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Patient Engagement: FDA Perspectives and Initiatives

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REiNS Winter Meeting

December 5, 2016

I have nothing to disclose.

Outline

- Advantages of engaging patients in drug development
- Existing FDA programs and outcomes
- Future plans for increasing patient engagement through FDA
- Challenges to meaningful patient engagement

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What is Patient Engagement?

- Refers broadly to obtaining patient perspectives at all stages of drug and device development
- Examples of strategies include:
  - Gathering patient advice and perspectives at meetings and public workshops
  - Obtaining written public comments
  - Acquisition of patient experience data generated through qualitative research or social media
  - Incorporation and analysis of clinical outcomes assessments data in clinical trials.

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Goals of Patient Engagement in Drug Development

- More meaningful clinical trial endpoints
- Outcomes in alignment with patient needs
- More robust assessment of adverse events and post market surveillance
- Approvals of medical products that better reflect outcome and quality of life measures most important to patients
- FDA decisions that better reflect patient tolerance for risk

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FDA Patient Representative Program

- Patients have an active role on FDA Advisory Committees and serve as consultants to offices across the agency
  - Over 200 patients and caregivers representing over 300 diseases and conditions
  - Special Government Employees (SGEs)

- Presence at the table

http://www.fda.gov/ForPatients/About/ucm412709.htm

Advisory Committees Meetings and Review Division Assignments

Year	Committee Meetings	Divisional Assignments
2012	27	4
2013	66	11
2014	55	12
2015	48	14
2016	34	3

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What is Patient Focused Drug Development?

- A systematic approach to incorporates the patient perspective throughout the drug development continuum
  - Translational
    - What symptoms or functions matter most to people with this disease?
    - How to best measure outcomes (frequency of assessments, mode of reporting, etc)?
  - Clinical studies
    - Do trial endpoints capture outcomes that matter to patients (including clinical outcomes assessments)?
    - Are the endpoints feasible?
    - Does the protocol facilitate enrollment and participation? How could it be improved?
  - Pre-market review
    - How can COA data be used in the risk:benefit assessment?
  - Post-market review
    - How best to convey information to facilitate informed clinician/patient/caregiver decision-making?

Adapted from slide by Paul Kluetz and Theresa Mullen, CDER

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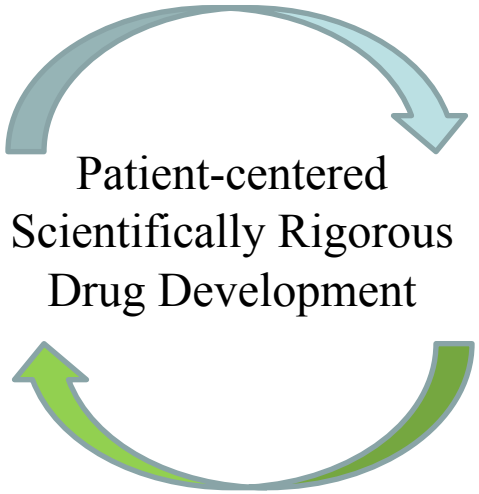
Successful Patient-Focused Drug Development Must be a Dialogue

Patients

- Experts in how they experience their disease
- Identify what matters most to patients
- Identify areas to make clinical trials more patient-friendly



Patient-centered Scientifically Rigorous Drug Development

Clinicians/ Trialists/ Health Policy Leaders

- Experts in clinical trial design and conduct
- Medical expertise
- Assess feasibility of trial modifications and outcome measures

slide from Paul Kluetz

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Patient-Focused Drug Development Program

- Part of FDA commitments under PDUFA V
  - FDA will conduct at least 20 meetings on specific disease areas FYs 2012 to 2017
  - Meetings help advance a systematic approach to gathering patients' input on their condition and treatment options
- The Program helps establish a therapeutic context
  - Patients are uniquely positioned to inform understanding of disease in broader context and scope
  - Patient representative input limited to specific marketing applications under review

