

A **YES** ON ETEPLIRSEN.

IT'S NOT EMOTION. **IT'S SCIENCE.**

MEDIA OUTREACH TOOLKIT

We need to make it very clear: the science supports approval of eteplirsen. This isn't an appeal to emotion. This is an appeal to reason. To that end, here are two key actions you can take over the next week to help make a difference:

1. Contact your local media outlet(s) and explain who you are and why this issue is important to you. Then explain that the science supports approval. Key points are provided below for your use.
2. Send a letter to the editor underscoring how the data supports approval of eteplirsen.

If you have any questions or need additional assistance, please do not hesitate to contact any one of us, or email us at makeduchennehistory@gmail.com.

Key Points:

- An FDA Advisory Committee met to consider eteplirsen on April 25. The FDA will make the final decision on whether to grant FDA approval for eteplirsen, making it the first FDA-approved treatment for Duchenne Muscular Dystrophy.
- Thirteen of the world's leading Duchenne scientists and clinicians participated in the Open Public Hearing at the AdComm meeting.
- Other than their hotel and airfare, these experts were not compensated for their time.
- Each of them stated that based on their extensive experience specifically with Duchenne, eteplirsen is effective and deserves approval.
- 36 scientists and clinicians, including many who spoke at the AdComm OPH, submitted a letter to the FDA, saying, in part:
 - “[W]e conclude that the aggregate data, described in the briefing documents, are providing substantial evidence of efficacy and use in the greater population of boys amenable to exon 51 skipping is appropriate.”

The letter can be found here:

http://www.cdmd.ucla.edu/files/view/FDA_ETEPLIRSEN_LETTER_02242016.pdf

- This is not about emotions. This is about science. And the science supports approval of eteplirsen.

Template for Media Outreach:

Dear Editor:

My name is XXX and I am from XXX. On April 25, 2016 the Food and Drug Administration (FDA) convened an Advisory Committee to consider promising treatment for Duchenne muscular dystrophy called eteplirsen. I/my family/friends/XXX attended the meeting. My XXX-year-old son XXX, was diagnosed with Duchenne in XXX. Duchenne is a rare and always fatal disease that impacts one in every 3,500 live male births in the U.S., and currently has no FDA approved treatment or cure.

More than a dozen world-renowned Duchenne clinicians and scientists donated their time and spoke during the Open Public Hearing, saying that based on their extensive experience and in their expert opinion, eteplirsen deserves FDA approval. The FDA now has to make a decision on whether to grant approval.

I think my family's participation in this historic meeting on April 25 would be of great interest to your readers. I would like very much to speak with you about our story, my son, and our efforts to win approval for this life-saving new therapy.

If you or a reporter would like to discuss our story, please contact me at: Email/Phone
Thank you very much.

Sincerely,

Xxxxxx

Tips on drafting a Letter to the Editor:

- Keep the letter short (under 200 words) and get right to the point.
- Sign your letter and include your name, affiliation and contact information, including address, city, town, phone number, and email address (your address and contact info will not be published).
- Proofread and have someone else read your letter to help ensure you are communicating clearly.
- Once your letter is submitted, follow up with the newspaper to ensure that the editor has received it and has all the information needed to publish it.
- If you need assistance drafting your letter, contact us at makeduchennehistory@gmail.com.

Clinicians and Scientists Who Spoke at the AdComm OPH on April 26:

Barry Byrne – Professor and Associate Chair of Pediatrics and Molecular Genetics and Microbiology at the University of Florida; Director of the UF Powell Gene Center for Rare Disease. Treats Duchenne patients, including one of the boys who has been on eteplirsen since 2011.

Jeff Chamberlain – Professor in the Departments of Neurology, Medicine, and Biochemistry; McCaw Endowed Chair in Muscular Dystrophy at the University of Washington; and Director of the Senator Paul D. Wellstone Muscular Dystrophy Cooperative Research Center of Seattle. Has conducted research focused on Duchenne muscular dystrophy field for 25+ years.

Ann Connolly – Professor of Pediatrics and Neurology at Washington University School of Medicine. Neuromuscular physician and researcher caring for children with Duchenne muscular dystrophy for over 25 years; has cared for more than 150 boys and men with DMD. Serves as an investigator of the confirmatory trial of Eteplirsen; two of her patients have been receiving Eteplirsen as part of the phase IIb trial for over three years.

John Day – Professor of Neurology and Pediatrics at Stanford University Medical Center, Director of Neuromuscular Division and Clinics. Has cared for Duchenne patients for the past 25 years. Served as Principal Investigator of the Duchenne Muscular Dystrophy Clinical Trials Network at the University of Minnesota; founded and was Director of the Paul and Sheila Wellstone Muscular Dystrophy Center.

Sue Fletcher – Principal research fellow in the Molecular Genetics Laboratory at the Centre for Comparative Genomics, Murdoch University, Australia. Has worked in the Duchenne muscular dystrophy field since 1991. Along with Professor Steve Wilton, became the first group to report specific dystrophin exon skipping in the mdx mouse in 1999.

Peter Heydemann – Head of Pediatric Neurology at Rush University Medical Center in Chicago. Has managed a muscular dystrophy clinic since the early 1980s. Manages the administration of eteplirsen to one of the boys in the phase IIb trial.

Lou Kunkel – Professor of Pediatrics and Genetics at Harvard Medical School, Member of the Division of Genetics and Genomics at Children's Hospital Boston, a member of the National Academy of Sciences, and a recipient of the Allen Award by the

American Society of Human Genetics, with over 30 years of experience and scientific success in the understanding of the basis for muscular dystrophies.

Elizabeth McNally – Head of the Center for Genetic Medicine (CGM) at Northwestern University's Feinberg School of Medicine in Chicago. She is the Ward professor of Medicine (Cardiology) and of Biochemistry and Molecular Genetics. Works closely with neurologists to optimize cardiac care for Duchenne Muscular Dystrophy and other neuromuscular diseases.

Carrie Miceli – Professor of Microbiology Immunology and Molecular Genetics at UCLA and co-Director of the Center for Duchenne Muscular Dystrophy at UCLA School of Medicine and the College of Letters and Sciences.

Stan Nelson – Professor of Human Genetics, Pathology and Laboratory Medicine at UCLA and co-director of the Center for Duchenne Muscular Dystrophy. Cares for Duchenne patients at the Pediatric Neuromuscular Clinic and Genetics Clinic at UCLA.

Terry Partridge – Professor of Integrative Systems Biology and of Pediatrics at George Washington University. Previously served as head of the Experimental Pathology and Muscle Cell Biology Group at the Medical Research Council Clinical Sciences Centre at Imperial College London. Has worked on muscular dystrophy research since 1970, and produced the first data establishing muscle stem cell transfer and exon skipping in the dystrophin gene as potential therapies for DMD.

Perry Shieh – Associate Professor of Neurology at the David Geffen School of Medicine at UCLA and Director of the Neuromuscular Program within the Department of Neurology. Has led the UCLA Pediatric Neuromuscular Clinic for over 10 years; site Principal Investigator of several multi-site clinical trials for Duchenne including active protocols for Eteplirsen.

Kathryn Wagner – Professor of Neurology and Neuroscience at Johns Hopkins Medical School and Director of the Center for Genetic Muscle Diseases at Kennedy Krieger Institute. Has led many clinical trials for Duchenne, and been actively involved in translational research for over 20 years.