



MYOTONIC
DYSTROPHY
FOUNDATION

Care and a Cure



MDF is 10!

- ❑ We're celebrating 10 years of Care and a Cure for DM
- ❑ Together we have built a best-in-class advocacy organization that is the largest in the world focused solely on DM
- ❑ So it's time for cake!





MDF is 10!

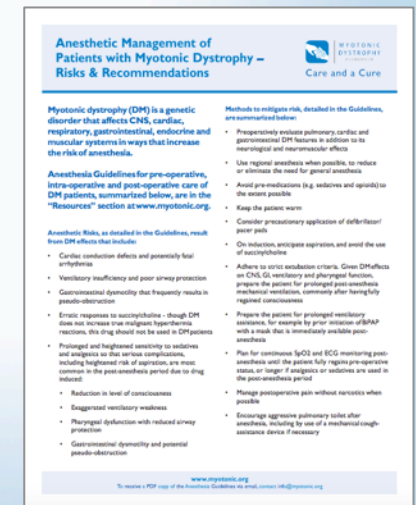
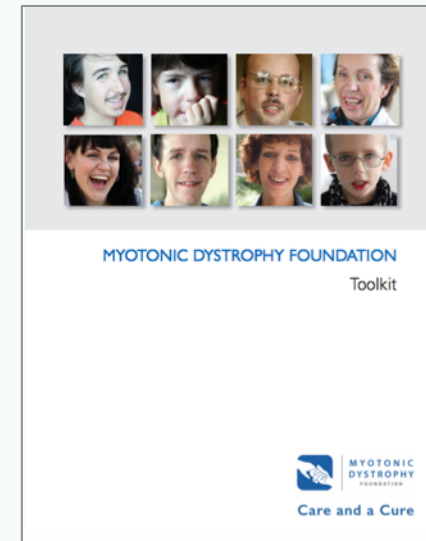
- Together we have:
 - Created community of over 14,000 members living with and working on behalf of DM care and treatment
 - 100% increase since early 2012; growing an average of 1,500 new members per year
 - More than 13,000 family members and donors
 - Over 1,100 DM professionals
 - 62 countries from around the world

- Launched Care and a Cure platform to improve quality of life and accelerate the search for treatments



10 Years of Care

- Launched a comprehensive and growing array of support and education programs
- Warmline serving 5-15 people a week through our Program Director
- MDF Toolkit, Anesthesia Guidelines and other tools to protect and improve Care
 - MDF has shipped over 1,000 printed versions of the Toolkit in the last two years; downloaded more than 2,500 times
 - Anesthesia Guidelines have been downloaded more than 4,500 times so far this year





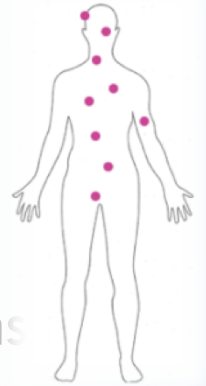
10 Years of Care



DIGITAL ACADEMY



HOW DM AFFECTS YOUR BODY



INTERACTIVE BODY SYSTEM TOOL ▶

Launched a comprehensive, growing array of support and education programs

- Community-focused website and Facebook pages:

- FB membership increased 923 % since early 2013 to almost 3,400 followers, and 3,500 visits per day
- Website users increased 45% since 2013 and now received over 14,500 visitors a month
- Digital academy, body systems tool, trial and study center, annual conference, support groups, and more

- More than 100 newsletters and more than 200 family stories and research updates since 2012 to keep you informed



10 Years of Care



Care and a Cure

- Launched a comprehensive and growing array of support and education programs
 - MDF has invested over \$675K in Care programs to improve the quality of life our community in the past two years alone
 - That is more than the entire MDF operational budget in the foundation's first 5 years
 - Our Care investment grows every year

MYOTONIC DYSTROPHY FOUNDATION

OUR IMPACT WHY REGISTER

HELP FIND A CURE

Join the Myotonic Dystrophy Registry today and help us better understand and improve the lives of the people and families living with Myotonic Dystrophy

JOIN NOW

\$675,000



10 Years of Cure

- ❑ Established and expanded Scientific Advisory Committee of researchers and clinicians from academia and industry
- ❑ Deeply committed to finding treatments and improving care