http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm347317.htm

PDUFA = Prescription Drug User Fee Act

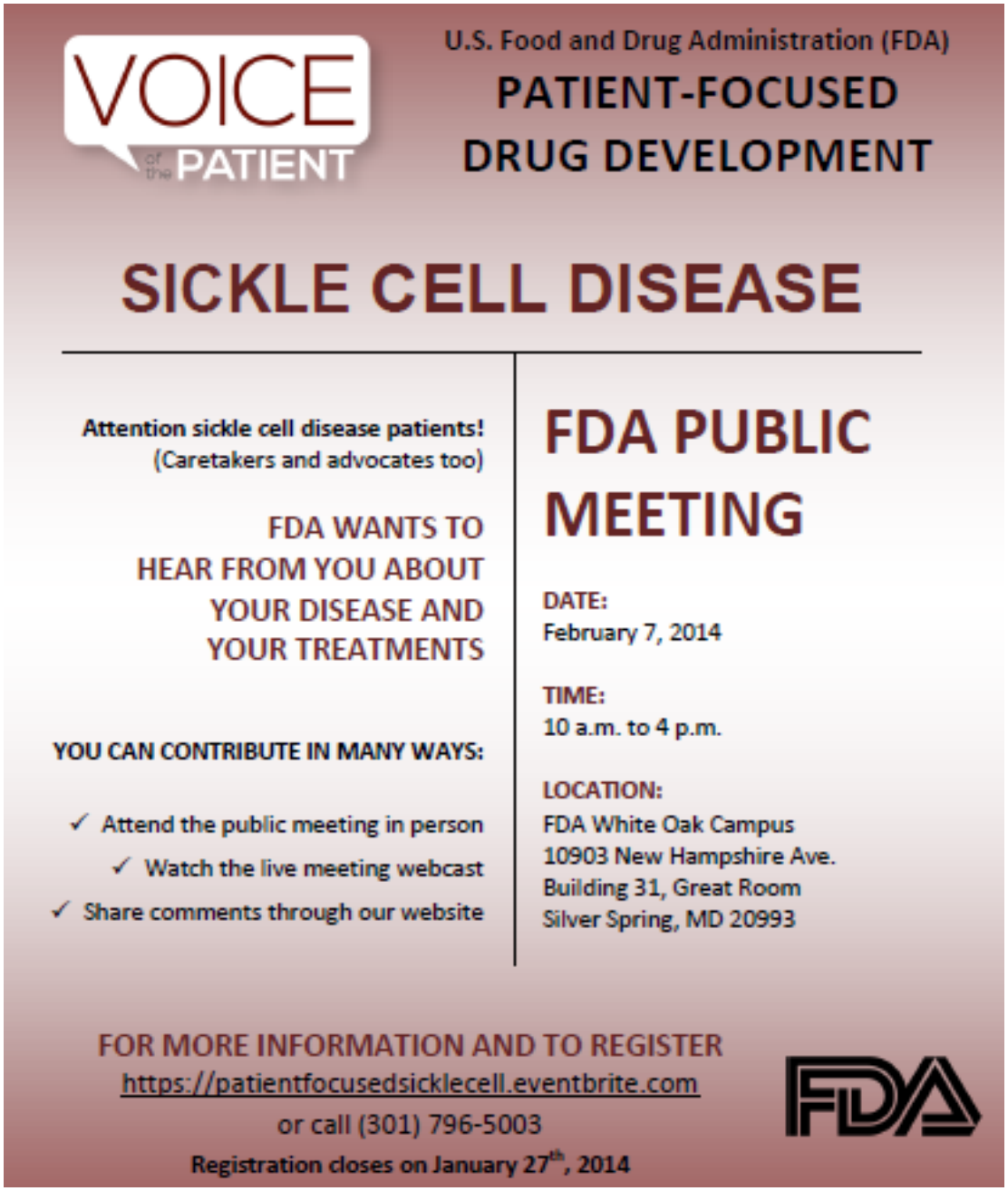
PFDD Meetings: Identifying Disease Areas

- Office of Strategic Programs worked with divisions in OND to identify candidate disease areas
  - Public input on these nominations was collected through a docket and at a public meeting held in October 2012
  - >4,500 comments were submitted, addressing 90+ disease areas

- Focused consideration on disease areas that:
  - Are chronic, symptomatic, affect functioning/daily activities
  - Currently have few or no therapies, or the available therapies do not directly affect how a patients feels or functions.
  - Have important aspects not formally captured in clinical trials
  - Have severe impact on identifiable subpopulations (e.g., children)
  - Represent a range in terms of size of the affected population

PFDD Meetings

- A ***Voice of the Patient*** report is prepared for each meeting
  - Includes an analysis of the condition and current treatment options
- Input can support FDA and industry:
  - Assessing benefit-risk for products under review
  - Advising sponsors on their drug development programs
- Input can support other aspects of drug development:
  - Help identify areas of unmet need
  - Develop clinical outcome tools (e.g., patient reported outcomes) that better address patient needs
- OHOP has participated in 3 PFDD meetings to date
  - Breast cancer (2015); Sickle cell disease (2014); Lung cancer (2013)


