U.S. FOOD & DRUG ADMINISTRATION

2021 FDA Update

12th Annual PRO Consortium Workshop

April 14, 2021

Disclaimer

The views expressed in this presentation are those of the speaker, and do not necessarily represent an official FDA position

Agenda

- Introductory remarks
- Clinical Outcome Assessment (COA) Qualification Program and other FDA updates
- CDER Pilot Grant Program: Standard Core COAs and their Related Endpoints
- Panel discussion

Panelists and Speakers

- **Robyn Bent**, Director, Patient Focused Drug Development, CDER
- **Michelle Campbell**, Sr. Clinical Analyst for Stakeholder Engagement and Clinical Outcomes Office of Neuroscience, Office of New Drugs (OND), CDER
- **Laura Lee Johnson**, Director Division of Biometrics III, Office of Translational Sciences, CDER
- **Elektra Papadopoulos**, Acting Deputy Director Division of Clinical Outcome Assessment (DCOA), OND, CDER
- **David Reasner**, Director DCOA, OND, CDER

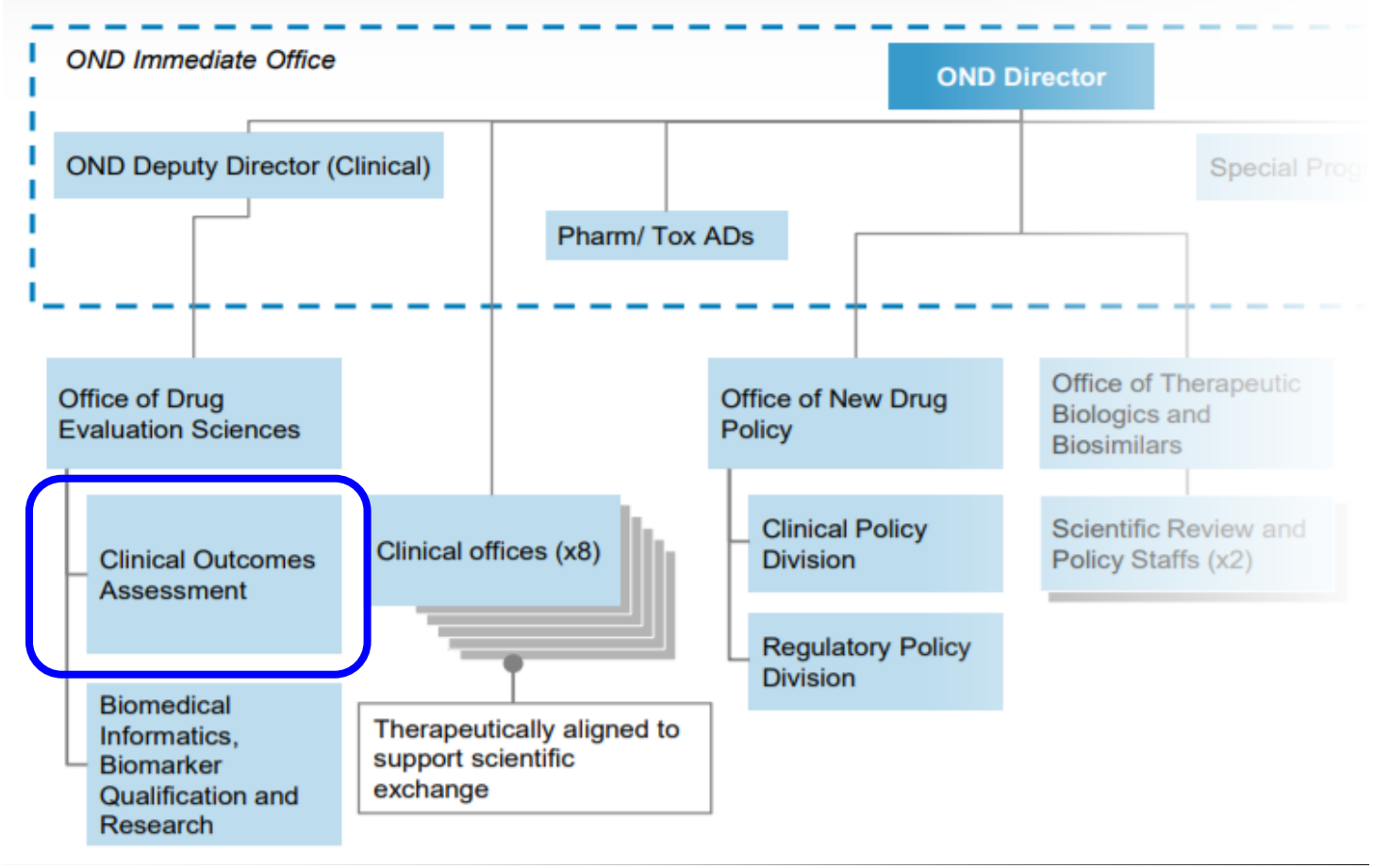
INTRODUCTORY REMARKS:

