muscle Walk for 2016, visit mdamusclewalk.org.

Why I Walk

“There isn’t a day that goes by where Bryce doesn’t apologize for ‘being the way he is’ or for having to ask for help. As a mother, it simply breaks my heart that Bryce feels this way, and there isn’t much I can do to help him feel better, nor [do I] have a good answer to his questions. But I can be fierce and try to raise as much money as possible so he can go to MDA Summer Camp, where for six days these questions never enter his mind. And I can be relentless and raise as much money as possible to get a step closer to finding a treatment and maybe even a cure for this devastating disease.” —*Kelly Madsen, mother of Bryce, who was diagnosed with spinal muscular atrophy (SMA) at 15 months.*

Members of Kappa Alpha Order support the MDA Muscle Walk.



Brotherly Love

National fraternity Kappa Alpha Order celebrates 40 years as an MDA partner

Since 1975, the Kappa Alpha Order fraternity has partnered with MDA as a national sponsor to raise funds and awareness for muscular dystrophy and related diseases that limit muscle strength and mobility. Kappa Alpha has raised more than \$2.7 million for MDA and participated in a wide range of MDA events, including Muscle

Walk, Pin-Up programs like MDA Shamrocks, letter-writing campaigns and other special fundraisers they host. The fraternity also has a personal connection to the cause, as former MDA National Youth

Chairman Luke Christie is an alumnus of the chapter at Furman College in South Carolina.

“This partnership is really important,” says Tracey Gianelli, MDA national vice president of organizational partnerships. “One of the things we’re proudest of is it’s a group of young guys, a fraternity, that has stuck with MDA and supported us. We’re fortunate to have them as partners for so long, and it helps us because we know we’ll have ongoing support from them. It’s not just this year [that they’re fundraising for us], but they support MDA year after year.”

+ MDA is proud to work with incredible partners and local businesses to build stronger communities and uncover urgent answers for families who are desperately counting on us. To meet MDA’s Partners in Progress, visit mda.org/partners-in-progress.

“Kappa Alpha Order is proud of its 40-year partnership with MDA. Service to our communities is one of our core values, and we know that giving back is a significant component of a man’s development in college. This relationship provides each of our 129 chapters across the country a unique, yet common support focus, in a national philanthropy.” —*Larry Stanton Wiese, Executive Director*

A Math Champ

Ben Lou earns a gold medal in the World Math Team Championship without a pen or paper

For Ben Lou, who received a diagnosis of **spinal muscular atrophy (SMA)** around his first birthday, going to China and speaking in front of 1,000 of his peers at the fifth annual World Math Team Championship last fall didn't really make him nervous. It was the math competition itself that he was worried about.

"[The speech] wasn't as nerve-wracking [as the competition]," says Lou, 12, who lives with his mother, Jenny Huang, in Southern California. "I had just come back from the Great Wall and took a two-hour nap, so I had to be dragged awake to make it in time to the ceremony [where I gave the speech]. My voice was a little croaky and I wasn't very loud, but luckily it was translated into Chinese by someone with a louder voice."

Lou spoke to the crowd about his life and family in California, and about those living with SMA in China, to help bring more public attention to the disease and the need for better educational rights for



PHOTO COURTESY OF ZEENA GREGG PHOTOGRAPHY

becoming a member of the U.S. Junior Team for the World Math Team Championship in Beijing, which was held in November 2014.

Putting Lou on the roster ended up being a smart decision, as he was the only member of the American team to receive a gold medal, and individually he scored 13th out of a total of 271 competitors. While most would be ecstatic with those results, Lou actually felt he could have done better.

insurmountable problem for him.

"I guess I just have more practice than most people, and some talent," Lou says humbly when asked how he does complex math in his head. "Because I can't really write, I'm forced to do it in my head, and that's how I got good at it."

Since November, Lou has participated in other math competitions, one of which was the Math Kangaroo International Competition in March, where he had a perfect score. He was also one of very few sixth-graders asked to attend the 40th annual American Regions Mathematics League Competition at the University of Nevada, Las Vegas in May, where he was a part of the San Diego team.