VOICE of the PATIENT
U.S. Food and Drug Administration (FDA)
PATIENT-FOCUSED DRUG DEVELOPMENT
SICKLE CELL DISEASE
Attention sickle cell disease patients!
(Caretakers and advocates too)
FDA WANTS TO HEAR FROM YOU ABOUT YOUR DISEASE AND YOUR TREATMENTS
YOU CAN CONTRIBUTE IN MANY WAYS:
✓ Attend the public meeting in person
✓ Watch the live meeting webcast
✓ Share comments through our website
FDA PUBLIC MEETING
DATE:
February 7, 2014
TIME:
10 a.m. to 4 p.m.
LOCATION:
FDA White Oak Campus
10903 New Hampshire Ave.
Building 31, Great Room
Silver Spring, MD 20993
FOR MORE INFORMATION AND TO REGISTER
https://patientfocusedsicklecell.eventbrite.com
or call (301) 796-5003
Registration closes on January 27th, 2014
FDA

Patient Engagement Cluster

- Initiated June 2016
- Discuss strategies for encouraging sponsors to collect information from patients
- Compare patient/caregiver input and process for including patient/caregiver input in regulatory decisions between agencies
- Share input with sponsors to create meaningful changes
- Develop strategies to measure and report impact of patient engagement in both agencies

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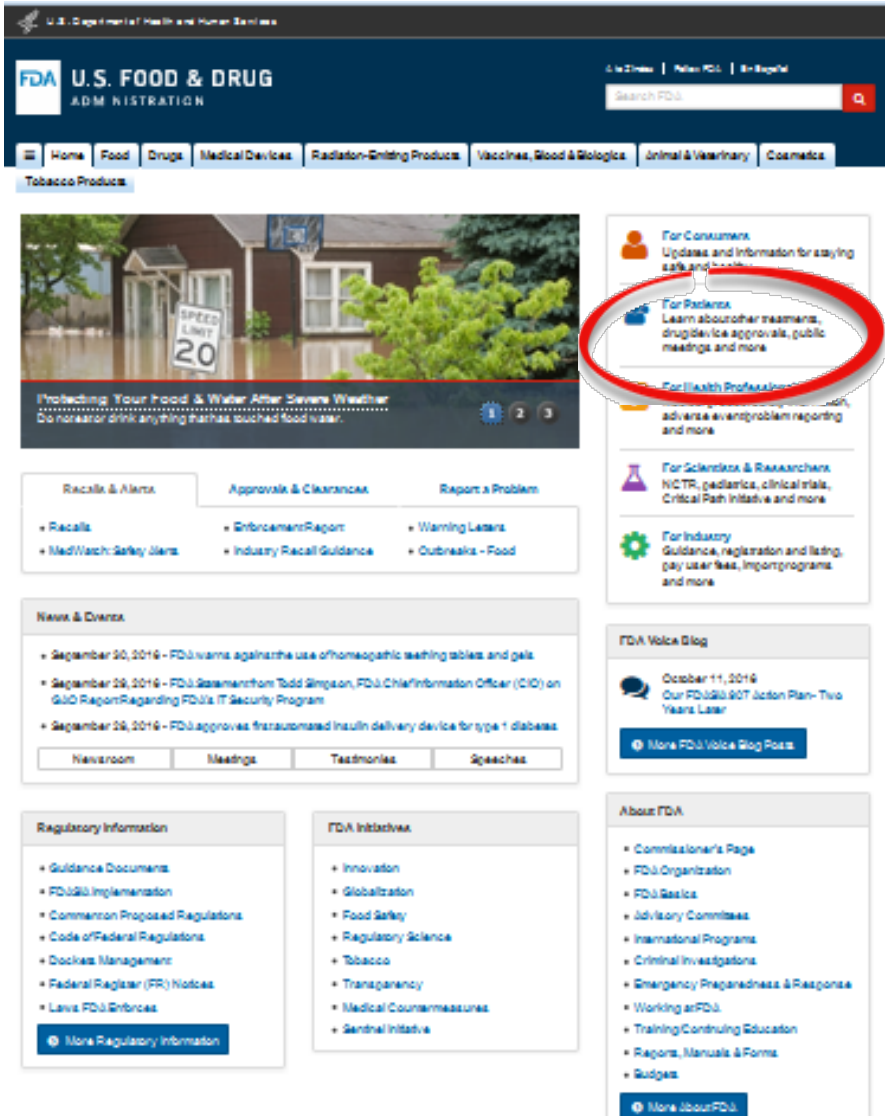
Individual patients, advocacy groups, and patient communities can take an active role