David Reasner

Division of Clinical Outcome Assessment (DCOA)

- DCOA Director
  - David Reasner, PhD
- DCOA Deputy Director (acting)
  - Elektra Papadopoulos, MD, MPH
- Team Leads
  - Selena Daniels, PharmD, MS
- Regulatory Project Managers
  - Kim Chiu, PharmD
  - Kristina Luong, PharmD
  - Lynne Ngouajio-Teguiafa, PharmD
- Project Specialist
  - Taccara Briggs
- Reviewers
  - Yasmin Choudhry, MD
  - Yujin Chung, PharmD
  - Robert Fieo, PhD
  - Onyekachukwu Illoh, OD, MPH
  - Julia Ju, PharmD, PhD
  - Naomi Knoble, PhD
  - Ji Li, PhD
  - Mira Patel, PhD
  - Susan Pretko, PharmD, MPH
  - Chris St.Clair, PharmD
  - Laura Swett, PhD
  - Qing Xie, PhD

OND Reorganization



COA QUALIFICATION PROGRAM, KEY COA-RELATED GUIDANCE AND OTHER UPDATES:

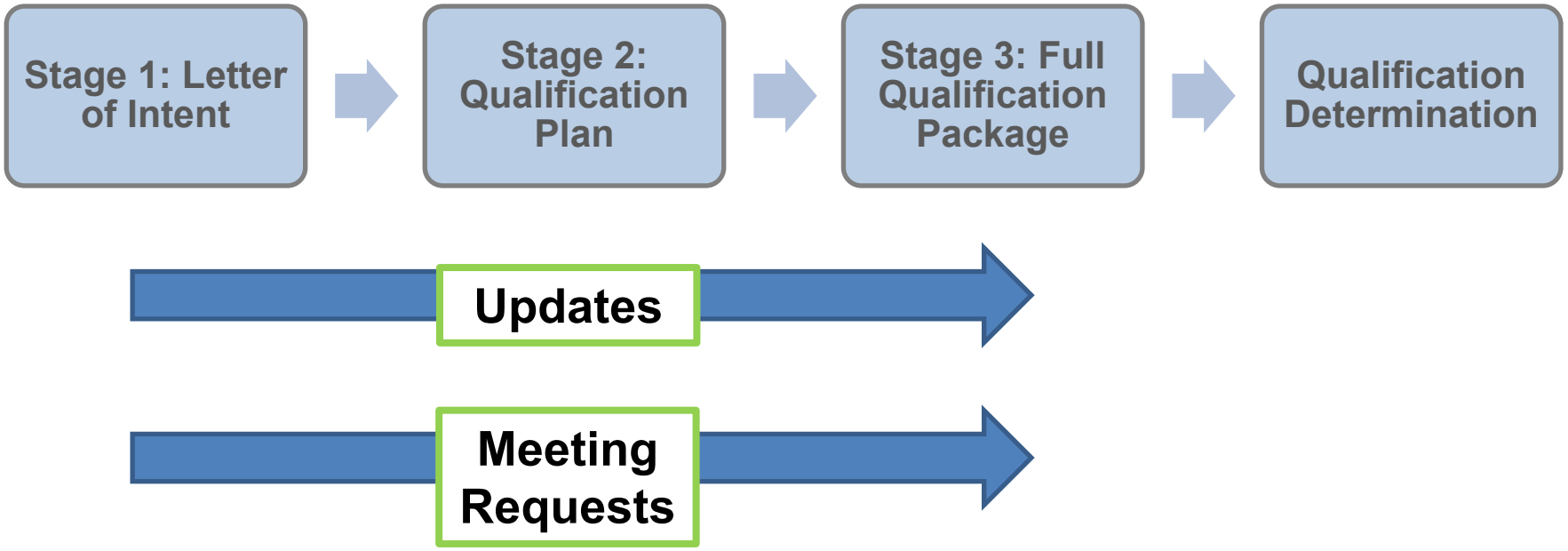
Elektra Papadopoulos

Drug Development Tool (DDT) Guidance

Qualification Process for Drug Development Tools

