

# Patient Centered Approach: What Do Efficacy Endpoints Mean?

November 27, 2018

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# Session Agenda

- 1:45-2 pm: Introductions and Framework
- 2-2:45 pm: Efficacy Outcomes Discussion
  - Overall Survival (OS)
  - Progression Free Survival (PFS)
  - Overall Response Rate (ORR)
  - Disease Free Survival (DFS)
  - Time to Treatment Discontinuation (TTD) – time permitting
- 2:45-3 pm: Outreach and Education



# **INTRODUCTIONS AND FRAMEWORK**

# Framing the Problem

- Efficacy results from clinical trials are important, answering the question “**Does the therapy work?**”
  - Overall survival (OS)
  - Progression free survival (PFS)
  - Overall Response Rate (ORR)
  - Disease Free Survival (DFS)
  - Time to Treatment Discontinuation (TTD)
- Differences between them can be nuanced and difficult to understand

# Patient Perspectives

- “Endpoint” is commonly misunderstood by patients – some have described the term as “scary”
- “Survival” is interpreted as “cure” by some
- Stable disease is important
- Quality of life versus length of life versus cost – means different things to different patients

# **EFFICACY OUTCOMES**

# FDA Internal Survey



- Administrative personnel within FDA surveyed
- Asked whether they self-identified as having a medical background
- Goal: identify current understanding of certain efficacy endpoints

# Overall Survival

In your own words, describe what the term “overall survival” means (as it relates to how effective a cancer treatment is). Please DO NOT look up the definition online.

## Responses:

- The likelihood that you will live.
- How many survive in total.
- Patient is alive.
- Outcome from taking research meds.
- It means how well someone is responding to treatment.
- How long a patient is alive after they are diagnosed with the cancer.
- Percent likelihood a patient will survive.
- To me it means that your entire survival not just based off of what you are being treated for.
- Percentage of persons treated who remain alive for a period of time.
- The survival rate of the overall population using that treatment.
- The overall rate of a cancer treatment in ensuring survival.



# Overall Survival

- Proposed patient-friendly definition:
  - **The amount of time the patient is alive (with or without cancer) after the start of this treatment.**
- Question: Is this a clear and patient-friendly definition for OS?
- Discussion

# Progression Free Survival

In your own words, describe what the term “progression free survival” means (as it relates to how effective a cancer treatment is). Please DO NOT look up the definition online.

## Responses:

- Surviving without further progression of the disease. No further symptoms.
- Remission or someone has just received treatment.
- Cancer goes into remission.
- Tumors are not growing.
- It means after treatment that the cancer is still there but has not gotten worse.
- How long a patient lives without the cancer progressing.
- Likelihood a patient will survive if the current amount of cancer present does not progress.
- I have no idea.
- Percentage of persons treated whose cancer does not get worse for a period of time.
- No idea.
- The effectiveness of treatment in ensure the cancer does not progress any further from its current state.

# Progression Free Survival



- Proposed patient-friendly definition:
  - **The amount of time after the start of this treatment the cancer does not substantially grow and the patient is alive.**
- Question: Is this a clear and patient-friendly definition for PFS?
- Discussion

# Overall Response Rate

In your own words, describe what the term “overall response rate” means (as it relates to how effective a cancer treatment is). Please DO NOT look up the definition online.

## Responses:

- How patients reacted to the treatment.
- How many respond positively to treatments.
- Average rate in group of patients.
- How the tumor responds to treatment.
- It means how well people respond to treatment. It measures the way people react to treatment.
- How long cancer reduces and stays reduced before it progresses again.
- Percent of patients that have a positive response to any given treatment.
- How well one is responding and how slow or fast the treatment is helping in aiding one that is sick.
- Percentage of persons treated whose cancer is either cured, stopped from progressing, or reduced in scope for a period of time.
- For the whole population of patients using that treatment, the number of patients that showed some kind of improvement with the treatment.
- The effectiveness of the cancer responding to a treatment.

# Overall Response Rate

- Proposed patient-friendly definition:
  - **The percentage of patients whose cancer shrinks or disappears after treatment.**
- Question: Is this a clear and patient-friendly definition for ORR?
- Discussion

# Disease Free Survival

In your own words, describe what the term “disease free survival” means (as it relates to how effective a cancer treatment is). Please DO NOT look up the definition online.

Responses:

- Cured. No more disease.
- Cancer free.
- Cancer is completely gone.
- Treatment causes disease to be in a state of remission.
- It means that your cancer is in remission after treatment.
- How long a patient remains cancer free once the treatment is completed.
- Unsure.
- No traces of the disease at all.
- Percentage of persons treated whose cancer remains undetectable for a period of time.
- Treatment leads to recovery with no lingering/additional diseases.
- The effectiveness of treatment in ensure the employees survives w/o disease.

# Disease Free Survival



- Proposed patient-friendly definition:
  - **The amount of time from this potentially curative treatment that patients have no sign of cancer and the patient is alive.**
- Question: Is this a clear and patient-friendly definition for DFS?
- Discussion

# Time to Treatment Discontinuation



- Progression free survival is frequently used as an endpoint in clinical trials, but can be difficult to understand
- Not on FDA survey
- Question: Would TTD be a meaningful endpoint from a patient's perspective?
- Proposed patient-friendly definition:
  - **The amount time from when this treatment was started to when it was stopped.**
- Question: Is this a clear and patient-friendly definition for TTD?
- Discussion





# LOOKING AHEAD

# Spreading the Word



- Publish on OCE website
  - Use in FDA press releases, podcasts, etc.
  - Planned manuscript on session take-aways
  - Advocacy groups
  - Other stakeholders
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- What terms would you like to see included in this developing glossary of patient-friendly clinical trial language?

# Thank You!



- FDA
  - Paul Kluetz
  - Julia Beaver
  - Laleh Amiri-Kordestani
  - Janice Kim
  - PIP Organizing Committee
  - Panelists: Kevin Wright, Kristen Booze, LaShawn Griffiths
- Mary Lou Smith
- All attendees in person and online



**BACKUP**

# Event Free Survival

- Not on FDA survey
- Proposed patient-friendly definition:
  - **Generally, the amount of time from this treatment that the potentially curable cancer is not growing and the patient is alive.**
- Question: Is this a clear and patient-friendly definition for EFS?
- Discussion