Division of Dockets Management (HFA-305)  
Food and Drug Administration  
5630 Fishers Lane, Rm. 1061  
Rockville, MD 20852

Re: Federal Register Number 2014-21051

Dear FDA Staff,

The EveryLife Foundation for Rare Diseases applauds Parent Project Muscular Dystrophy (PPMD) for their leadership as a rare disease patient advocacy organization in collaborating with the FDA and proposing the current guidance, *Guidance for Industry Duchenne Muscular Dystrophy Developing Drugs for Treatment Over the Spectrum of Disease*, Federal Register Number 2014-21051. The EveryLife Foundation fully endorses and supports the proposed guidance created by PPMD, and that the final guidance serves its purpose to more effectively guide industry sponsors as potential new drugs are considered for development to treat DMD.

Duchenne Muscular Dystrophy (DMD) is a rare genetic disease associated with a progressive degeneration that is significantly limiting in the physical quality of life and also shortened life span. There is an urgent need to spur the development of innovative drugs to benefit those suffering with the disease that currently lacks causative treatment. The proposed draft guidance by PPMD is a symbol of the community’s harnessed energy to open gateways and develop pathways for future potential treatments, including the use of potentially novel clinical or surrogate endpoints and/or biomarkers.

All sections of the proposed guidance address critical areas of clinical and regulatory need as applied to DMD. The detailed descriptions in the guidance begin to address some of the major challenges that are known inhibitors in furthering the development of drugs for rare genetic diseases. In general, some of the accomplishments of the proposed guidance include addressing the following issues:

1) How clinical outcomes measures and endpoints inform needed and new regulatory standards;
2) Why advances in basic research and clinical medicine require new regulatory standards and that these standards cover the entire continuum of disease;
3) Why carefully measured and analyzed benefit/risk ratios as expressed by patients and parents should shape new regulatory standards; and
4) The importance of including patients’ and parents’ needs and preferences in new regulatory standards (which the draft document does).
Furthermore, the EveryLife Foundation commends PPMD for addressing multiple specific clinical and regulatory challenges in the guidance document. With an entire section dedicated to the use of biomarkers, and another focusing on clinical trial design, this is the most detailed guidance information to date on these topics for the DMD and rare genetic disease community. The Accelerated Approval regulatory pathway explained on page 32 of the guidance includes information about the potential for novel clinical endpoints, development of surrogate clinical endpoints and/or use of biomarkers in creating novel drugs to treat these diseases.

The EveryLife Foundation is very enthusiastic about the downstream effects of this thoughtful guidance for those affected by DMD, and looks forward with great anticipation to a bright future full of positive gains for those suffering with DMD. Topping that list is developing drugs to improve the lives of those affected by DMD.

Sincerely,

Dr. Emil Kakkis
Founder and Board President, EveryLife Foundation for Rare Diseases