

PPMD Advocacy Day

Congressional Meeting Talking Points

Wednesday, March 10, 2021

Intros

- Introduce your team to the Members/staffers.
- Take a few moments to tell your story about your experience with Duchenne and why the asks are so important to you.
- Be sure to thank the Member/staffer for their time.

Duchenne and PPMD Overview

- Duchenne muscular dystrophy is a fatal genetic disorder characterized by the progressive loss of muscle. It is a multi-systemic condition, affecting many parts of the body, which results in deterioration of the skeletal, cardiac (heart), and pulmonary (lung) muscles.
- Duchenne affects approximately 1 out of every 5,000 live male births. About 20,000 children are diagnosed with Duchenne globally each year.
- Parent Project Muscular Dystrophy fights to end Duchenne. We accelerate research, raise our voices to impact policy, demand optimal care for every single family, and strive to ensure access to approved therapies.

Summary of Asks:

Ask #1 Sign the FY22 Appropriations Request for Duchenne

Ask #2 Cosponsor the BENEFIT Act

Ask #1

Sign the FY22 Appropriations Request for Duchenne

- Thanks in large part to the leadership of Congress starting with the passage of the MD CARE Act in 2001 and continuing through two updates of the law, significant progress has been made over the past 20 years in the fight to end Duchenne Muscular Dystrophy.
- The annual appropriations process is key to advancing research for therapies and better care. Federal investment in Duchenne has resulted in much progress, including 35+ trials and 5 approved therapies, but these therapies only slow progression and we must continue to drive research and care programs at the federal level.
- This year, we are asking for:
 - **Department of Defense (DOD)** - A modest increase from **\$10 million to \$12 million** for Duchenne research
 - **Centers for Disease Control (CDC)** - A modest increase from **\$6 million to \$8 million** for muscular dystrophy programs
 - **CDC should focus effort understanding:**
 - **The impact of the Duchenne Care Considerations**, including in rural and underserved areas.
 - Availability of consistent and coordinated **care for adults** with Duchenne.
 - Impact of Duchenne on **mental health** on patients and caregivers.
 - **CDC should also issue a report on program priorities** including progress to date and priorities for upcoming years.
 - **National Institutes of Health (NIH)** should focus research on:
 - **Challenges related to gene therapies**, including delivery to individuals with neutralizing antibodies and manufacturing.
 - The impact of Duchenne on both the **brain and the heart**.
 - **NIH should encourage data sharing** from all NIH funded studies.
 - **FDA** should focus on:
 - Updating the *2018 FDA Guidance on Duchenne* to reflect advancement in **gene therapies**.
 - Convene a multi-stakeholder meeting to evaluate the use of external controls in order to **minimize the use of placebo in clinical trials**.

To sign on to support FY22 Duchenne appropriations, please contact:

- Senate: Sen. ROGER WICKER (R-MS) or Sen. DEBBIE STABENOW (D-MI)
- House: Rep. DORIS MATSUI (D-CA) or Rep. STEVE STIVERS (R-OH)

Ask #2

Cosponsor the **BENEFIT Act**

- The FDA has also made progress in recent years on incorporating the patient voice into the drug development process, in large part due to Congress' leadership on Prescription Drug User Fee Authorization (PDUFA) reauthorizations and the 21st Century Cures Act.
 - Since the 2012 PDUFA passed, FDA has worked on how to capture patient experience and preference data. The agency has developed guidances to inform how communities, like the Duchenne community, can develop tools to collect data that presents the patient perspective.
 - Since 21st Century Cures passed in 2016, FDA has included information about whether or not they have received that data.
- However, while FDA now collects this data, **there is no requirement** in the FDA law that patient engagement data be used in the drug development process. **The BENEFIT Act would update the FDA law to make sure patient engagement data is included as a part of the benefit-risk framework of a new drug approval.**
- The Duchenne community knows how important information from patients and caregivers is to clinical research and that this information must inform FDA decisions. For example, a clinical study might show that a drug had relatively minor impact on the condition or symptom that it was attempting to address and it may be determined that the risks of the drug outweigh its benefits. However, patients might prioritize risks and benefits differently than a clinical researcher.
- Now that we and FDA have more experience with patient experience data, **now is the time to take the next step and put that information to work!**

• **To sign on to support the BENEFIT Act, please contact:**

• Senate: S. 373 Sen. ROGER WICKER (R-MS) or Sen. AMY KLOBUCHAR (D-MN)

• House: The bill has not yet been introduced in the House, but we are expecting Rep. DORIS MATSUI (D-CA) to introduce it with a Republican co-lead soon and will follow up.

- Thank you again. Please don't hesitate to contact PPMD with any questions.