Parent Project Muscular Dystrophy

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November 13, 2017

U.S. Food and Drug Administration
10903 New Hampshire Avenue
Silver Spring, MD 20993
Attn: Dr. Janet Woodcock

Dear Dr. Woodcock,

As you are aware, on September 28, 2017, the FDA Advisory Committee for Peripheral and Central Nervous System Drugs convened to review the new drug application for ataluren. We thank the FDA for convening the Advisory Committee, and for providing our patient and clinical community the opportunity to share our direct experiences with ataluren through written and oral testimony during the open public hearing.

On October 25, 2017, PTC Therapeutics shared the news that the FDA had issued a Complete Response Letter (CRL) regarding their application for ataluren, noting that FDA had stated that "an additional adequate and well-controlled clinical trial(s) will be necessary at a minimum to provide substantial evidence of effectiveness." Following that announcement PTC also announced that they planned to file a formal dispute resolution.

We understand that the formal dispute resolution is a regulatory process that concerns confidential information being exchanged between the agency and the sponsor and that there is no formal mechanism for patient engagement within the process of a dispute resolution. We acknowledge that process is underway and respectfully ask that you accept and consider this letter from Duchenne caregivers and patients who wish to communicate their collective support of the approval of ataluren based on direct experience with ataluren.

Parent Project Muscular Dystrophy (PPMD) supports all therapy development in an independent and objective manner as our mission dictates. Our work has included an emphasis in recent years on patient-focused drug development strategies as the Duchenne therapy pipeline has become more robust. One of our efforts included a rigorous study on patient and caregiver preference where our team, supported by Dr. John Bridges of Johns Hopkins, found that caregivers are willing to accept a serious risk when balanced with non-curative slowing or stopping of the progression of muscle weakness, even absent improvement in lifespan.[i] In a subsequent study, focused on non-skeletal muscle targets,

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caregiver and patient participants rated maintaining effective cough strength and reduced lung infections as important treatment priorities for Duchenne, placing high value on pulmonary outcomes that are meaningful to their daily lives[ii]. These preference findings are important both for regulatory review and for determining the value of a treatment for a health plan beneficiary.

Real world evidence – how an individual feels, functions, and survives – is of great concern to the FDA and to families living with Duchenne muscular dystrophy. Prior to the FDA Advisory Committee meeting for ataluren, PPMD initiated a patient experience survey in an effort to fully understand the experiences of individuals and their caregivers who had direct experience with ataluren. Sixty-one families participated in the survey, anxious to convey real world evidence and first-hand observations related to benefit and risk. All respondents believed ataluren to be safe and most suggested they saw benefit in terms of slowing progression. The report is enclosed with this letter and was submitted to the docket prior to the FDA Advisory Committee meeting.[iii]

The undersigned families ask that you consider the preferences and experiences of the Duchenne community members with direct experiences on ataluren when making a decision regarding the path forward for ataluren. All agree that, based on experience and public information about the data, ataluren provides meaningful benefit, is well tolerated, and presents an acceptable benefit-risk tradeoff for use in patients amendable to this therapy.

Sincerely,

Joseph Omari & Elfreda Agboka
Joseph Ofori Agboka, Jr***

Ethan & Michelle Barshay
Connor Barshay***

Gene & Liz Bowden
Teaghan Bowden ***

Chris & Kristie Browne
Logan Browne*

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Kari Byrnes
Parker Byrnes***

Jill Castle
Anthony Castle*

Mike & Denise Chorzewski
Joey Chorzewski*

Ginger Edwards
Liam Edwards*
Jaxx Edwards*

Brian & Gretchen Egner
Nick Egner*

Charlie & Julie Farwell
Ryan Farwell*

Cheri & John Gunvalson
Jacob Gunvalson*

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Matthew Harrison*

Shawn & Deb Jenssen
Sarah Jenssen***
Megan Jenssen***

Paul & Joanna Johnson
Elliott Johnson*
Henry Johnson*

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Jack & Terry Kirley
Maxx Kirley*

Darrell & Angela Knight
Jack Knight*

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Maria McDonnell
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Tim & Carolyn Monson
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Darlene & Ron Mueller
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Ramiro Urrea
Ramiro Urrea, Jr*

Jason and Betty Vertin
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Josh Wagner*
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Timothy Wagner*

Theresa and David Wood
Matthew Wood*

Kevin & Melissa Yurik
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* currently on ataluren
*** waiting for access

[i] Peay, H. L., Hollin, I., Fischer, R., & Bridges, J. F. P. (2014). A community-engaged approach to quantifying caregiver preferences for the benefits and risks of emerging therapies for duchenne muscular dystrophy. Clinical Therapeutics, 36(5), 624–637.

[ii] Ilene L. Hollin, PhD MPH1, Holly Peay, PhD2,3, Susan D. Apkon, MD4, John Bridges, PhD1 Patient-centered benefit-risk assessment in Duchenne Muscular Dystrophy

[iii] www.parentprojectmd.org/translarnaexperiencereport