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Learn about and Participate in upcoming FDA Public Meetings

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Submit comments through the Federal Register

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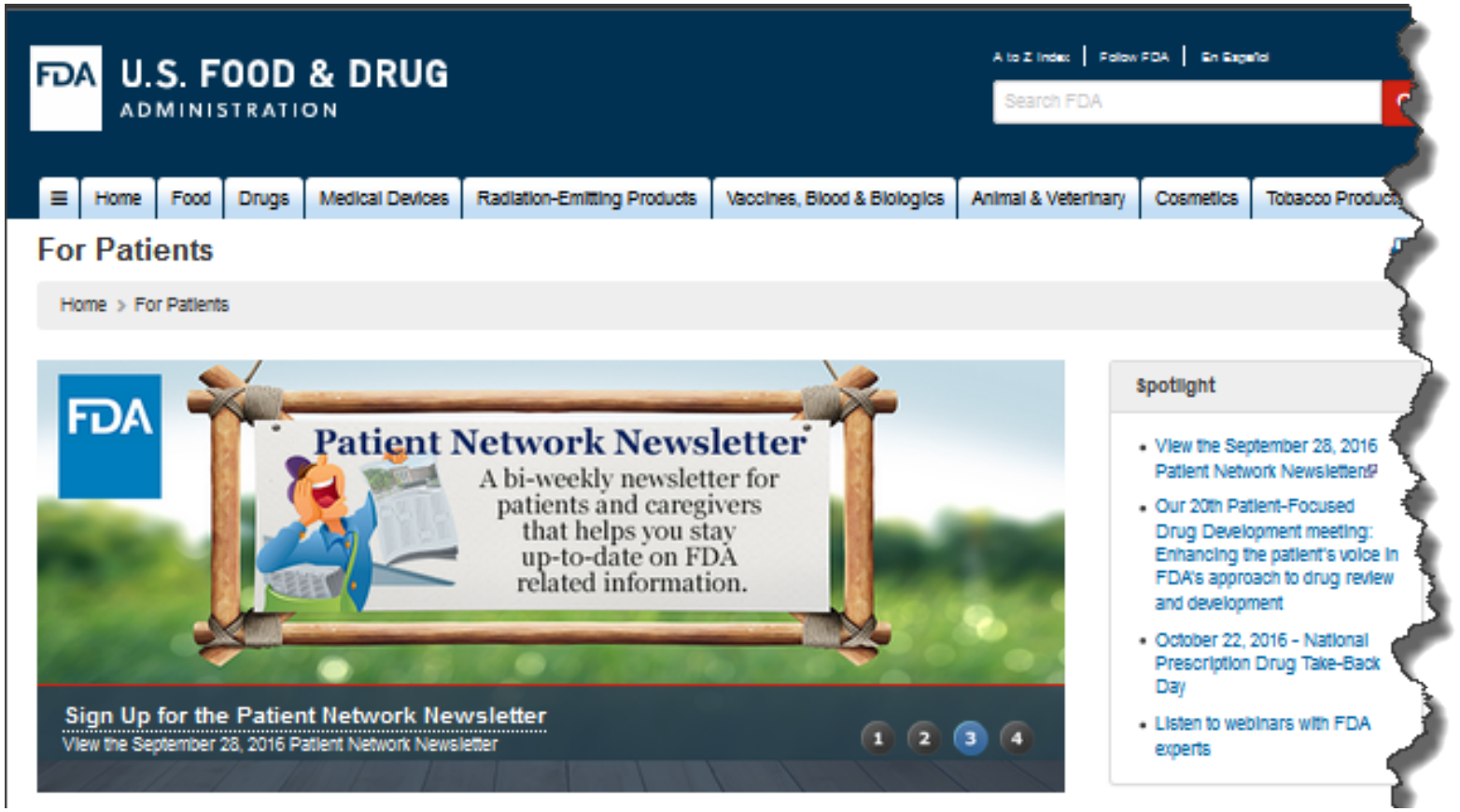