– Final Guidance published in Nov 2020

New DDT Process: Qualification Stages



Important: Each of the three milestone submissions should be a stand-alone package.

New DDT Process: Review Clocks

Qualification Stage	Review Clock
Letter of Intent (LOI)	3 months (calendar days)
Qualification Plan (QP)	6 months (calendar days)
Full Qualification Package (FQP)	10 months (calendar days)

CDER conducts a reviewability assessment.
Review clock begins when a reviewable memo issued.

QUALIFICATION PROGRAM METRICS

Qualification

- April 2020: Kansas City Cardiomyopathy Questionnaire-23 (KCCQ-23)
  - Total Symptom Score (TSS)
  - Physical Limitations Score (PLS)
  - Clinical Summary Score (composite of the TSS and PLS)

- Dec 2020: Diary for Irritable Bowel Syndrome Symptoms- Constipation (DIBSS-C)

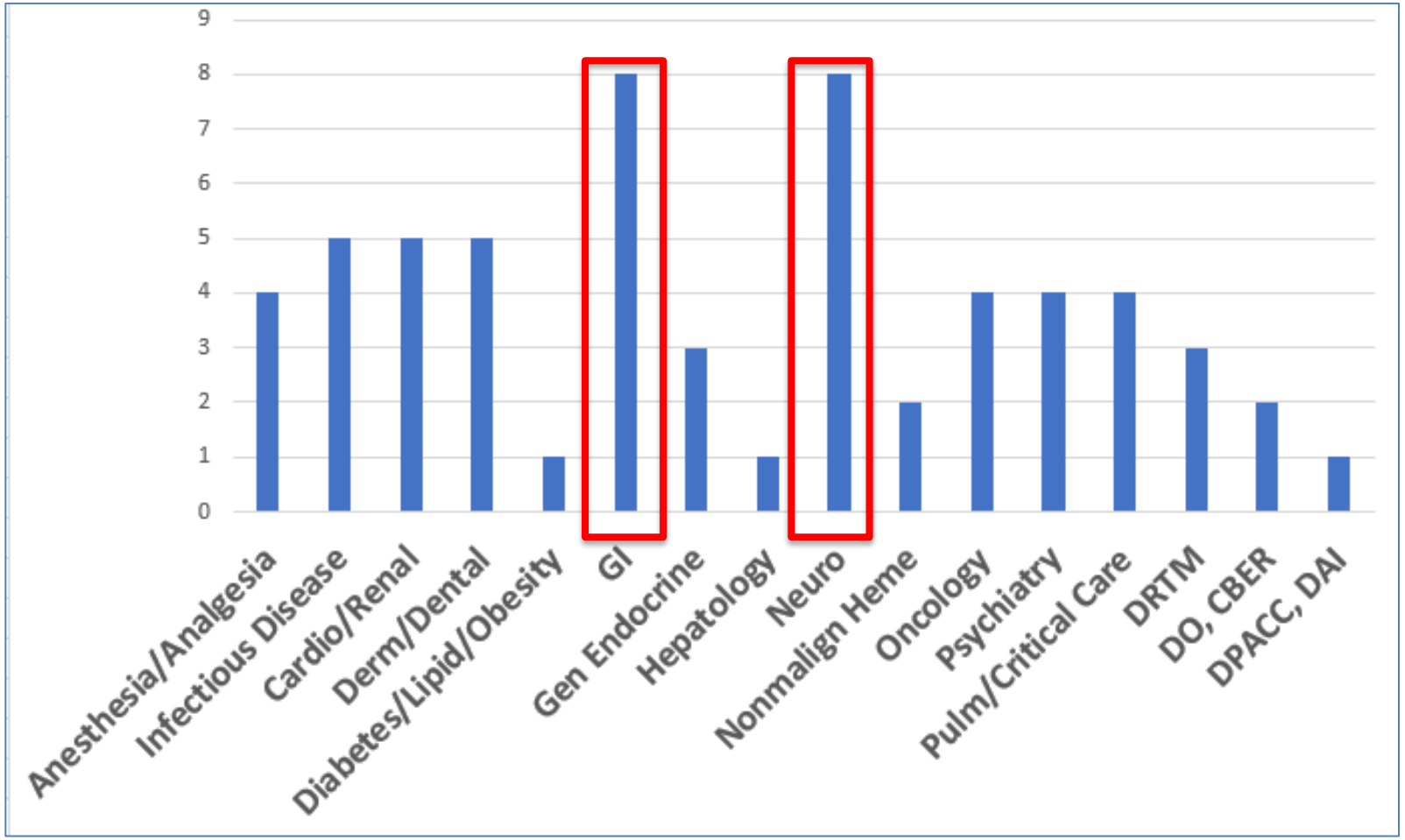
CONGRATULATIONS!

