

PPMD

2021 Request “Ask” to Congress

**Parent
Project
Muscular
Dystrophy**

**JOIN THE FIGHT.
END DUCHENNE.**

Summary: Make the Ask!

Ask #1

Sign the FY22 Duchenne Appropriations letter

Congressional Champions leading the letters

House – Letter is led by Rep Doris Matsui and Rep Steve Stivers

Senate – Letter is led by Senator Wicker and Senator Stabenow

Ask #2

Cosponsor the BENEFIT Act

Senate – Please cosponsor BENEFIT **S. 373** in Senate led by Senators Wicker and Klobuchar

House – Mention that we hope to have a House bill soon and request you cosponsor once introduced. **Rep. Doris Matsui** will be the Democratic House lead. We are waiting on our Republican lead to confirm.

Ask #1

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**Parent Project
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What are we asking Congress to do?

Ask #1

Sign the FY22 Duchenne Appropriations Letter

**Please sign the FY 2022 (FY22)
Duchenne Muscular Dystrophy
Appropriations Letter**

(Duchenne “Dear Colleague” letter)

**The letter includes funding amounts and
Duchenne specific report language**

The letter is led by:



Rep Doris Matsui (D-CA)



Rep Steve Stivers (R-OH)



Senator Debbie Stabenow
(D-OH)



Senator Roger Wicker
(R-MS)



Ask #1

Sign the FY22 Duchenne Appropriations Letter



Funding Request: Increase funding from **\$6 million to \$8 million**

CDC report language summary:

- **Focus on evaluating:**
 - **Impact of 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.
- **Issue a report on program priorities** including progress to date and priorities for upcoming years.

Ask #1

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National Institutes
of Health

NIH report language summary:

- **Data sharing:** Establish a framework for data-sharing generated or collected within six months of completion of any NIH-funded clinical study.
- **Gene therapy:** Focus research on challenges related to gene therapies, including delivery to individuals with neutralizing antibodies, and manufacturing supply to ensure all patients can receive treatment.
- **Duchenne and the brain:** Increase research related neurobehavioral/cognitive diagnosis and characterize the role of dystrophin in the brain.
- **Duchenne and the heart:** Increase research that characterizes cardiomyopathy in Duchenne and Becker. Convene a workshop on establishing viable cardiac outcome measures for the development of therapeutic agents to combat cardiomyopathy.

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Report language summary:

- **Gene Therapy:** Consider updating the *2018 FDA Guidance on Duchenne* to reflect advancement in gene therapies.
- **Clinical Trial Improvement:** Convene a multi-stakeholder meeting to evaluate the use of external controls in order to minimize the use of placebo in clinical trials.

Ask #1

Sign the FY22 Duchenne Appropriations Letter



Funding Request: Increase funding from \$10 million to \$12 million for the Duchenne Program (DMDRP) within the Department of Defense's Congressionally Directed Medical Research Programs (CDMRP).

Summary: Ask #1

Ask #1

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Ask #2

Cosponsor the BENEFIT Act

**Parent Project
Muscular
Dystrophy** **JOIN THE FIGHT.
END DUCHENNE.**

What are we asking Congress to do?

Ask #2

Cosponsor the BENEFIT Act

Support the BENEFIT ACT by
cosponsoring the legislation

Ask #2

Cosponsor the BENEFIT Act

The BENEFIT Act would **require that the FDA disclose** whether and how patient experience data – *Patient Reported Outcome Data, Patient Testimonies, Patient Preference Data* – was used in the assessment of a new therapy.

Figure 1: FDA Benefit-Risk Framework

Decision Factor	Evidence and Uncertainties	Conclusions and Reasons
Analysis of Condition		
Current Treatment Options		
Benefit		
Risk		
Risk Management		
Benefit-Risk Summary Assessment		

This will ensure that patient voices are being heard and allow for patient stakeholders to continue to work with FDA to refine the data that they need to make decisions.

Ask #2 What we are trying to accomplish

Current matrix

Proposed change

Figure 1: FDA Benefit-Risk Framework

Decision Factor	Evidence and Uncertainties	Conclusions and Reasons
Analysis of Condition		
Current Treatment Options		
Benefit		
Risk		
Risk Management		
Benefit-Risk Summary Assessment		

This is the matrix FDA has to fill out once a drug is approved

Figure 1: FDA Benefit-Risk Framework

Decision Factor	Evidence and Uncertainties	Conclusions and Reasons
Analysis of Condition		
Current Treatment Options		
Benefit		
Risk		
Risk Management		
Patient Experience Data		
Benefit Risk Summary Assessment		

New section

Summary: Ask #2

Ask #2

Cosponsor the BENEFIT Act

ASK in the Senate: Senators Wicker and Klobuchar have introduced the the BENEFIT as S.373. Senators can support BENEFIT by cosponsoring S.373

ASK in the House: We hope to have a House bill soon and request you cosponsor once introduced. Rep. Doris Matsui will be the Democratic House lead. We are waiting on our Republican lead to confirm



Senator
Amy Klobuchar
(D-MN)

Senator
Roger Wicker
(R-MS)



Rep Doris Matsui
(D-CA)



Not
confirmed

R lead

Final tips for meetings...

Parent **JOIN THE FIGHT.**
Project **END DUCHENNE.**
Muscular
Dystrophy

How to Sabotage Your Meeting in Six Simple Steps:

1. Arriving late or not at all to your virtual meeting without contacting office (or group)
2. Being political
3. Not communicating your ask
4. Being cranky
5. Forgetting to say thank you
6. Not following up



Successful Meeting Strategies:



- **Coordinate** with your team before the meeting – divide and conquer
- **MOST IMPORTANT: TELL YOUR PERSONAL STORY**
- **Practice** your ask
- **Arrive early to the online meeting**
- **DO NOT BE POLITICAL. We are all one party on the 10th**
- **If you don't know the answer to a question** – don't worry! We can follow up.
- **Thank** the Member if they have supported Duchenne in the past. (see schedules)
- **Send** thank you email after meeting
- **Follow up/stay active**

Congressional Scorecard Coding

Your Soapbox Schedule has coding - Know who you are meeting with:

- If your member is on the **Appropriations Committee** you will see
 - [S.Appr-Full] - Appropriations
 - [H.Appr-Full] - Appropriations
 - **Appropriators** normally don't sign letter (it would be a letter to themselves) but you should thank them for the role they play and inform them of the request.
- If they have **signed a past funding letter** for Duchenne you will see [LTR]
- If they **cosponsored the MD-CARE** Act you will see [COSPON]
- If they are a **brand new member** it will say (New)

IGNORE THESE – they are for in person and indicate the building the members are in RHOB-### or LHOB-### , CHOB-### , or SR-### , SH-### , SD-###

Additional Coding

[H.Ldr] - H. Leadership

[Ldr] - S. Leadership

X (New) to 116th

X (Up) in Senate

X (Ret.) Retiring

X (Run Other) for other office

Check the score card and access additional resources

join.parentprojectmd.org/HUB

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Questions?

- Ryan Fischer Ryan@parentprojectmd.org
- Cell phone: 917-251-6684
- **ALL QUESTIONS ABOUT SCHEDULES OR MEETING TIMES:**
 - Contact Soapbox Kevin Schultze schultze@soapboxconsulting.com
- Advocate Resources: Visit join.parentprojectmd.org/HUB