FDA continues to increase efforts to engage with patients

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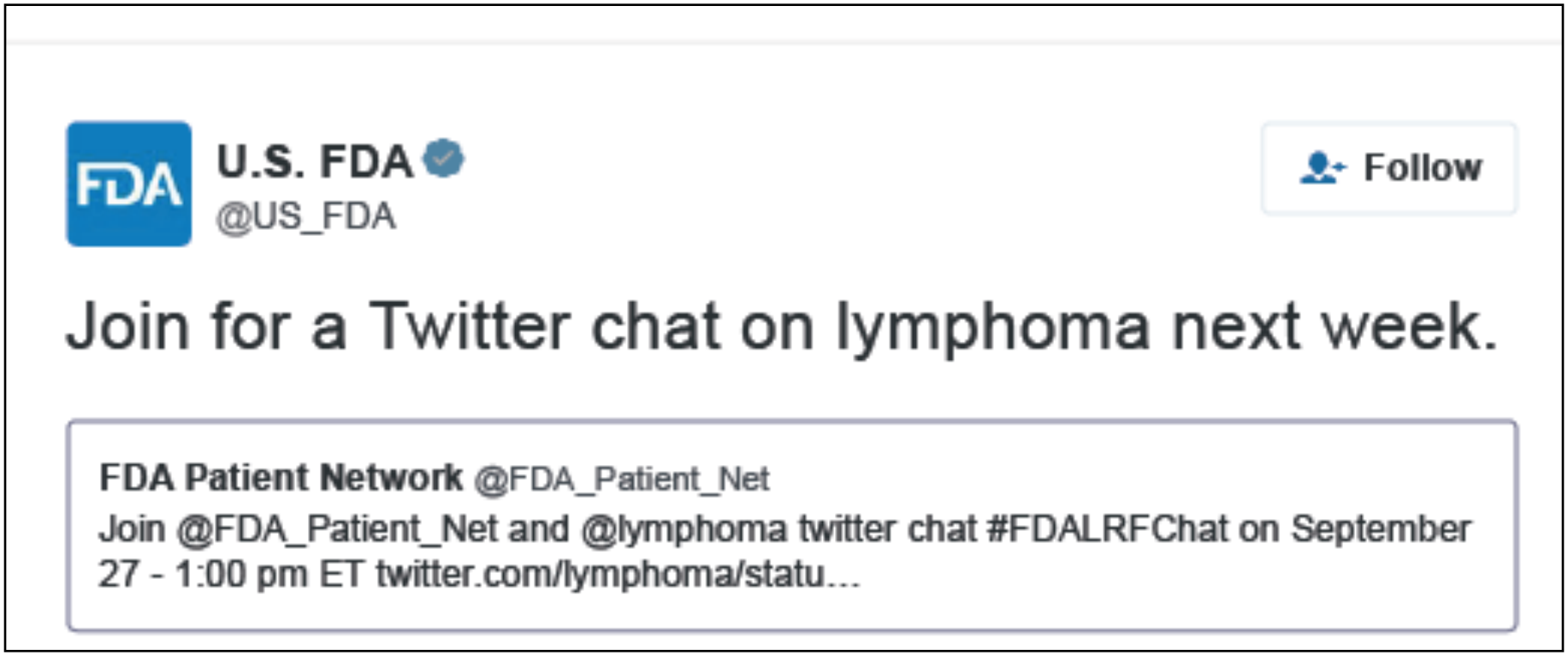
FDA Patient Network: Twitter

@FDA_Patient_Net



Meetings with Patient Advocates

Meetings between patients and advocacy organizations and various agency components to discuss issues of importance to patients

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Public Meeting: Childhood Cancer Advocacy Forum 2016

The FDA Offices of Hematology and Oncology Products, and Health and Constituent Affairs invite you to participate in a FDA Outreach to the Pediatric Cancer Advocacy

DATE:

Held on Friday April 22, 2016

TOPICS FOR DISCUSSION:

Update of cancer drugs approved for pediatric use, BPCA/WR study results which have informed product labeling, PREA and iPSPs for oncology drugs- impact on issuance of WRs, Expanding patient-focused drug development to children with cancer and Pediatric PROs, Expanded Access to investigational drugs, Expanding Eligibility Criteria for clinical trials to accommodate early evaluation of certain products in children, and promising new Vaccine and Engineered Cell Products for cancer.