Number of Projects

- As of March 18, 2021, the total number of projects in the program totaled 60

- Accepted 11* LOIs between 1/1/20 – 3/18/21

Projects by Clinical Review Divisions



COA Types

DDT Type	Number
PRO Measures	37
Other*	8
PerfO Measures	7
ObsRO Measures	3
ClinRO Measures	4
PRO/ObsRO	1

*Digital Health Technologies (DHT)- not falling into other categories e.g. activity monitors

Number of 2020 Submissions

Type	Number (2018)	Number (2019)	Number (2020)
Letter of Intents (LOIs)	10	18	22
Qualification Plans (QPs)	2	8	15
Full Qualification Packages (FQPs)	2	0	2
Updates	13	9	9
Meeting Requests	7	5	10

COA DDT Research Grants Updates

- 6 COA DDT Research Grants awarded FY2020
  - For developers of DDTs with accepted Letter of Intent who are working towards to next qualification submission
- FY2021: **May 25, 2021** application due date
  - Funding opportunity announcement: PAR-21-178

Other COAQP Updates

Sign-Up! to the **COAQP Email Listserv** for timely updates and announcements such as:

- Newly qualified COAs
- Updates to existing COAQP resources (e.g., LOI outline edits, etc.)
- COAQP process changes (e.g., switching electronic submission portals)

https://public.govdelivery.com/accounts/USFDA/subscriber/new?topic_id=USFDA_531

GUIDANCE AND OTHER UPDATES

PFDD Guidance Series

- **Guidance 1:** Collecting Comprehensive and Representative Input
  - Public Workshop Dec 18, 2017
  - Final guidance June 2020
- **Guidance 2:** Methods to Identify What is Important to Patients
  - Public workshop Oct 15-16, 2018
  - Draft guidance Oct 2019

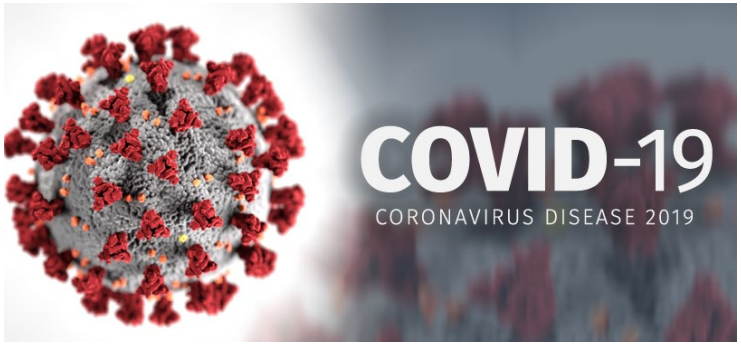
PFDD Guidance Series

- **Guidance 3:** Selecting, Developing or Modifying Fit-for-Purpose Clinical Outcome Assessments
  - Public Workshop Oct 15-16, 2018
  - Discussion document published
- **Guidance 4:** Incorporating Clinical Outcome Assessments into Endpoints for Regulatory Decision Making
  - Public Workshop Dec 6, 2019
  - Discussion document published

COVID-19 related guidance*

- FDA Guidance on Conduct of Clinical Trials of Medical Products During the COVID-19 Public Health Emergency
  - Q14. What factors should sponsors consider when deciding whether to change their clinical trial protocol during the COVID-19 public health emergency to include remote clinical outcome assessments?