But math isn't the only thing that Lou enjoys. He has a wide range of interests that includes writing fiction and poetry, watching sports (particularly tennis), learning about science and the universe, and eating and observing the fresh fruits and vegetables that Lou and his mom grow in their yard. As far as the future is concerned, along with competing in more math events, Lou is interested in getting involved in science and inventing.

"I have several ideas," he says. "My most recent idea is a wind-powered car. Everyone's thinking about solar-powered cars, but I don't know why no one thought of wind-powered cars."

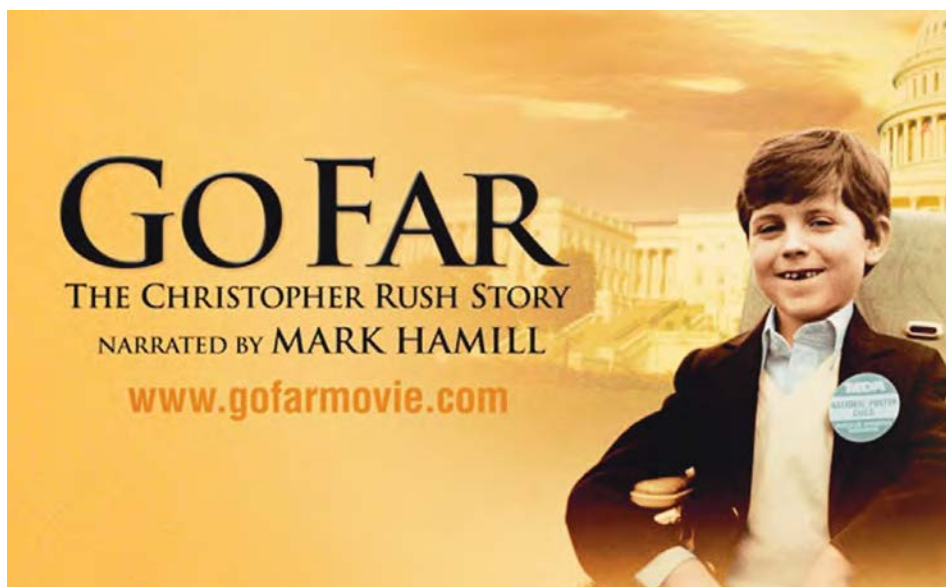
"I guess I just have more practice [at mental math] than most people, and some talent. Because I can't really write, I'm forced to do it in my head, and that's how I got good at it." — Ben Lou

people in China living with a disability. According to a 2013 Human Rights Watch report, more than 25 percent of children with disabilities in China do not receive even a basic education.

Lou received this opportunity by catching the eye of a recruiter after scoring in the top 2 percent of competitors in the American Mathematic Competition 10, at just age 10. This eventually led to Lou

"I made a stupid error where I only wrote one answer when there were two," he says. "If I hadn't made that error, I might have gone way higher."

Lou's success is even more impressive when you consider that he does all of this math in his head. Because of his SMA, he can't work problems out on paper the way the vast majority of his peers can. However, that clearly hasn't been an



“When [Rush] was being eulogized, I was astounded. Someone mentioned there is a [goal-setting] program he was working on called ‘GO FAR,’ and that hit me like a lightning bolt. That began the journey of making this documentary.”

— Zack Arnold

Going the Distance

A Hollywood film editor creates an inspiring documentary about his friend and former MDA National Goodwill Ambassador



Zack Arnold

When Zack Arnold, a film editor currently working on season two of the hit FOX TV series “Empire,” attended his friend Chris’s funeral in 2007, he had no

idea it would inspire him to create a documentary. Arnold’s friend was Christopher Rush, a former MDA National Goodwill Ambassador. Diagnosed with muscular dystrophy at seven months, doctors told Rush’s parents he would barely make it to age 2. Defying the odds, Rush lived to age 30 and achieved many of his goals, such as learning to scuba dive, graduating from law school and spreading positive messages about people living with disabilities.

