FDA Oncology Center of Excellence
Initiatives to Advance Patient Engagement

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Jessica Boehmer, MBA
Vishal Bhatnagar, MD

NHC Science of Patient Engagement Symposium
September 29, 2020
Introduction & Overview
‘Lola Fashoyin-Aje, MD, MPH
Deputy Director, DO3;
Assoc. Director for Science & Policy to Address Disparities

Opportunities for Patients to Engage with FDA
Jessica Boehmer, MBA
Regulatory Scientist, Office of Oncologic Diseases, OND/CDER

Project Patient Voice
Vishal Bhatnagar, MD
Associate Director for Patient Outcomes, OCE
Introduction & Overview

‘Lola Fashoyin-Aje, MD
Deputy Director, DO3;
Assoc. Director for Science &
Policy to Address Disparities
Project Equity

SCIENTIFIC AND POLICY PROGRAM TO ADDRESS CANCER DISPARITIES AND PROMOTE HEALTH EQUITY

ONCOLOGY CENTER OF EXCELLENCE

Lola Fashoyin-Aje, MD, MPH
U.S. Food and Drug Administration
Racial/ethnic minorities in clinical trials

• Racial/ethnic minorities underrepresented in clinical research
  • Clinical trials supporting approval of cancer therapeutics: less than 10% patients are racial/ethnic minorities

• Contributing factors
  • Clinical trials are global
    • Approx. 20% of patients are enrolled from the U.S.
    • US racial/ethnic categories may not be applicable outside US
      • Ethnicity frequently not reported
    • Lack of diversity in global trial sites
  • Trials enrolling patients entirely outside the US may support approval in the U.S.
Addressing Underrepresentation

• Improve access to potentially promising drugs
  – Quick access important for pts with life-threatening illnesses/conditions like cancer
  – For many diseases, CTs offer best treatment options

• Generate evidence
  – Lack of data ≠ Lack of difference
  – Data needed to characterize parameters (e.g., drug metabolism, disease biology, etc.,) that may result in clinically important differences in safety &/or efficacy by race or ethnicity
Oncology Center of Excellence Initiatives

• Community outreach program
  • build relationships with communities, patients/patient advocates

• Science & Policy program
  • coordinate efforts to improve the inclusion of underrepresented demographic subgroups to generate data that informs safe and effective use of cancer therapeutics
  • Seeking diverse perspectives to inform regulatory decisions
Science & Policy Program

• Increase enrollment in oncology clinical trials
  – Increase data collected in the pre- and post-approval settings
  – Identify data that may be useful/feasible/informative to collect in the pre-and post-approval settings

• Engage in internal and external research and policy efforts
  – Provide transparency regarding data gaps

• Develop policy/guidance

• Integration with all OCE programmatic areas
  – Pediatric program
  – Real world data/Real world Evidence
  – Patient Voice
Patient/Patient Advocacy in OCE Activities

• Formal Agency programs (Jessica Boehmer to discuss)
  – Special Government Employee (marketing applications)
  – Patient Listening sessions

• OCE-specific
  – Workshops
  – Conferences (AAADV, ASCO, San Antonio Breast, etc.)
  – Mini symposia
  – OCE Patient listening sessions
  – Research/policy collaborations with patient advocacy groups (PanCAN, Cholangiocarcinoma Foundation, LUNGevity, etc.,)
Opportunities for Patients to Engage with FDA

Jessica Boehmer, MBA
Regulatory Scientist
Office of Oncologic Diseases
FDA/CDER/OND
Overview

