2016 MDF Conference
Report to the Community

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!
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

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
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
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
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
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
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
And All of You

- Patients and family members
- Clinicians
- Researchers
- Donors
- Industry professionals
- Federal agency reps
- Other advocacy organizations