Arnold first met Rush in a film production course at the University of Michigan.

“Other students were saying ‘I hope he’s not in our group,’ and that made me angry,” says Arnold. “So I approached him and invited him to join our group, and that was easily the single most life-changing decision I’ve ever made. He’s the best

producer I’ve ever worked with and he has a great sense of humor, and since then we were personal friends.”

While Arnold and Rush’s friendship continued past college — the last time Arnold saw Rush was at Arnold’s wedding, where Rush was a groomsman — Arnold didn’t realize how much Rush had accomplished throughout his life until he heard people talking about Rush at his funeral.

“I knew very little about what is in the film when he was alive,” says Arnold. “When he was being eulogized, I was astounded. Someone mentioned there is a [goal-setting] program he was working on called ‘GO FAR,’ and that hit me like a lightning bolt. That began the journey of making this documentary.”

So Arnold had his idea and his title, however the rest of the creation of the documentary, which is his directorial debut, did not come as easily.

“I can’t even begin to list [the challenges],” says Arnold. “Nobody believed that without a movie studio or distribution or money that you could tell the story properly. But I really ended up using [Rush’s] GO FAR program where you take your ultimate goal and break it down into small, achievable tasks.”

Doing this, Arnold put the documentary together in bits and pieces over the last seven and a half years, jumping over each new hurdle that came up along the

way. Eventually, he was able to pull all of the pieces together, even securing Mark Hamill as the narrator of the film — a fitting role because Rush was a huge “Star Wars” fan and loved the idea of The Force. Hamill not only donated his fee on the film back to the project after hearing about its inspirational backstory, but he also helped Arnold get the rights to use footage from “Star Wars” in the film, which many people told him would be impossible to do.

Now that the film is completed, Arnold is enjoying sharing it and Rush’s message with a wider audience.

“Chris was someone who was really good at distilling things down, and he loved quotes,” says Arnold. “The film has a lot of external quotes, but the one that I really think synthesizes the film is from Chris: ‘Everyone has a disability.’ [His may have been more readily noticeable,] but every person has some form of disability, and he wanted to inspire people to realize they could overcome these disabilities and achieve their goals. That’s where GO FAR comes from.”

+ For more information about the film, including how to host your own local screening, visit gofarmovie.com. A portion of the proceeds from the film will go toward funding a scholarship in Rush’s name.

Do a Little Time, Do a Lot of Good

Consider going behind bars to support MDA through a Lock-Up event

In MDA Lock-Up events, local business and community leaders agree to be “arrested” for having big hearts. These do-gooder jailbirds then encourage their family members, friends, co-workers and neighbors to get involved and help raise “bail” money — which supports MDA’s lifesaving mission.

Many Lock-Up events allow participants to meet MDA families.



MDA Lock-Up offers participants a unique, playful way to rally communities to raise funds for people with neuromuscular diseases, helping accelerate progress in research, care and support for MDA families in hometowns across America.

+ To get started, simply email lockups@mdausa.org to express your interest, and an MDA representative will respond and provide all the details you need to launch your own Lock-Up event and start fundraising. For more details, visit mda.org/lockup.

Fighting the Flu

MDA offers flu shot reimbursement and more

Flu season is approaching, and for people with neuromuscular disease, it’s particularly important to be prepared because influenza can be a serious and sometimes life-threatening illness. However, be sure to talk with your doctor before receiving the shot, as you would with any vaccine.

If your current health coverage doesn’t include the flu vaccine, MDA’s Flu Shot Program will reimburse the cost of your flu shot up to \$35.

+ For more information about the program and the flu in general, visit MDA’s Flu Season Resource Center at mda.org/flu-season-support.