• Evolution of patient engagement at FDA
• FDA patient engagement opportunities
• FDA Patient Representative Program®
• Patients Ask FDA
• Resources
<table>
<thead>
<tr>
<th>Year</th>
<th>Event Description</th>
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<tbody>
<tr>
<td>1988</td>
<td>Office of AIDS Coordination established</td>
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<tr>
<td></td>
<td>- Office of AIDS Coordination renamed to Office of AIDS and Special Health Issues (OASHI) and broadened to include patients with cancer and other serious and life-threatening diseases</td>
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<tr>
<td></td>
<td>- First FDA Patient Representative® served on an advisory committee</td>
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<td>1993</td>
<td>- FDA Patient Representatives® received voting rights on advisory committees</td>
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<td>1996</td>
<td>- FDA Patient Representative Program® role expanded to serve as consultants to scientific and regulatory reviewers</td>
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<tr>
<td>2001</td>
<td>- Patients and consumers encouraged to report medical product problems using FDA’s existing MedWatch system</td>
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<td>2008</td>
<td>- A section of the FDA website is created specifically For Patients</td>
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<td>2012</td>
<td>- Internal working group examines ways to increase patient involvement in FDA processes</td>
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<td>2013</td>
<td>- Consumer-friendly form introduced in FDA’s MedWatch system to report medical product problems</td>
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<td>2015</td>
<td>- Patient Preference Information (PPI) framework and guidance for medical device decision making</td>
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<td></td>
<td>- Patient Engagement Advisory Committee (PEAC) meetings regarding medical devices</td>
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<td>2016</td>
<td>- FDA and European Medicines Agency (EMA) Patient Engagement Cluster created</td>
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<td>- First Patient Council (internal) meeting held</td>
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<td>2017</td>
<td>- PAS established in the Office of the Commissioner</td>
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<td>- Public Workshop on PFDD guidance</td>
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<td>2018</td>
<td>- Memorandum of Understanding with National Organization For Rare Disorders (NORD) launched the Patient Listening Session pilot program</td>
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<td>- Patient Engagement Collaborative (PEC) launched with Clinical Trials Transformation Initiative (CTTI)</td>
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<td>- Center for Devices and Radiological Health (CDRH) Patient and Caregiver Connection (P&amp;CC) program launched</td>
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<td></td>
<td>- Public Workshops on PFDD guidances and drafts released</td>
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<td>2019-2020</td>
<td>- Patient Affairs Staff (PAS) online webform, Patients Ask FDA</td>
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<td>- PFDD Workshop on Guidance 4</td>
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<td>- Draft PFDD Guidance 2 released</td>
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<td>- COVID-19 Patient Resources Page Launched</td>
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<td>- Final PFDD Guidance 1 released</td>
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Slide credit: Andrea Furia-Helms, MPH, Director, FDA Patient Affairs Staff
Patient Engagement Opportunities

- Rare Disease Patient Listening Sessions
- Patient Engagement Collaborative (PEC)
- FDA Patient Representative Program®
- Patient-Focused Drug Development Meetings
- Devices Patient Engagement Advisory Committee (PEAC)
Oncology Center of Excellence (OCE) COVID-19 Listening Sessions

OCE held 11 listening sessions with patient advocacy groups and community stakeholders on impacts of COVID-19
What is the FDA Patient Representative Program?

FDA’s flagship program aimed at including patient and caregiver perspectives to the Agency’s decision-making process as it regulates medical products (drugs, biologics, and devices).

Access to confidential and proprietary data
Special (or Regular) Government Employees

PRP slides credit: Salina Miller, MS, MBA, Manager, FDA Patient Representative Program
FDA Patient Representative Program®

Serve on FDA Advisory Committee Panels

• A panel of outside experts convened periodically to advise the FDA on safety and efficacy issues about regulated medical products.

• 31 Advisory Committees; Medical Devices Advisory Committee (18 panels)

• Committee/Panel members include:
  • Chair
  • Medical Experts
  • Statisticians
  • Consumer Representative
  • Industry Representative
  • FDA Patient Representative®

FDA Patient Reps on Advisory Committee Panels
FY20: 36 Patient Reps assigned
FY19: 45 Patient Reps assigned
FDA Patient Representative Program®

Consult with Reviewers and Clinical Team

• Brings the patient voice to regulatory discussions earlier in the process
• Consult directly with clinical review staff and sponsors
• Closed/confidential teleconference

Consult with FDA Reviewers and Clinical Team
FY19-20: 6 assignments
What CRITERIA is Used?

• **Personal experience:** with a disease, condition or device (self or caregiver)
• **Advocacy experience:** active within patient community, understands issues and concerns
• **Knowledgeable:** aware of current treatment options and research
• **Analytical and scientific:** should grasp basic scientific principles and issues
• **Good communications skills:** comfortable with public speaking
• **Commitment to serve:** reliable and committed to service
• **Remain unbiased and unconflicted:** activities and roles should not conflict with assignment—financial, appearance, ethical considerations
Disease, Condition & Device Experiences Represented in Program

200 FDA Patient Representatives
300-500 diseases/conditions/device experiences

- AIDS/HIV
- Alzheimer’s Disease
- Asthma
- Breast implants
- Cancers--including: colon cancer, brain tumors, rare cancers, breast cancer, renal cell carcinoma, neuroendocrine tumors, leukemia, ovarian, pancreatic, multiple myeloma, pediatric cancers
- Cardiovascular disease
- Cerebral Palsy
- Crohn's disease
- Cystic Fibrosis
- Diabetes
- Duchenne Muscular Dystrophy
- Fabry Disease
- Infantile Spasms
- Inborn Errors
- Lysosomal Acid Lypase Deficiency
- Major Depressive Disorder
- Muscular Dystrophy
- Nicotine Replacement Therapy
- Opioid Use
- Organ transplantation
- Parkinson’s Disease
- Pompe Disease
- Schizophrenia
- Short Bowel Syndrome
- Sickle Cell Disease
When a Patient Speaks...

