Objective: Illustrate a pioneering collaboration between patient advocates, academics, and policy experts to advance drug development for this serious and ultimately fatal genetic disorder.
speakers

John F P Bridges
PhD
Associate Professor, Johns Hopkins Bloomberg School of Public Health

Timothy Franson
MD
Chief Medical Officer, YourEncore

Holly Peay
MS, CGC
Vice President of Outreach & Education, Parent Project Muscular Dystrophy

K. Kimberly McCleary
Director of Strategic Initiatives, FasterCures
MODERATOR
Strength Happens Together: PPMD Submits FDA Draft Guidance on Duchenne

PPMD and a broad coalition of stakeholders has submitted the first-ever patient advocacy-initiated draft guidance for a rare disease to the FDA to help accelerate development and review of potential therapies for Duchenne.

Read more.
Putting Patients First

Recommendations to speed responsible access to new therapies for Duchenne muscular dystrophy and other rare, serious and life-threatening neurologic disorders

Benefit-Risk Assessments in Rare Disorders

THE CASE FOR THERAPEUTIC DEVELOPMENT IN DUCHENNE MUSCULAR DYSTROPHY AS THE PROTOTYPE FOR NEW APPROACHES
## Example BWS choice task

<table>
<thead>
<tr>
<th>Best</th>
<th>Treatment</th>
<th>Worst</th>
</tr>
</thead>
<tbody>
<tr>
<td>☐</td>
<td>Slows the progression of weakness</td>
<td>☐</td>
</tr>
<tr>
<td>☐</td>
<td>2 year gain in expected lifespan</td>
<td>☐</td>
</tr>
<tr>
<td>☐</td>
<td>1 year of post-approval drug information available</td>
<td>☐</td>
</tr>
<tr>
<td>☐</td>
<td>Causes loss of appetite</td>
<td>☐</td>
</tr>
<tr>
<td>☐</td>
<td>Increased risk of bleeding gums and increased bruising</td>
<td>☐</td>
</tr>
<tr>
<td>☐</td>
<td>Increased risk of harmless heart arrhythmia</td>
<td>☐</td>
</tr>
</tbody>
</table>

Choose the best thing in this treatment by clicking the circle under “best” and choose the worst thing by clicking the circle under “worst”. You have to choose a best thing and worst thing to move on. Remember that a computer chose the combinations to make the experiment work, and some may seem bad. Even so, please pick the best and worst thing.
Conditional attribute importance

- Arrhythmia
- Weakness
- Bleeding
- Lifespan
- Nausea
- Drug info
LEADING THE FIGHT TO END DUCHENNE

June 25, 2014

Guidance Document Submission
Division of Dockets Management [HFA-305]
5630 Fishers Lane, Rm. 1061
Rockville, MD 20852

Dr. Janet Woodcock,
Center for Drug Evaluation and Research
Food and Drug Administration
10903 New Hampshire Ave
Silver Spring, MD 20993-0002

Dear Dr. Janet Woodcock and colleagues at the FDA,

This correspondence constitutes a formal submission of a draft guidance authored by a consortium of stakeholders, under the coordination of Parent Project Muscular Dystrophy (PPMD), for consideration by the Food and Drug Administration (FDA). This material is intended as a submission to the docket as provided under the advice from the FDA’s good guidance practice work group, with the expectation that FDA will seriously consider adoption of all or significant sections of this submission.

When FDA, PPMD and other interested parties met on December 13, 2013 in the spirit of public-private partnership to convene a Duchenne policy forum, we discussed the challenges designing and implementing clinical trials for rare diseases like Duchenne muscular dystrophy and the need to develop guidance to help accelerate development and the review of potential therapies for Duchenne muscular dystrophy (Duchenne). The forum concluded with an agreement that the Duchenne community, led by PPMD, would develop the first draft guidance on Duchenne for industry.

After an intensive five month long process, overseen by a steering committee, developed by a working group composed of clinical experts, developers and patients, and further reviewed by a community advisory board, we are pleased to hereby present to you the Duchenne muscular dystrophy community’s draft of the Guidance for Industry: Duchenne Muscular Dystrophy: Developing Drugs for Treatment over the Spectrum of Disease, the first-ever patient advocacy initiated draft guidance for a rare disease, written to help accelerate the development and review of potential therapies for Duchenne muscular dystrophy (Duchenne).

Our submission is prefaced by the Duchenne Imperatives, which begins with a few case studies, summarizes the document’s key points and explains the Duchenne community’s key imperatives — what we hope will be the take home messages from the community for the sponsors, the academic community and for the FDA, and to serve to frame the importance of the development of guidance for the community. We understand that the FDA may choose not to formally adopt this preface, though it is hoped that such information will inform FDA’s deliberations regarding adoption of the formal draft guidance, which follows.
FDA Wants Input on Patient-Developed DMD Guidance

Background

In June 2014, Parent Project Muscular Dystrophy (PPMD) and other stakeholders submitted a draft guidance document to the FDA which they said would “help accelerate development and review of potential therapies for DMD.”

The guidance, the first developed by a patient advocacy group, has been seen by some as a harbinger of other patient-developed guidance documents. FDA has recently been encouraging more patient involvement in the regulatory process through its patient-centered drug development program, which was founded in 2012 after the passage of the Food and Drug Administration Safety and Innovation Act (FDASIA). The thinking of the program, as reflected in the PPMD guidance, is that patients living with a disease are better arbiters of meaningful outcomes than regulators are.

“"The Pink Sheet”

Group Submits Duchenne Muscular Dystrophy Guidance, Hoping To Direct FDA Policy

By Derrick Gingery / Email the Author / View Full Issue

Regulatory Update / Word Count: 1188 / Article # 00140707011 / Posted: July 7 2014 12:02 AM

Executive Summary

Parent Project Muscular Dystrophy’s proposed guidance gives detail on biomarkers and clinical trial designs for the beleaguered field, but most importantly addresses benefit-risk assessment — which is FDA's underlying ambition for the patient-focused drug development initiative.
Questions?