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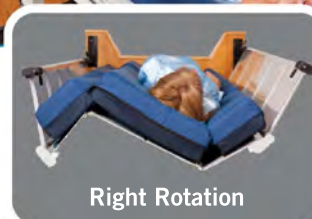
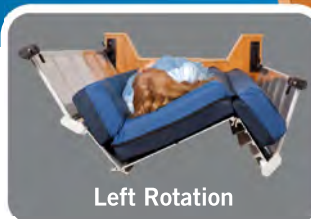
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Wish Granted

MDA family goes on a Hawaiian adventure, thanks to Make-A-Wish

When 9-year-old James Albaugh, who lives with **Duchenne muscular dystrophy (DMD)**, first learned about volcanoes in school, he was fascinated by the idea of these lava-spewing mountains. While he enjoyed learning more about volcanoes online, James longed to see one up close and in person.

“One thing I want to point out is how amazing everyone was to us when they found out we were part of Make-A-Wish. They accommodated us in every way possible.”

— *Jamie Albaugh, James' mom*

The Albaugh's Make-A-Wish Hawaii trip included siteseeing at a volcano.



For many, even turning this page is a breakthrough.



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And recently, Make-A-Wish granted that very wish, making it possible for James and his family to travel from California to Hawaii.

The Hawaii trip, attended by the entire Albaugh family — James' mom and dad and three siblings, one of whom, 5-year-old Jayden, also has DMD — included, of course, a chance to see volcanoes. But the family also enjoyed snorkeling, a helicopter ride for better volcano viewing and the water slides and pool at their resort.

The trip marked another exciting adventure in James' summer, as he also attended MDA Summer Camp, where archery, the talent show and meeting members of the MLS' Los Angeles Galaxy were some of his favorite activities.

+ Read an extended version of this story at quest.mda.org. For information about Make-A-Wish referrals, consult your MDA clinic team and visit wish.org/refer-a-child, or wish.org/local-chapters to find your local chapter.



The National ALS Registry: Get The Facts

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

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



Who can sign-up?

Anyone with ALS

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



What do I need?

- A computer with an internet connection
- An email address



What if I need help?

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



What kind of information is collected?

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



Do I need to update my information?

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



Will my information be private?

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



YOU JOINING





An avid cyclist recently diagnosed with ALS, Ray Spooner is biking across the country to raise money for MDA.

Making His Way

Ray Spooner raises money for MDA with a cross-country bike ride

When Ray Spooner, a certified nurse-midwife from Urbana, Ill., visited with his neurologist in January to discuss his recent **ALS (amyotrophic lateral sclerosis)** diagnosis, the doctor suggested it may be time for him to start checking things off of his bucket list. Spooner took

that advice to heart and began prepping for a coast-to-coast bike ride. "I'm a nutcase for cycling," he says. "I've been assured by most people that riding 100 miles before lunch isn't normal human behavior."

Spooner decided to use MDA's new Your Way for MDA

program to raise money through his ride to help support MDA's efforts to find treatments and cures. Your Way for MDA gives MDA community members the ability to set up their own special fundraising projects online and solicit donations through social media and email, as well as in-person requests. Spooner and his family and friends, some of whom will be making the journey with him, either riding along or providing support, raised more

than \$25,000 before departing for the more than 3,000-mile bike ride from San Diego to St. Augustine, Fla., this October.

"MDA supporters can turn their everyday activities into a fundraiser with Your Way for MDA," notes Natalie Stamer, MDA's national vice president of online fundraising. "No one is restricted by organization-led events, but instead, supporters can use their imagination and raise money to help find a cure for people with neuromuscular diseases."

+ Do you have a great idea for a fundraiser? To find out how you can launch your own Your Way for MDA fundraiser, visit mda.donordrive.com. Find Ray Spooner's campaign by searching "Spooner" on the site.



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Boston Marathon
April 18, 2016

Colfax Half & Full Marathon
May 15, 2016

Michelob Ultra Chicago Spring 13.1
May 22, 2016

Boston's Run To Remember
May 29, 2016

+ To join or support MDA Team Momentum, visit mdateam.org.