PDUFA Reauthorization Performance Goals And Procedures FYs 2018 through 2022

"Enhancing Benefit-Risk Assessment in Regulatory Decision-Making

FDA will further the agency's implementation of structured benefit-risk assessment, ***including the incorporation of the patient's voice in drug development and decision-making****, in the human drug review program through the following commitments to be accomplished during PDUFA VI…"*

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Patient Engagement, OHOP Initiatives

- Peds cancer advocacy community public meetings 2014, 2016
- Lung cancer, breast cancer, sickle cell disease public meetings
- Pediatric subcommittee of the ODAC meetings followed by workshops on relevant topics
  - Developing anticoagulants for pediatric patients
  - PROs in oncology trials
  - Brainstem biopsies in patients with diffuse intrinsic pontine glioma
- Patient representative SGE consults requested on many applications
- Patient representatives are invited to relevant symposia conducted through OHOP

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PRO Assessment in Oncology

- Identify Core PRO Measures
  - Relevant to context, responsive to drug
- Identify/Validate Instruments
- Optimize Trial Design and Conduct
  - Increased engagement with FDA COA and Statistical reviewers
  - Providing consistent advice to sponsors
- Commitment to review PRO data as part of risk:benefit assessment in marketing applications
- Standardize Data Analysis and Presentation
- Identify Venues to Get the Information to Patients and Prescribers

Adapted from slide by Paul Kluetz

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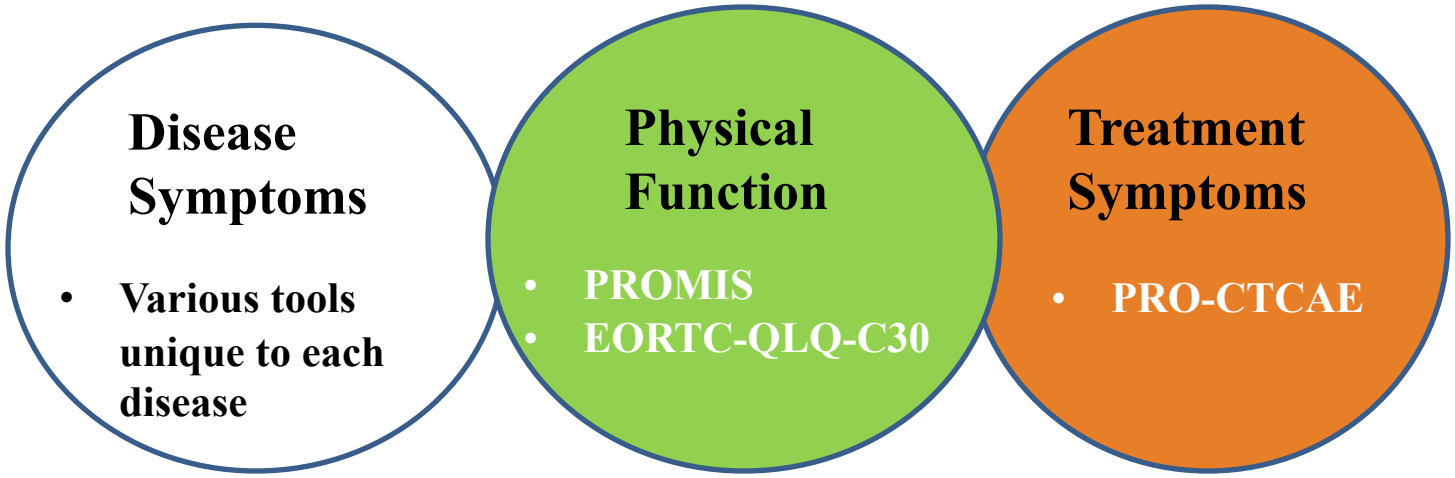
Core measures, tools and trial design & conduct



- Optimize the frequency and timing of assessments
- Include procedures for minimizing missing data
- Provide a pre-specified plan for the analysis of PRO data including the threshold for and interpretation of a meaningful change in score(s).
- Carefully record the use of concomitant medications that may affect the interpretation of the concept(s) being measured

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Challenges to Meaningful Patient Engagement

- Understanding of trial design
  (meaningful endpoints and data, measuring outcome, control arms)
- Understanding the regulatory framework, standards, and requirements (level of evidence)
- Legal and practical limitations facing sponsors
  (promotion v. education and engagement)
- Division within patient communities
- Different objectives or agendas among organizations
- Disagreement on meaningful measurement

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Conclusions

- FDA is committed to increasing patient engagement in the drug development process
- There are ongoing FDA efforts and new initiatives to engage patients in a meaningful way
  - Patient representative/SGE program
  - PFDD disease-specific meetings
  - Public meetings/workshops/joint projects
  - Careful use and interpretation of COA assessments
- Ultimate goal: facilitate development of medical products that meet patient needs and improve methods to provide information to patients and healthcare providers to optimize medical decision-making
- PFDD is an evolving, iterative process and much work needs to be done

Acknowledgements

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Thank you!

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