EveryLife Foundation PDUFA VII Public Stakeholder Meeting

Annie Kennedy, EveryLife Foundation Chief of Policy & Advocacy,
Invited Stakeholder Reaction Remarks
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Thank you for inviting me present on behalf of the Foundation and the rare disease community. My name is Annie Kennedy and I am pleased to serve as Chief of Policy and Advocacy for the EveryLife Foundation for Rare Diseases.

We are a rare disease policy organization that understands that no disease is too rare to deserve treatment, and rare disease therapies should be safe and effective. To that end, we convene a robust coalition comprised of patient advocacy organizations, biopharmaceutical partners, coalition groups, and other relevant stakeholders with shared interests policy focused on rare therapy development and regulatory infrastructure. Today's remarks are a reflection of the engagements of this coalition around our shared PDFUA priorities.

I'd like to start today with a tremendous THANK YOU.

A THANK YOU to the leadership and personnel here at the FDA – and those within our therapeutic development ecosystem. While the term 'unprecedented' has probably been overused over the last 18 months, I'm unsure of what other term to use that would be appropriate for this forum to capture the climate of "forced evolution" in which we have worked and lived. We'd be remiss to not acknowledge the leadership and tireless work conducted by this Agency to protect our nation and our loved ones. Thank you.

We are also grateful – that while your capacity was being stretched to attend to the global pandemic – you also understood that the urgency of rare disease was not waning, but was in fact instead **further exacerbated** by the pandemic. An urgency that is being felt by the more than 30 million Americans living with rare diseases, a disproportionate percentage of whom are children. And as most here are well aware, 95% of the more than 7000 rare diseases have no FDA-approved therapy

Yet the technologies and methodologies deployed to pivot during the pandemic that resulted from COVID-19 underscored the opportunities for innovations that have been long-championed by rare disease community stakeholders – and further reflected core themes within the PDUFA agreements of scientific rigor, transparency and stakeholder engagement.

So, for pivoting and deploying new innovations to protect our public's health throughout our Covid-19 pandemic – we thank you. But we also thank you for not taking your foot off the accelerator -- and for understanding that rare disease is also a public health crisis deserving our continued urgent attention.

That continued commitment and understanding is clearly evident in the PDUFA VII enhancements we're discussing here today.

The Arc of Patient Engagement

I'd like to start with a bit of a broader focus and to take a moment to reflect on the past 10 years. We as communities and collaborative stakeholders have experienced a tremendous evolution of patient engagement and related areas since their infancy in PDUFA V -- to their continued development via 21st Century Cures and PDUFA VI and now their continued maturation today.

In this time, the landscape has shifted for the rare disease community:

- We've seen a deepened engagement and understanding of the patient perspective via numerous approaches such as the Patient Focused Drug Development meetings (FDA and externally led)
  - Of the more than 70 PFDD meetings convened to date, at least half have focused on rare diseases

- We've had opportunities for meaningful participation in formal service on advisory committees, the formation of patient engagement advisory committee (CDRH), reporting on use of patient engagement information in reviews of approved products.

- We've benefited from the Implementation of the Patient Experience Data Table in November of 2017 enabling a new level of transparency and engagement between the review division and relevant stakeholders around the integration of patient experience data within the regulatory review

- And the CDER-issued series of guidance documents for conducting patient-focused drug development have been critical tools in providing stakeholders with practices for
  - collecting comprehensive and representative input;
  - methods to identify what is important to patients;
  - selecting, developing, or modifying fit-for-purpose clinical outcome assessment; and,
  - incorporating clinical outcomes assessments into endpoints for regulatory decision-making.

In addition, FDA has published numerous other guidances that are informing patient engagement and patient-focused product development activities for drugs, cell- and gene-based therapies, diagnostics, and medical devices that have been critical to our pipelines.

- **But most importantly are the lives changed and saved through products approved to treat rare conditions, especially those that have benefited from the inclusion of more robust patient insights and patient experience data as part of the review process.**

It is important to note that these approvals have not come because FDA lowered the evidentiary bar for rare disease products.

Rather, they came because FDA and Congress have recognized the need for a nuanced approach to review of such products given the unique nature of rare diseases, including patient populations, trial sizes and other critical data points.

And by instituting a structured approach to listen to the voice of the patient in a meaningful way, the FDA has demonstrated its commitment to science and to ensuring appropriate processes are in place to quantify the perspective of the patient or the caregiver.

Specific to the PDUFA VII Agreement

The EveryLife Foundation is very pleased to see the FDA's commitment to advancing the field of patient engagement clearly evident in the PDUFA VII Commitment letter released last month.

- the **Rare Disease Endpoint Advancement (RARE) pilot;**
- the **Split Real Time Application Review (STAR) pilot;**
- advancing **patient-focused drug development within CBER; and**
- refining and advancing policies to support use of **Real-World Evidence (RWE) and innovative trial designs**

are all just examples of the commitments agreed to by FDA and industry that - over the performance period - will further support patient engagement and patients with rare conditions.

Given the promise and anticipation in innovative development in rare disease, we are particular gratified to see the expanded emphasis on enhancements critical innovation areas through resources, public workshops, pilots, and guidances to address emerging issues such as Real World Evidence, Bayesian Modeling, and enhanced engagement formats such as the proposals for INTERACT, MIDD, and the new Type D meeting models

We are very pleased to see the priorities we've articulated throughout this process reflected in these commitments.

Considerations for Further Refinement

We would also encourage that as we work towards further refinement of these commitments, we think about how we can incentivize and allow for collaboration between sponsors, the agency, and patient organizations on these critical concepts within the pre-competitive space; and not just when connected to a specific IND.

To the degree possible - we would like to see FDA commit even more to advance rare disease policy, including by going above and beyond the commitments set in the letter, such as by exceeding stated deadlines.

- As an example, during PDUFA V, the FDA convened more PFDD meetings than what was put forward in the agreement, helping to significantly advance our field. The same type of commitment – to do more – is needed today.

Conclusion

The EveryLife Foundation is encouraged by the provisions within the PDUFA VII Commitment letter. We are grateful to all of the community members who have worked so hard to ensure that the needs, priorities, opportunities, and urgency of our rare disease community is so strongly reflected in these considerations. And our collective patient community organization partners are eager to continue to lean in and work with the agency, sponsors, and patient communities to ensure that the rare disease communities' patient experience continues to inform considerations and decision-making all throughout the product development life-cycle.

Thank you for your tireless work on behalf of our rare disease community -- and for your commitment to ensuring that the **promise of today's pipelines** will change health outcomes for **THIS generation of patients.**