“Six years ago, I lost my brother, Kyle, to Duchenne muscular dystrophy. He was one of the most amazing people I have ever known, and he is my motivation. Despite his disability, he had a joyful spirit and an attitude of determination. When I grow tired during training, his memory keeps me going. I run for those who can't — to bring hope and joy to those with muscle disease. Being a part of Team Momentum feels right. The opportunity to train alongside my teammates who are also running for a cause so close to my heart has been amazing, and I feel so honored to be a part of this incredible team.”

— Emily Garcia, Team Momentum member running in the 2015 Chicago Marathon (also ran in 2014 Chicago Marathon)



Emily Garcia (right) and Team Momentum members at the 2014 Chicago Marathon.

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*Limb-girdle muscular dystrophy. Sponsored in part through a grant from Genzyme Corporation, a Sanofi company.

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Rob Curry (right), an owner-operator of 19 Jiffy Lubes, climbed Mt. Kilimanjaro with his son, Bobby, as an MDA fundraiser.



Conquering Kilimanjaro

A Jiffy Lube owner/operator and his son make the climb to raise awareness, funds to help fight neuromuscular disease

Rob Curry, owner and operator of 19 Jiffy Lube locations throughout Southern California, has long been a supporter of MDA through his business and the MUSCLE UP

campaign. Recently however, he made his MDA connection more personal. He and his son, Bobby, decided to challenge themselves to hike Mount Kilimanjaro in Tanzania — which at over 19,000 feet is the highest mountain in Africa — as a fundraiser for MDA.

“We had been thinking about doing a long hike, and it coordinated with [my

son’s] graduation from high school,” says Curry. “We thought it would be great to partner with a group, and MDA came to mind because of Jiffy Lube and the muscle connection. It goes hand-in-hand.”

Even though Curry and his son are both Eagle Scouts, they still took training for the climb very seriously.

“I lost 20 to 25 pounds, and I was training almost every single day in the last six months,” Curry says. “At Jiffy Lube, we do a lot of goal-setting, and I took this on as if I was going to do another project at work. Every month I would sit down and see [where I was physically], and if I didn’t make my goals, I knew I would have to reset the future goals.”

Curry attributed their success on the hike, which culminated in them placing an MDA flag at the summit of Mount Kilimanjaro on July 10, to this planning. And while placing the flag and raising over \$6,000 for MDA — their goal was one dollar for every meter of Kilimanjaro’s 5,985 meter height — were certainly highlights of the hike, Curry also relished spending time with his son.

“After a hike we’d get to camp and have dinner and have two or three hours to play chess, hang out, talk and things like that,”



he recalls. “At home, he’s a senior in high school; there’s a lot of stress of applying to college and playing sports, but on the hike we just had one goal: finishing the hike.”

