IVI INNOVATION AND VALUE INITIATIVE

UNDERSTANDING VALUE THROUGH THE PATIENT'S EYES: PERSPECTIVES FROM PATIENTS WITH GENETIC MUTATIONS IN NON-SMALL CELL LUNG CANCER TREATMENT

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Objective

To understand the perspectives and experiences of patients diagnosed with mNSCLC, and the factors that inform their cancer-related treatment decision-making and treatment goals in order to identify determinants of value

Background

- Targeted therapies for non-small lung cancer (NSCLC) have vastly improved survival and other outcomes for patients whose tumors have genetic mutations such as ALK, BRAF, EGFR, and ROS1.
- Identification of genetic mutations often indicates a mutation-specific course of therapy, however, the relationship between genetic mutation status, patient treatment preferences, and other determinants of value in NSCLC cancer care is not well understood.
- Awareness of the importance of the patient perspective in value assessment has led to an acknowledgment of the need to systematically and rigorously include considerations and determinants of value that are most meaningful to patients.[1-3]
- To include mNSCLC patient perspectives on value in a new open-source value model focused on NSCLC, primary qualitative data on patient experience and priorities were needed.

IVI's Open-Source Value Project (OSVP)

- The Innovation and Value Initiative (IVI) is a nonprofit organization that seeks to advance the science and improve the practice of value assessment in the U.S. healthcare system.
- The OSVP is a transparent and open-source system for estimating the value of medical technologies that centers on the patient experience, allows for a broad range of perspectives, incorporates the latest available evidence, and considers the full range of scientifically defensible approaches.
- A primary goal of the OSVP process is meaningful engagement of patients in model development, evaluation, and revision through: (i) patient input in development phases; (ii) qualitative research on patient perspectives on value; (iii) patient input in public comment period; and (iv) patient participation in Technical Expert Panels that recommend specific changes to models.
- IVI develops disease-specific decision models as part of the OSVP, with the most recent model focused on EGFR+ NSCLC: **www.thevalueinitiative.org/ivi-nsclc-value-model**

Methods

- We conducted structured in-depth interviews and focus groups between June and July 2018 with metastatic NSCLC (mNSCLC) patients to investigate patient perspectives of disease burden, experiences with treatment, and the impact of cancer therapy on patients' lives and treatment decision-making.
- Inclusion criteria:
  - Individual diagnosed with de novo or recurrent stage IV NSCLC
  - Age ≥18 years
  - Fluent in English
- Eligible participants residing in two large metropolitan regions were invited by email and screened by telephone to confirm eligibility by Schlesinger Group, a market research firm.
- Semi-structured discussion guides were designed to elicit thoughts, opinions, and experiences about cancer care and treatment, as well as reports of the factors most important to patients when considering treatment options and sources of perceived value in treatment for mNSCLC **(Table 1)**.
- Discussions were audio-recorded and transcribed verbatim.
- Thematic analysis was used to identify salient themes and factors that patients with mNSCLC consider meaningful when making treatment decisions; and the degree of concordance between patients on the issues identified.
- Study protocol and discussion guides were reviewed and approved by Advarra Institutional Review Board (Columbia, MD).

Table 1: Sample Discussion Guide Questions

Domain	Objective	Question
Diagnosis	Understand diagnosis experience	• What motivated you to see a doctor (symptoms, family history, annual screening, etc.)? • What questions did you have for your oncologist or care team? • Did you run into any challenges during the diagnosis process? Please explain.
Initial Treatment Decision Making	Understand how treatment options were presented and prioritized	• After your diagnosis, what types of treatment options were offered to you? • How did you discuss and evaluate the benefits, risks, and costs associated with the various options?
Subsequent Treatment Decision Making	Explore how patients' treatment decisions changed over time	• Throughout the course of your treatment for NSCLC, can you describe the different types of care and/or treatments that your healthcare team has provided or prescribed? • Looking back across your cancer treatment journey, how have your decisions about treatment or your approach to decisions about treatment changed over time, if at all?
Values and Preferences	Understand what matters to patients and identify determinants of value	• What matters the most to you about the care that you receive for your cancer?

Results

Participant Demographics

- Of 19 total participants with mNSCLC (mean [SD] age, 55.8 [12.6] years; 79% female), 15 (79%) reported a known genetic mutation and nearly three-quarters of the study population had been diagnosed with metastatic disease at initial diagnosis (73.7). **(Table 2)**

Key Findings

- Participants cited a number of trade-offs that they were required to make after receiving their diagnosis and throughout their treatment journey, including:
  - Weighing treatment efficacy against the potential impacts of treatment on QOL and day-to-day functioning (particularly given an uncertain or poor long-term prognosis),
  - Tolerance of side effects, and
  - Availability of therapies to mitigate side effects.

