FDA – CERSI Workshop

Advancing Use of Patient Preference Information as Scientific Evidence in Medical Product Evaluation

December 7, 2017
A world where no one dies of lung cancer.
WHO IS LUNGEVITY?

LUNGevity is an organization that seeks to:

UNDERSTAND

REPRESENT

SERVE

People diagnosed with lung cancer
LUNGEVITY’S THREE PILLARS

We accomplish this through:

• Funding research

• Providing comprehensive education and support services

• Working with policy, regulatory and other partners to improve timely access to treatments
PATIENT FOCUSED RESEARCH CENTER

LUNGevity
Patient FoRCe
Translating the patient voice into action
Project Transform
Patient Preference Study

History
Goals

• Understand disease symptoms and daily impacts that matter most to patients
• The patient perspective on treatment of this condition

Audience

• ~15 lung cancer patients and 15 patient representatives
• ~25 patients via live webcast
The in-person participants represented a higher proportion of
  • Women
  • Patients diagnosed more than 5 years ago
  • Patients whose cancer is currently in remission

In actuality,
  • Lung cancer population is closer to 50/50 split between men and women
  • There is only a small % of the overall patient population whose lung cancer is in remission.
 Patients find it difficult to distinguish between symptoms of the disease and side effects of cancer treatments

Lung cancer treatment decisions are highly individual and personal and depend on a number of things including:
- the patient’s disease manifestation
- the treatment options that are available to them
- their experiences with treatment and
- their personal circumstances

Another theme that emerged was that the impact on patients’ lives varies widely - all the way from debilitating to leading a “normal life”
Project Transform
Patient Preference Study

Overview
What do lung cancer patients want from treatments?

- Longer survival?
- Better quality of life?
- Fewer side effects? Which ones?
- Longer duration of disease-free survival?

What is the intersection between length of life and quality of life?
MULTI-STAKEHOLDER INITIATIVE

- LUNGevity Foundation
- Johns Hopkins University
- Patient Action Committee
- External Advisory Committee
WHO ARE THE PATIENT ACTION COMMITTEE (PAC) MEMBERS?

PAC members are:

• Living with lung cancer
• Actively involved in lung cancer advocacy
• Spread across the country
• Varied in disease severity (stages 1-4)
• Varied in years since diagnosis (1-13)
<table>
<thead>
<tr>
<th>Name</th>
<th>Institution/Position</th>
</tr>
</thead>
<tbody>
<tr>
<td>Jeff Allen</td>
<td>Friends of Cancer Research</td>
</tr>
<tr>
<td>Cynthia Grossman</td>
<td>PhD, Faster Cures</td>
</tr>
<tr>
<td>Joel Beetsch</td>
<td>Celgene</td>
</tr>
<tr>
<td>Frank Liu</td>
<td>Merck</td>
</tr>
<tr>
<td>Gideon Blumenthal</td>
<td>MD, FDA</td>
</tr>
<tr>
<td>Linnea Olson</td>
<td>lung cancer advocate, blogger, and artist</td>
</tr>
<tr>
<td>Philip Bonomi</td>
<td>MD, Rush University Medical Center</td>
</tr>
<tr>
<td>Salome Samant</td>
<td>MD, Merck</td>
</tr>
<tr>
<td>Julie Brahmer</td>
<td>MD, Johns Hopkins School of Medicine</td>
</tr>
<tr>
<td>Jamie Studts</td>
<td>PhD, University of Kentucky</td>
</tr>
<tr>
<td>Emuella Flood</td>
<td>ICON</td>
</tr>
<tr>
<td>Michelle Vichnin</td>
<td>Merck</td>
</tr>
<tr>
<td>Susan Gorky</td>
<td>Celgene</td>
</tr>
</tbody>
</table>
Project Transform
A partnership between LUNGevity and researchers at Johns Hopkins University to incorporate the patient experience into lung cancer research, treatment, and policy.