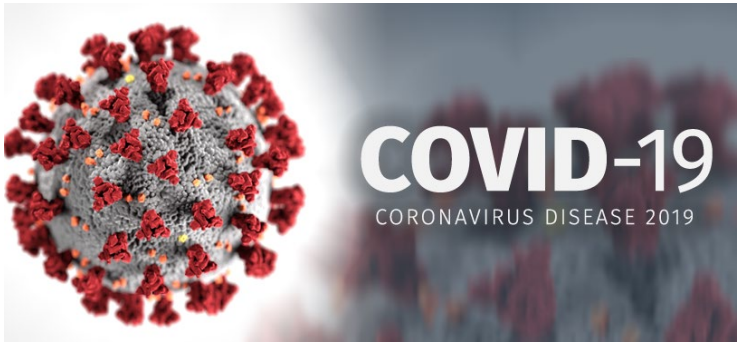
Check FDA guidance website for updates



COVID-19 related guidance*

- Assessing COVID-19-Related Symptoms in Outpatient Adult and Adolescent Subjects in Clinical Trials of Drugs and Biological Products for COVID-19 Prevention or Treatment

*Check FDA guidance website for updates



2nd COA Compendium Expansion

- **Coming soon!**
- Communication tool:
  - COAs that have been previously labeled and may serve as a starting point for consideration and discussion between drug developers and FDA
  - Qualified COAs: COA DDT Qualification Program

STANDARD CORE COAS AND THEIR RELATED ENDPOINTS PILOT GRANT PROGRAM:

Robyn Bent

Standard Core COA and Endpoints Pilot Grant Program

WHY

- Reviewers currently may see **multiple independent efforts**
- Duplication of effort and diversity of measures and proprietary tools that limit affordability and sustainability
- **Variable quality of tools and resulting data** that limit utility for regulatory decision making

GOAL

- Enable development of standard core sets of measures of disease burden and treatment burden for a given area—that will be made publicly available at nominal or no cost

UG3/UH3 Cooperative Agreement

involves 2 phases:

Milestone-driven planning phase **(UG3)** will provide funding for 1 to 2 years to conduct planning activities.

Implementation phase **(UH3)** will provide funding for 3 to 4 years to projects that successfully complete the planning activities and reach the projected milestones set in the UG3 phase.

Stakeholder Engagement

Public Meetings

- Regular public meetings with an opportunity for stakeholders to ask questions and provide feedback both as part of the meetings or by submitting feedback to the public docket

External Technical Advisory Committee

- Made up of disease specific experts, COA experts, biostatisticians, patient experts, and other technical experts as appropriate who oversee and monitor the specific projects

Scientific Policy Board

- To bring a global perspective to the Standard Core COA development process

Standard Core COA and Endpoints Pilot Grant Program

On September 11, 2019, the FDA made three awards:

Migraine Clinical Outcome Assessment System (MiCOAS)

Clinical Outcome Assessments for **Acute Pain** Therapeutics in Infants and Young Children (COA APTIC)

Northwestern University Clinical Outcome Assessment Team (NUCOAT) – **Physical Function**

Standard Core COA and Endpoints Pilot Grant Program

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https://vpgcentral.com/micoas/

Related Material

MiCOAS - Acute Lit Review Report

Acute Report Appendix

MiCOAS - Preventive Lit Review Report

Preventive Report Appendix

Northwestern University Clinical Outcome Assessment Team (NUCOAT) – **Physical Function**