10 Years of Cure

- ❑ Launched Fellowship grants program:
 - ❑ Increase the number of scientists studying DM
 - ❑ Leverage the fellowship funds to increase overall DM research funding at the federal and international levels
 - ❑ 22 Fellows funded to date, totaling over \$2,200,000 and more planned for later this year
 - ❑ 71% have stayed in the DM research field, over 60% have attracted millions in new funding
 - ❑ Over 10 current and former fellows are here today



10 Years of Cure

- Launched Myotonic Dystrophy Family Registry
 - ▣ Focused on trial readiness and better disease understanding
 - ▣ First patient-entered, web-based DM registry
 - ▣ Over 1,500 patients and 60 researchers have registered
 - ▣ Has supported both clinical trials and research studies
 - ▣ First registry publication and annual report to you coming this year





10 Years of Cure

- Launched MDF 3.0, 3 year, \$5M drug development acceleration initiative
 - Eliminating barriers to drug development
 - More than 30 initiatives
 - 35 grantees from Eastern Europe and Milan to California
 - Initiatives focus on full span of drug development pipeline from basic science to drug review and approval, and patient reimbursement and access
- Over 35 research grants funded since 2007
- More than 60% of those in the last two years



10 Years of Patient Advocacy

- Launched first DM advocacy platform and engaged WA, DC policy consultants to:
 - ▣ Increase NIH funding for DM research
 - ▣ Gain access to new federal research funding streams
 - ▣ Improve patient and family access to Social Security Administration disability benefits





10 Years of Advocacy

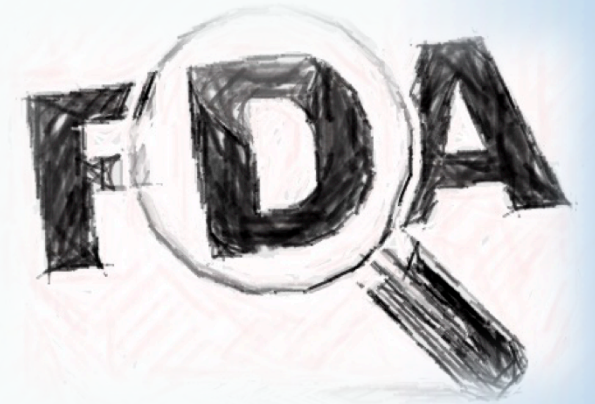
- Launched annual MDF Hill Day to bring MDF families to Congress
 - 3rd Hill Day in 2016 – more than 200 family members have met participated
 - Met with more than 88 Congressional offices representing 42 states
 - 2nd Congressional Briefing on DM
 - Have educated over 100 staffers on what myotonic dystrophy is, what we need from Congress





10 Years of Patient Advocacy

- Multi-year effort to educate the Food and Drug Administration (FDA), and other international regulators about DM
 - 2014 science workshop at MDF Annual Conference:
 - Over 50 research professionals in academia and industry
 - Participation from head of Neurology review division at FDA
 - 2015 All day workshop in 2015
 - Focused on clinical trial design, biomarkers and endpoints
 - Designed to get FDA input on moving potential therapies forward
 - 2016 Patient-Focused Drug Development (PFDD) meeting on Thursday
 - Bring patient voice and experience to FDA therapy review
 - Describe the burden of living with DM, and what we want from therapies





You Have Made This Possible



Over \$8,500,000 funds raised for
Care and a Cure since 2006

77% since 2013



A Great MDF Team:

- ▣ Abe Aldrete, Operations Director
- ▣ Paul Formaker, Program Director
- ▣ Liz Haas, Communications Director
- ▣ Elizabeth Habeeb-Louks, Research Grants Manager
- ▣ Pam Lewis, Project Development Manager
- ▣ John Porter, PhD, Chief Science Officer
- ▣ Jessica Powers, Development Director
- ▣ Yasmene Taiym, Project Coordinator
- ▣ Katrina Yamazaki, PhD, Registrar
- ▣ John Brekka
- ▣ John Fitzpatrick
- ▣ Elizabeth Florence
- ▣ David Herbert
- ▣ Senator Tim Kaine
- ▣ Jeremy Kelly, Chair
- ▣ Woodie Kessel, MD, Vice-Chair
- ▣ Melvin Kohn, MD
- ▣ Todd Stone

