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FDA – CERSI Workshop

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

December 7, 2017

LUNGEVITY'S VISION

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A world where no one dies of lung cancer.

WHO IS LUNGEVITY?

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LUNGevity is an organization that seeks to:

UNDERSTAND

REPRESENT

SERVE

People diagnosed with lung cancer

LUNGEVITY'S THREE PILLARS

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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

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Project Transform Patient Preference Study

History

FDA PFDD MEETING – 6/2013

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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

FDA PFDD MEETING – 6/2013

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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.

MEETING THEMES

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- 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

PROJECT TRANSFORM

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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

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- LUNGevity Foundation
- Johns Hopkins University
- Patient Action Committee
- External Advisory Committee

WHO ARE THE PATIENT ACTION COMMITTEE (PAC) MEMBERS?

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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)

EXTERNAL ADVISORY COMMITTEE

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Jeff Allen, *Friends of Cancer Research*	Cynthia Grossman, PhD, *Faster Cures*
Joel Beetsch, *Celgene*	Frank Liu, *Merck*
Gideon Blumenthal, MD, *FDA*	Linnea Olson, *lung cancer advocate, blogger, and artist*
Philip Bonomi, MD, *Rush University Medical Center*	Salome Samant, MD, *Merck*
Julie Brahmer, MD, *Johns Hopkins School of Medicine*	Jamie Studts, PhD, *University of Kentucky*
Emuella Flood, *ICON*	Michelle Vichnin, *Merck*
Susan Gorky, *Celgene*	

PROJECT TRANSFORM

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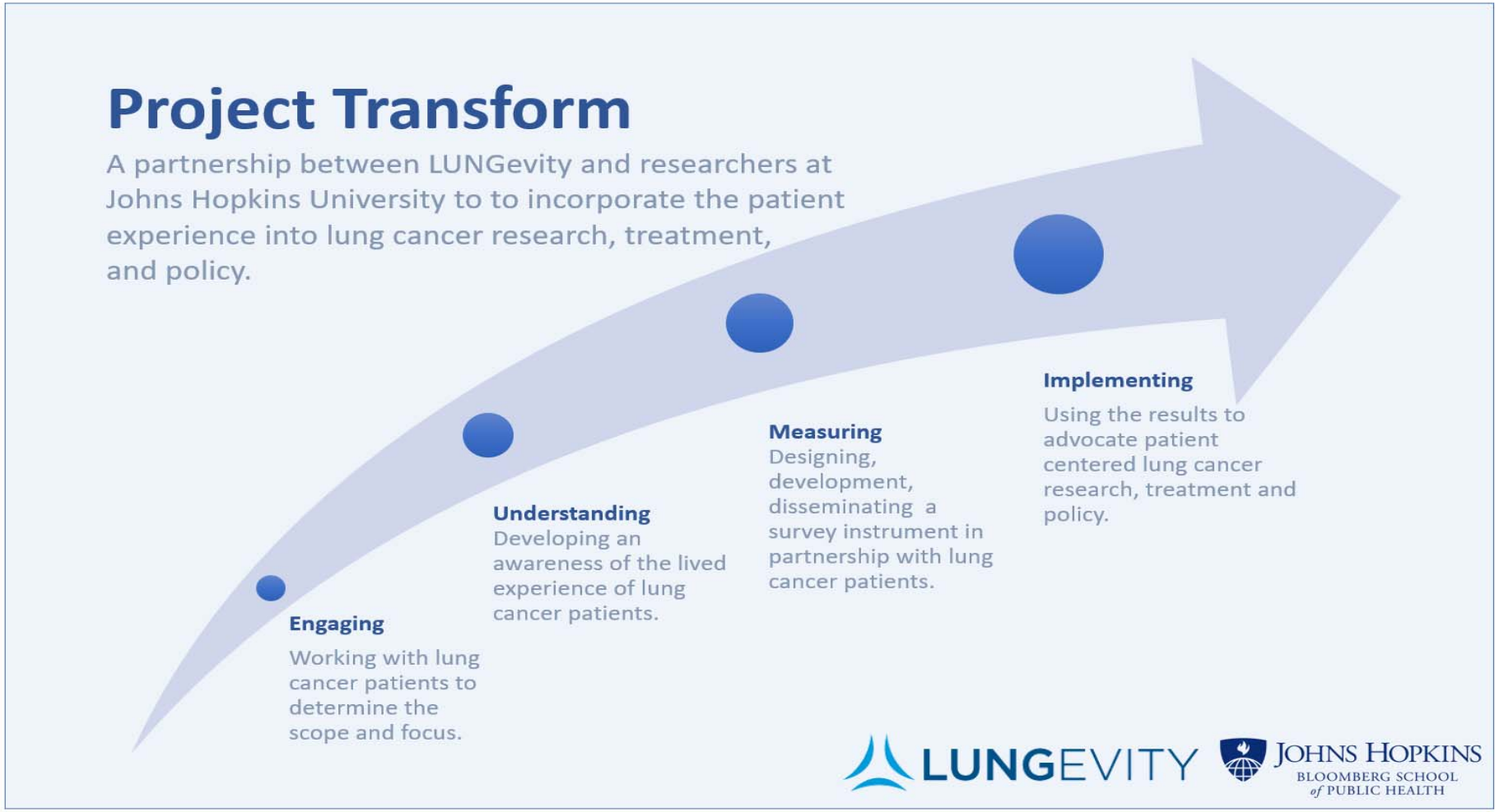