Gigi McMillan, FDA Patient Representative®
FDA Patient Representatives bring the non-scientific points of view that are from their real-world experience.

Video
FDA Patient Representative Program®

QUESTIONS?
Salina Miller, MS, MBA
Manager, FDA Patient Representative Program
salina.miller@fda.hhs.gov

ABOUT THE PROGRAM
Patient Engagement Across FDA

FDA Patient Affairs Staff:
PatientAffairs@fda.gov
https://www.fda.gov/PatientAffairs

FDA Patient Representative Program:
FDAPatientRepProgram@fda.hhs.gov
https://go.usa.gov/xfB4h

Patient Engagement Initiatives:
https://go.usa.gov/xfBdx
CDRH_PatientEngagement@fda.hhs.gov

Patient Engagement Meeting Requests:
CDRH_PatientMeetings@fda.hhs.gov

CDRH’s Division of Industry and Consumer Education:
DICE@fda.hhs.gov

Office of the Commissioner
Center for Biologics

Center for Devices
Center for Drugs

CBER’s Patient Engagement Initiatives:
CBERPatientEngagement@fda.hhs.gov

Office of Communication, Outreach and Development: OCOD@fda.hhs.gov

Professional Affairs and Stakeholder Engagement:
https://go.usa.gov/xfBpG
CDERPASE@fda.hhs.gov

CDER Division of Drug Information:
https://go.usa.gov/xfBpM
DrugInfo@fda.hhs.gov

Patient Focused Drug Development:
https://go.usa.gov/xfBph
patientfocused@fda.hhs.gov

Resources

Slide credit: Andrea Furia-Helms, MPH, Director, FDA Patient Affairs Staff
Project  Patient Voice

Vishal Bhatnagar, MD
Associate Director for Patient Outcomes, OCE
**ClinRO**
A measurement based on a report that comes from a trained health care professional after observation of a patient’s health condition.

**PRO**
A measurement based on a report that comes directly from the patient about the status of the patient’s health condition without interpretation of the patient’s response by a clinician or anyone else.

**ObsRO**
A measurement based on a report of observable signs, events or behaviors related to a patient’s health condition by someone other than the patient or a health care professional.

**PerfO**
A measurement based on a standardized task performed by a patient, administered and evaluated by an appropriately trained individual or independently completed and intended to assess or infer patient capabilities relevant to their day-to-day functioning.
PRO in labels of FDA Hematology and Oncology Products

PRO Objective
- Efficacy
- Safety/Tolerability
- Preference
21st Century Cures Act encourages FDA to review and communicate patient experience data submitted in product reviews

PRO data are frequently submitted; heterogeneity exists in analysis and presentation of data

Product label (USPI) offers limited space to communicate patient experience data adequately
Project Patient Voice is an online platform for patients and caregivers along with their healthcare providers to look at patient-reported symptom data collected from cancer clinical trials.

Launched June 23rd, 2020

**Aim:** web-based source of patient experience data accessible to patients, caregivers, and providers

July 17th public workshop & now reviewing feedback

https://www.fda.gov/about-fda/oncology-center-excellence/project-patient-voice
Patient-Reported Nausea During the First 24 Weeks on Treatment for Patients Who Completed a Questionnaire:

Figure 1 shows the percentage of patients reporting how often they had Nausea at each time point. For example, at week 2, 20% of patients taking Tagrisso reported Nausea (ranging from Rarely to Frequently). The range of patients who had any Nausea during the first 24 weeks of treatment with Tagrisso was between 12% - 29%. Click here for more information on how to read the graphs below.

<table>
<thead>
<tr>
<th>Tagrisso 80 mg</th>
<th>Chemotherapy</th>
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Snap shots: patients who filled in PRO-CTCAE that week

2 sets of horizontal stacked bar charts: all patients & patients with no symptoms at baseline

Data available for download for all bar charts
Next Steps for Project Patient Voice

- Review and analyze feedback obtained from the July 17th workshop
- Conduct focus groups and incorporate feedback for the next iteration of data visualizations
- Seek sponsor volunteers for the next phase
Acknowledgements

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- Laura Lee Johnson

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- Andrea Furia-Helms, MPH, Director, FDA Patient Affairs Staff
- Salina Miller, MS, MBA, Manager, FDA Patient Representative Program