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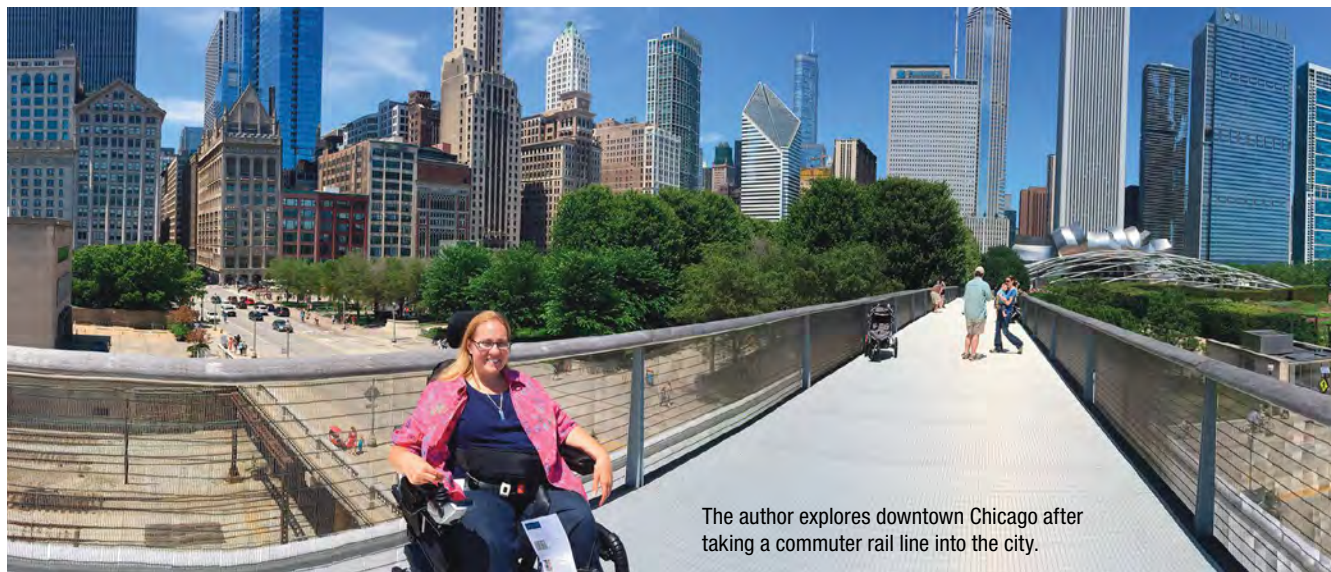


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Overcoming the Unknown

Sometimes conquering fear of the uncertain and finding a world of possibilities is just a train ride away



The author explores downtown Chicago after taking a commuter rail line into the city.

HAVE YOU EVER FELT TRAPPED, isolated or suffocated by transportation-related issues? I'm sure those of you who use a wheelchair, like I do — I have **spinal muscular atrophy** — or rely on some other form of mobility aid have felt these things a time or two. I know I have. But in the past couple of years, I've been determined to face my greatest fear, which includes transportation and so much more: risk-taking.

I love reliability and dependability. I do things I know I can do, and I try things that I'm at least 95 percent certain I will be able to do. But the things I'm really unsure of can take me months, or even years, to work up the courage to do. For me, certain forms of travel always held this level of especially scary uncertainty.

My parents and I moved to Illinois in 1999 after I graduated from George Mason University in Fairfax, Va. Northern Virginia is a pretty wheelchair-accessible place. They have taxis, trains and buses specifically for wheelchair users. Interestingly, when I moved to Illinois, the unknown created an internal fear that things wouldn't be as accessible. And frankly, at that time, the level of accessibility in Chicago and its surrounding suburbs wasn't as comprehensive as it is now. At the same time, I was really excited and felt very privileged when I received my driver's license and my first modified minivan in 2001. However, the density of downtown Chicago traffic and the expense of paying for accessible parking were too much for me. I've never acquired the confidence to drive in that kind of traffic, and I'm unwilling to pay such outrageous prices for parking.

For years, I had been deeply yearning to try the Metra train system, the Chicago metro area's commuter railroad, but I was too afraid. I knew from experience that northern Virginia's equivalent Metro train was somewhat accessible, but I recalled that you had to cross a gap between the platform and the train to ride. This gap was only about 3 to 5 inches wide, and my previous power wheelchairs had big wheels in the front and back, so this



I love reliability and dependability. I do things I know I can do, and I try things that I'm at least 95 percent certain I will be able to do.



was never an issue. However, my more recent wheelchairs, including my current one, have smaller wheels, which made me nervous about getting caught in a gap like this. In fact, I had such an intense fear of this happening that I refused to even go to a Metra train station here in Illinois to see if that's how its system worked.

Can you imagine getting your tires stuck in this gap? I'd have nightmares of being run over by a train. It was frightening.