**Engaging**
Working with lung cancer patients to determine the scope and focus.

**Understanding**
Developing an awareness of the lived experience of lung cancer patients.

**Measuring**
Designing, development, disseminating a survey instrument in partnership with lung cancer patients.

**Implementing**
Using the results to advocate patient centered lung cancer research, treatment and policy.
PILOT STUDY

Estimating time equivalents for cancer side effects among lung cancer survivors and caregivers: a discrete-choice experiment

Andrea Ferris, MBA; Upal Basu Roy, PhD, MPH; John FP Bridges, PhD; Ellen M Janssen, BA; Sydney M Dy, MD

'LEgevity Foundation; †Johns Hopkins Bloomberg School of Public Health; ‡Johns Hopkins School of Medicine

Project Transform was initiated in 2015 to integrate the patient experience into lung cancer treatment, research, and policy. Project Transform’s vision is to ensure that the preferences of patients with lung cancer are recognized, their values are valued, and that living well with lung cancer can be the norm.

Objective

Lung cancer is the leading cause of cancer mortality in the US [1]. The treatment landscape of lung cancer has evolved over the last two years, and novel treatments have improved outcomes. With improved survival, issues of long-term side effects and quality of life arise. Project Transform aims to change the paradigm in lung cancer from assumptions being made about patient wishes to evidence-based conclusions about patient desires about their treatments.

Approach

Through rigorous engagement of a national advisory board of lung cancer survivors, a discrete-choice experiment (DCE) was developed, prototyped, and piloted [2]. The DCE was administered to 114 lung cancer survivors and caregivers at LUNGevity’s National HOPE Summit. Respondents completed 13 paired-comparison choice tasks described across six attributes. The preferences for avoiding side effects were estimated using their time equivalents by using maximum simulated likelihood.

Table 1 – Attributes and levels

<table>
<thead>
<tr>
<th>Attribute</th>
<th>PFS</th>
<th>Short term side effects</th>
<th>Long term side effects</th>
</tr>
</thead>
<tbody>
<tr>
<td>Levels</td>
<td></td>
<td>Mild</td>
<td>None</td>
</tr>
<tr>
<td>6 months</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>12 months</td>
<td></td>
<td>Moderate</td>
<td>Mild</td>
</tr>
<tr>
<td>18 months</td>
<td></td>
<td>Severe</td>
<td>Moderate</td>
</tr>
</tbody>
</table>

Table 2 – Respondent Demographics

<table>
<thead>
<tr>
<th>Respondent type</th>
<th>Patient—N (%)</th>
<th>Caregiver—N (%)</th>
<th>Total Sample (n = 114)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Years since diagnosis</td>
<td>4.43 (1.20)</td>
<td>4.43 (1.20)</td>
<td>4.43 (1.20)</td>
</tr>
<tr>
<td>Disease stage</td>
<td>81 (61)</td>
<td>81 (61)</td>
<td>81 (61)</td>
</tr>
<tr>
<td>Treatment received</td>
<td>Chemotherapy</td>
<td>Chemotherapy</td>
<td>Chemotherapy</td>
</tr>
</tbody>
</table>

Conclusions

Lung cancer survivors:
1. Value PFS as the most important component in their treatment choice.
2. Consider functional long-term side effects as important in their treatment choice.
3. Value reduction in long-term side effects the same as increasing PFS by 1.35-3.59 months.

We are grateful to the lung cancer survivor community for making this study possible. Funding provided by Calgene.
• National survey with focus on reaching unengaged patients
• N ≥ 1000
• Using novel recruitment strategy – Patient Ambassadors
• Working with partners
• Include demographic data to look at:
  o Histology
  o Age, race, income
  o Line of therapy
  o Type of therapy
RELEVANCE TO DIVERSE STAKEHOLDERS

**Patient**
Development of patient-centric endpoints for clinical trials
Incorporation of the patient voice

**Provider**
Patient-oriented education to empower and help patients become a partner in their treatment decisions

**Pharma**
Design clinical trials that are meaningful to the lung cancer patient, leading to increased patient recruitment

**Regulatory**
Lay informational groundwork for patient-centered regulatory process
QUESTIONS?