> *Well, every therapy had some side effects. But basically, I was looking for something that had fairly low adverse events… things like cardiovascular…things like .a little bit of diarrhea or nausea, I mean, that's not a big deal. You're looking for more serious: anything respiratory, CV, some kind of anemia…other than my diagnosis, I'm healthy…. it's much more challenging when people have what they call other comorbidities. So maybe they come in and they've got other conditions that can really impact the way the therapy works because of the potential for side effects to work against each other. ...so you're weighing the efficacy of the therapy, but you're also weighing, well, what are the side effects?*

- Participants with identified mutations valued the oncogene testing they received and reported facing different decisions than those without known mutations.

> *…I think that with this particular cancer and where we're at with research, that many of us are being forced into accepting anything that we think will work because you get to a point when the one that's FDA approved, when you develop resistance and you have to find something else, you're going to be willing to try just about anything to keep going.*

- Participants highlighted unmet needs for diagnosis, treatment, and support tailored to their genetic mutation sub-group, including a critical need for better provider training and awareness of genetic testing and mutation-specific treatment options.
- Several participants also described their involvement in education about NSCLC and their particular mutation, contributions to clinical research for their mutations, and fundraising events for research, with the support of their mutation sub-groups.

> *Well, my perspective with the ROS1 is that, right now, there's a very limited pool of therapies that applies directly to this mutation. So we have one that's FDA approved, and after that, it's a series of clinical trials. So being in this group, it almost feels like you have to step up your knowledge; you have to start reading research studies and keep abreast of what's happening because it's changing so fast that if you don't have a wider pool of knowledge to draw from, it might be hard to know that your oncologist was also staying on top of it as well.*

> *So we're going to see this cancer's not so rare. So it's going to get more research dollars for it. We're going to get more awareness of it. And everybody in the future's going to benefit from us pushing forward with it.*

- Across the different genetic mutation patient sub-groups, mutation-specific social media and support networks were highly valued.
  - Participants valued connecting with and the support of other patients with the same mutation.
  - Social media was especially valued by those with rare mutations, limited treatment options, or less experienced providers.

> *…it is a group that is all ROS1 positive cancer survivors, and on Facebook, it's a really tight-knit community where people are posting when they go for their scans and their results. And then what I find valuable now is that there are people that are on there that have been on the drug I was on—or I am on for 5 to 10 years and have just started to develop resistance, and they're talking about what their next steps are. So it's kind of nice to know that it does work for a while and there are plans coming up next.*

Table 2: Participant Demographics

	n/mean	% (SD)
Total	19	100
Gender		
Female	15	80.0
Male	4	20.0
Age, mean (SD), years	53.8	(12.1)
Race/Ethnicity		
Asian	1	5.3
Hispanic	1	5.3
Caucasian	18	94.7
Educational Attainment		
High school (or equivalent)	2	10.5
Some college	4	21.1
College	8	42.1
Post Graduate	5	26.3
Employment		
Employed full-time	5	26.3
Employed part-time	3	15.8
Unemployed (retired, homemaker, health)	11	57.9
Household Income		
Less than $25,000	---	---
$25,000-$35,000	4	21.1
$36,000-$50,000	1	5.3
$51,000-$75,000	7	36.8
$76,000-$100,000	3	15.8
$101,000 or more	3	15.8
Prefer not to answer	1	5.3
Insurance		
Medicare	4	21.1
More than one plan	3	15.8
Private health plan offered through an employer or other organization	9	47.4
Private health plan or insurance that you purchased yourself	2	10.5
Other	1	5.3
Time since diagnosis, mean (range), years	4.1	2.6
Stage at initial diagnosis		
Stage III	5	26.3
Stage IV (de novo)	14	73.7
Mutation Status*		
ALK	3	15.8
EGFR	9	47.4
KRAS	1	5.3
ROS1	2	10.5
Other	2	10.5
None of Above	3	15.8

Limitations

- The study sample was educated, largely insured, and English-proficient. Other less advantaged populations – due to race/ethnicity, acculturation, language proficiency, socioeconomic status, access to care, or insurance status – may deliberate other factors related to mNSCLC treatment decision-making that merit investigation.
- This study included a convenience sample of patients and participant perspectives may not be generalizable to the broader mutation positive mNSCLC cancer patient population. As this was a self-selected sample, in which participants volunteered to be part of the focus groups, a larger scale study is needed to establish generalizability of findings.

References

1. Perfetto, E.M., et al., Emerging Good Practices for Transforming Value Assessment: Patients’ Voices, Patients’ Values. Value in Health, 2018. 21(4): p. 386-393.
2. Perfetto, E.M., et al., Value to Whom? The Patient Voice in the Value Discussion. Value in Health, 2017. 20(2): p. 286-291.
3. Seidman, J., et al. (2017). Patient-Perspective Value Framework. (PPVF) https://www.fastercures.org/programs/patients-count/patient-perspective-value-framework/

Disclosure

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