

Annie Kennedy ~ Oral Public Comment
PDUFA VII Workshop
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Good afternoon, I am Annie Kennedy with the EveryLife Foundation for Rare Diseases. But I am actually here today representing much more than just our nation's rare disease community.

More than a year ago, a group of colleagues and I came together to reflect on all that had yielded from the PDUFA and 21st Century Cures initiatives – and to together think about what was needed going forward. Within moments of our first gathering, we all agreed that our communities have benefited significantly from this “patient focused drug development” movement -- and thus we grew to refer to our efforts as ‘The PFDDworks’ Collaborative.

Our PFDDworks membership includes the National Psoriasis Foundation, the Amyloidosis Research Consortium, the LUNgevity Foundation, the Michael J. Fox Foundation for Parkinson's Research, the COPD Foundation, the Lupus Foundation of America, the ALS Association, the National Eczema Association, the Medical Research Fund, Parent Project Muscular Dystrophy, and the EveryLife Foundation for Rare Diseases – and has been convened with the leadership of Faegre Drinker Consulting and the Kith Collective.

Together, we represent a broad array of patient communities with a diverse expertise in patient engagement.

Collectively, we have led:

- 8 FDA-led Patient Focused Drug Development Workshops
- 8 Externally-led PFDD Workshops
- 2 Meetings that pre-dated the PFDD terminology but included significant FDA engagement
- 2 patient-community led draft guidances
- numerous patient preference studies that have been incorporated into clinical development and regulatory review
- and most notably -- have seen formerly stark disease spaces transformed into robust therapeutic pipelines.

In the spirit of transparency, many of our PFDD works members efforts are supported through collaborations with biopharmaceutical organizations.

It is through this lens of our PFDDworks Collaborative that I offer some comments here today...

First and foremost, we thank you.

Today, our patient community organizations are working within the ecosystem as trusted conveners of industry, agency partners, and academia to help advance PFDD in an open and pre-competitive environment.

To that end, as we lean into PDUFA VII, we have identified a number of priority areas for further expansion. They include the categorical areas of:

- Transparency,
- Authenticity,
- Consistency, and
- Comprehensiveness

For the sake of time, I will highlight key points from 2 of these priority areas:

Transparency –

As our organizations and others continue to make significant investments of time and community resources to develop robust patient and caregiver data, we support continued efforts to incorporate this data early in target identification and clinical trial design – and to seek additional opportunities for both sponsors and regulators to share how this information is utilized.

- We applaud the agency's implementation of the Patient Experience Data checklist and encourage further expansion of its application and use throughout sponsor, agency, and patient community engagements;
- Further, we recognize the immense resources that have been collected through the PFDD Workshops, Externally-led Meetings, and Voice of the Patient Reports -- and urge additional resources be made available to the agency to further support PFDD innovation. These could include:
 - Supporting expanded cooperative agreements that include the patient community to use PFDD data in critical areas such as the development of core sets of clinical outcome assessments that are shown to be meaningful to patients and caregivers.
 - Supporting activities that center on obtaining caregiver input in drug development and expanding the role of the caregiver in regulatory science.

And - Comprehensiveness

Perhaps most importantly, we recognize that the principles of patient-centered, data-driven product development must be applied across the entire continuum.

We encourage efforts to reduce the barriers between regulatory approval and market access – a gap which is emerging as a second “valley of death” in the lifecycle of therapeutic development.

- As core outcomes are developed, they should be created with a consideration of outcomes of importance to stakeholders all through the development pipeline – and must include patient community involvement to ensure that the outcomes collected are meaningful and of value to both patients and caregivers
- We also encourage advancement of opportunities to support label determinations that are made based in-whole or in-part on patient experience data – and the availability of such data to inform decision making beyond the regulatory environment

In Closing

Our PFDDworks partners - and the communities we represent - are grateful to the FDA for your leadership and continued commitment to placing patients at the heart of product development – and we look forward to continuing to collaborate and innovate alongside you.