PHOTO COURTESY OF JACQUELINE MARIE JOHNSON


Let's jump to July 2015. A good friend of mine lives in Chicago. He loves downtown and all the sites you can see. I confessed to him that I secretly longed to ride the train and explore the city, and that for the last year I've been trying to work up the courage to just figure it out and go. "What's stopping you?" he asked. I replied with a list of excuses that were wrapped around my fears. Afterward, he visited Union Station in downtown Chicago and talked with the conductors about the process of traveling. He even took pictures of the platform and a proposed walk we'd take from Union Station to the Art Institute. He also made a video showing me parts of the journey.

Not long after that, on July 12, I faced the uncertainties and conquered my fear. My friend's support and encouragement before and during the entire trip helped me see the limitless possibilities of the Metra train system. Of course, there is a process we have to take, and it's best to know that process ahead of time.

Yes, there is a gap between the train and platforms at some stations, but Metra trains are equipped with lifts that extend out of the train onto the platform. You simply ride onto the lift base, and it brings you up to the entrance of the train car. You can either go left or right into the car of your choosing. There are even places for your wheelchair. However, there are no straps to hold you down. For me, this didn't really matter. I felt safe and secure. There aren't any seatbelts for the other riders either; we're all the same. A conductor walks through the cars asking

for tickets; you either pay for one ahead of time or do so on the spot.

Our destination that day, Union Station, had ramps and was very accessible. And that was it! I was in downtown Chicago, which has crosswalks and cutout sidewalks on every corner. There also are accessible buses with fairly convenient schedules, as well as wheelchair taxis, but you do need to call ahead a day or two to schedule that option.

It's incredible all the things I can do now. I've come to realize that facing fear with a bit of love, support and encouragement from someone special can make all the difference in life. So my advice to everyone reading this? Never let fear stand in your way of seeing the possibilities that await you. Exploration increases enlightenment, self-confidence and self-worth. So go for it — take the risk and enjoy the adventure today! 

Jacqueline Johnson is a leading expert in holistic counseling, life coaching and spiritual well-being. Her focus is on reconstructing self-related issues to bring about total and long-lasting changes. For details, visit jacquelinemariejohnson.com.



Transitions on Transportation

On the MDA Transitions Center, bloggers, including Jacqueline Johnson, delve into topics related to life with a neuromuscular disease. From inspiring personal journeys to practical travel tips and more, find a story that moves you at transitions.mda.org/blogs.



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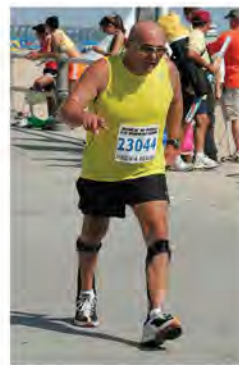
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Swimming, S'mores and More

A first-time MDA summer camper reminisces about his unforgettable experience



THE EXCITEMENT THAT SURROUNDS MDA Summer Camp is evident from the energy and smiling faces of the campers. That's perhaps especially true of first-time campers. For 10-year-old Axel Vazquez-Solis of Pennsylvania, who was diagnosed with **Ullrich congenital muscular dystrophy** in 2009, that excitement lasted all week long as he enjoyed the many pleasures of camp life — swimming, singing, meeting new people, having a friendly food fight and even taking motorcycle sidecar rides.

"I liked riding on the motorcycles," Axel beams. "I like to feel the wind when I'm going fast!"

Along with his mother, Yessenia Solis, and younger brother, Nicolas, who also has Ullrich congenital MD, Axel also participates in the MDA Muscle Walk to help raise money for MDA programs, including summer camp. This year, the family raised more than \$2,000 for the Greater Philadelphia-area Muscle Walk.

+ To learn more about MDA Summer Camp, including an FAQ for parents, visit mda.org/summer-camp. And if you or your child is a camp veteran, we'd love to hear your camp stories; email us at mycampstory@mdausa.org.



Still bubbling with excitement from this year's summer camp, Axel is already thinking about going back next year. When asked what he is most looking forward to, Axel says that while every day and activity was special, there is one thing that stands out. "The campfire and the s'mores," he says. "[Sitting by the] campfire and roasting marshmallows was a lot of fun." **Q**

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