Patient Representative Participation in the Review Process: A Reviewer Perspective

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Patient Representative Input

- Participation in FDA Advisory Committee (AC) meetings
- Divisional Assignments
- Minisymposia
- Other opportunities, resources

Further integrating patient perspective into drug development and decision making

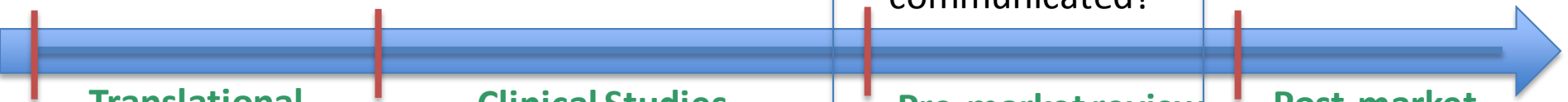
What matters most to patients and how can it be measured?

What patient outcomes should we measure? How can trials be more patient-friendly?

How can patient data be best integrated into FDA benefit risk determination?

How can this data be best communicated?

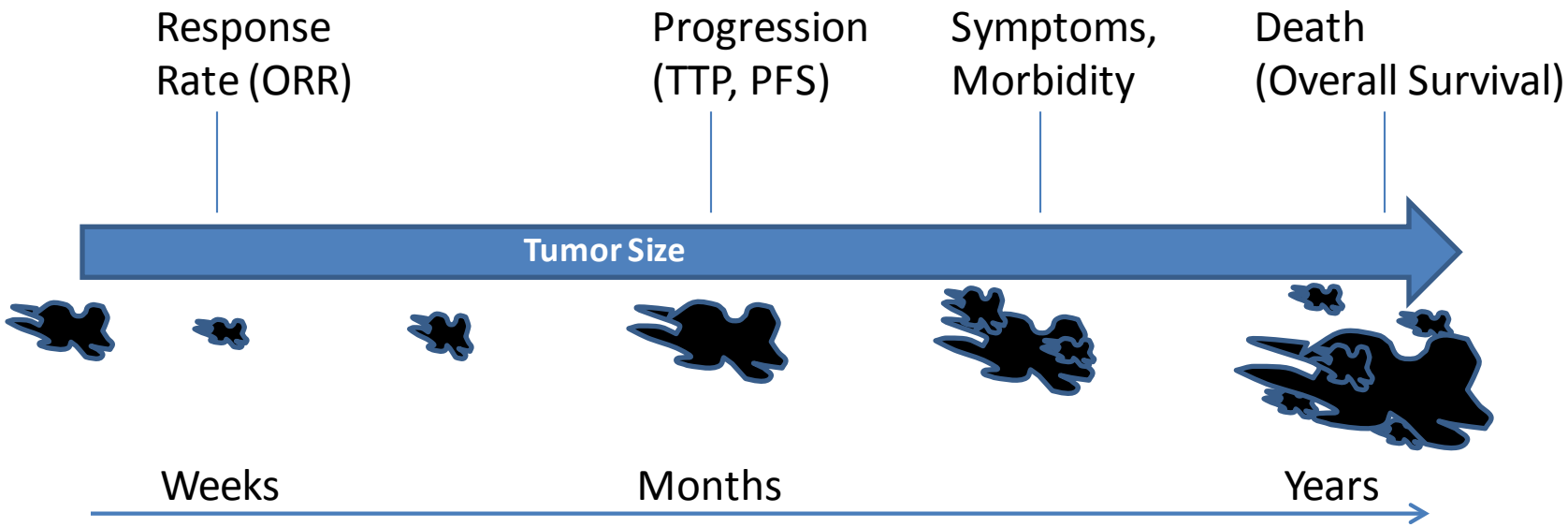
How can patient data be generated in the post-market setting?



Translational | Clinical Studies | Pre-market review | Post-market

Adapted slide with permission from Theresa Mullin, Office of Strategic Programs, FDA

First- A Crash Course on Standard Efficacy Endpoints in Oncology



Objective Response Rate (ORR): % of patients achieving a 30% shrinkage in tumor
Time to Progression (TTP): time from randomization to disease progression
Progression Free Survival (PFS): time from randomization to disease progression or death
Overall Survival: time from randomization to death from any cause

Why the Patient Perspective

- One challenge in NDA/BLA review is assessing true **clinical benefit**
  - Concrete study endpoints: OS, PFS
  - Surrogate endpoints: ORR (durable)
  - PRO

- "Cost" to achieve benefit= toxicity, and burden of therapy→ Benefit:Risk Assessment
- Scientific metrics vs. human impact and relevance

Patient Representative Participation in FDA AC Meetings and Divisional Assignments

- We need your input:
  - Efficacy data meaningful?
  - Burden of treatment?
  - Quality of Life
- Your opinion is unique
- **Sole source of patient perspective of clinical benefit in this context**

Myelodysplastic syndrome (MDS)

I personally have had over 700 units of blood. That's a unit of blood every week. Any drug that reduces or eliminates the need for transfusions is life saving. I can't tell you what that does to the quality of life – to be able to go six months or a year without a transfusion, spending 7 or 8 hours in a hospital each week when you have a disease that's life threatening – and many patients only have 2 – 4 years. There is no cure. Making patients transfusion independent is the next best thing

- Robert

Breast Cancer

It's a very painful reality that metastatic breast cancer is not curable. I don't think that means, then, that we should just say, "here, try this" if there isn't meaningful data to support it. In this study, as presented, there is missing data, there are inconsistencies, and I remain very uncomfortable about that. Considering all the toxicities that I and others have mentioned, I think that is too high a price to pay.

-Natalie

FDA Webinar, Patient Representative Program, Richard Klein, 2015; http://www.pharmacy.umaryland.edu/media/SOP/wwwpharmacyumarylandedu/centers/cersievents/pfdd/Klein.pdf

Divisional Assignments

- No AC, but patient perspective still critical
- Example: Safety issue not requiring an AC, rather input re: how it should figure into our calculations
  - How should it be conveyed?
- Other phases of drug development

Minisymposia

- Context (disease) specific
- Limited number, informal interaction
- Mutually educational and informative
- Individuals vs “the Agency”/ “the organization”

Patient Perspective: Summary

- We value your judgment
- Your opinion matters and your vote counts
- What do these mean to you?
  - “very well tolerated,” “no significant toxicity.”
- Service to the American public

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Thank you!