PILOT STUDY

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

Andrea Ferris, MBA[1]; Upal Basu Roy, PhD, MPH[1]; John FP Bridges, PhD[2]; Ellen M Janssen, BA[2]; Sydney M Dy, MD[2,3]

[1]LUNGevity Foundation; [2]Johns Hopkins Bloomberg School of Public Health; [3]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 evolve din the past 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, pretested 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 preference for avoiding side-effects were estimated using their time equivalents by using maximum simulated likelihood.

Table 1 – Attributes and levels

Attribute	PFS	Short-term side effects	Long-term side effects
Levels	6 months	Mild	None
	12 months	Moderate	Mild
	18 months	Severe	Moderate

Results

What is a discrete choice experiment?

A DCE is based on the idea that even if people can't provide a direct measure of value, they can usually indicate which scenario they prefer. Choices are made for a hypothetical third person with specific health outcomes to minimize biases that can arise due to personal choices for treatments.

EXAMPLE

Please select the person you think is better off:		
Attributes	Person A	Person B
Progression free survival	6	18
Short-term side effects	Mild	Moderate
Physical long-term effects	Mild	None
Emotional long-term effects	Moderate	None
Cognitive long-term effects	None	Moderate
Functional long-term effects	None	Mild
Who do you think is better off?	☐	☐

Table 2 – Respondent Demographics

		Total sample (n = 114)
Respondent type	Patient – N (%)	87 (76%)
	Caregiver* – N (%)	24 (21%)
Years since diagnosis – mean (SE)		4.43 (0.28)
Lung cancer type	Adenocarcinoma – N (%)	81 (71%)
Disease stage	Stage 1-3 – N (%)	9 (9%)
	Stage 4	53 (46%)
Treatment received	Chemotherapy	72 (63%)
	Radiation	50 (44%)
	Targeted Therapy	59 (52%)
	Immunotherapy	15 (13%)
	Surgery	54 (47%)

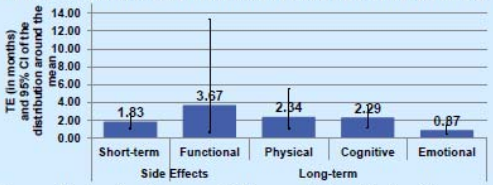
*caregivers responded for the patient they were a caregiver for

Major Findings

"Well, maybe I might actually live, and if I do, I want to live with quality."

National Patient Advisory Board member on why participation in Project Transform is important

Figure 2 – Additional PFS required to accept an increase in side effect severity



Respondents valued a one-unit decrease in functioning the most (equivalent to extending PFS by 3.67 months). Changes in physical (2.34) and cognitive (2.29) long-term effects were valued more than a composite of short-term side effects (1.83).

Conclusions

Lung cancer survivors
1. Value PFS as the most important component in their treatment choice
2. Consider unctional long-term side effects as important in their treatment choice
3. Value reduction in long-term side effects the same as increasing PFS by 1.39-3.59 months

We are grateful to the lung cancer survivor community for making this study possible. Funding provided by Celgene

[1] U.S. National Institutes of Health. National Cancer Institute. SEER Cancer Statistics Review, 1975-2012. 2015. [2] Bridges JF, Janssen EM, Ferris A, Dy SM. Project Transform: Incorporating the patient experience into lung cancer treatment, research, and policy. *Working Paper*

NATIONAL SURVEY

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- 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:
    - Histology
    - Age, race, income
    - Line of therapy
    - Type of therapy

RELEVANCE TO DIVERSE STAKEHOLDERS

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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

CONCLUSION

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QUESTIONS?