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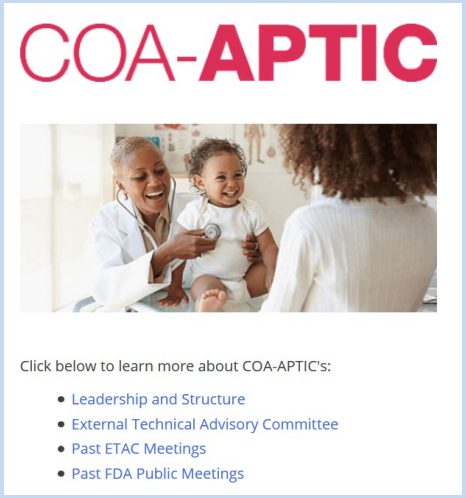

Standard Core COA and Endpoints Pil Grant Prograr

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https://sites.northwestern.edu/nucoat/

NUCOAT
Northwestern University Clinical Outcome Assessment Team

Planning Phase

NUCOAT's Planning Phase is a two-year milestone-driven planning phase to inform the development of a Physical Function Clinical Outcome Assessment that will capture physical function limitations across a wide variety of conditions.

Northwestern University Clinical Outcome Assessment Team (NUCOAT) – **Physical Function**

Share your thoughts

Federal Docket for Standard Core COA Grant Program

- https://www.regulations.gov/document/FDA-2020-N-1727-0003

Resources

- COVID-related guidance:
  - https://www.fda.gov/emergency-preparedness-and-response/coronavirus-disease-2019-covid-19/covid-19-related-guidance-documents-industry-fda-staff-and-other-stakeholders
- DDT Qualification process guidance:
  - https://www.fda.gov/regulatory-information/search-fda-guidance-documents/qualification-process-drug-development-tools-guidance-industry-and-fda-staff
- PFDD-related guidance series:
  - https://www.fda.gov/drugs/development-approval-process-drugs/fda-patient-focused-drug-development-guidance-series-enhancing-incorporation-patients-voice-medical
- CDER Pilot Grant Program: Standard Core COAs and Related Endpoints
  - https://www.fda.gov/drugs/development-approval-process-drugs/cder-pilot-grant-program-standard-core-clinical-outcome-assessments-coas-and-their-related-endpoints

ARE YOU INTERESTED IN PATIENT-CENTERED OUTCOMES?

Consider a rewarding career at the FDA!

FDA's Office of New Drugs (OND) in the Center for Drug Evaluation and Research (CDER), located in Silver Spring, MD, is recruiting **outcomes researchers, psychometricians, survey methodologists/instrument developers, epidemiologists, patient-preference researchers and behavioral/social scientists (including psychology, cognitive science and neuroscience)** to serve as experts in the evaluation and use of clinical outcome assessments (including use of innovative platforms for data collection) to measure health outcomes in the dynamic, challenging, and innovative atmosphere of drug development and research.

Selected candidates will join the **Division of Clinical Outcome Assessment (DCOA)** where they will contribute to a culture that ensures the patient voice is integrated into medical product development through incorporation of clinical trial endpoints that are meaningful to patients, valid, reliable, and responsive to treatment. DCOA Staff members provide advice and consultation on endpoint development, instrument development, validation, and interpretation for use in drug development.

Team members also participate in guidance and policy development to ensure that meaningful drug product information is available to health care providers, caregivers, and patients.

Team members collaborate with diverse stakeholders both internally and externally across a variety of therapeutic areas including representatives from academia, medical product industry, consortia, instrument developers, patient groups, regulators and other government agencies to advance the science of clinical outcome assessment and enhance the development and implementation of appropriate assessment tools in drug development.

SALARY & BENEFITS: Salary is commensurate with education and experience. We offer an excellent benefits package (health insurance, life insurance, thrift savings plan, retirement) and many opportunities to continue professional development.

REQUIRED SKILLS:

- Relevant doctoral degree (health services or health outcomes research, educational research, public health, pharmacy, medicine, osteopathy)
- At least one year of instrument development or survey methodology experience (qualitative and/or quantitative methods) preferred
- Ability to adapt to a changing environment and handle multiple priorities
- Excellent writing, critical thinking and analytical skills

INTERESTED CANDIDATES: Please submit your Curriculum Vitae to DCOA@fda.hhs.gov. Additional information on DOCA may be found at: www.fda.gov/coa.

Q & A

U.S. FOOD & DRUG ADMINISTRATION

THANK YOU!