How Patients Can Engage with Food and Drug Administration (FDA):

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Director for the Office of Orphan Products Development, FDA

Prescription Drug User Fee Act (PDUFA):

Eric Gascho
Vice President of Government Affairs, National Health Council (NHC)

Patient Focused Drug Development:

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Senior Vice President, CRD Associates

How Patients Can Engage with National Institutes of Health (NIH):

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Director of the Office of Rare Diseases Research and Division of Clinical Innovation, National Center for Advancing Translational Sciences, NIH
The Promise of Agency Partnership

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How Patients Can Engage With the Food and Drug Administration (FDA)

Rare Disease Week on Capitol Hill: 2016 Legislative Conference

Gayatri R. Rao, M.D., J.D.
Director, Office of Orphan Products Development
U.S. Food and Drug Administration
March 1, 2016
Patients can engage with FDA in many ways...

- Request meetings with FDA on a variety of issues
- Participate in the advisory committee process
- Participate in public meetings, workshops, and webinars
- Provide comments to proposed rules and guidances
- Apply for natural history grant funding
Request to meet with FDA

• When do patients request to meet with FDA?
  - For questions about FDA and its processes – particularly if they can’t find those answers on FDA’s website
    • Main FDA webpage: www.fda.gov/
    • Patient-specific webpage: www.fda.gov/forpatients
  
  - If a patient organization is engaged in conducting research (e.g., natural history studies) and they are seeking input
  
  - Occasionally to discuss a specific product under review
    • Note, however, if the sponsor (company) is not part of those discussions, FDA will be limited in what it can say
Request to meet with FDA

• **Who to contact?**
  - Office of Health and Constituent Affairs (OHCA), Patient Liaison Team:
    patientnetwork@fda.hhs.gov
  - Office of Orphan Products Development (OOPD):
    - [www.fda.gov/orphan](http://www.fda.gov/orphan)
    - [orphan@fda.hhs.gov](mailto:orphan@fda.hhs.gov)
  - Center for Drug Evaluation and Research (CDER)
    - CDER Rare Diseases Program:
      [http://www.fda.gov/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDE R/ucm221864.htm](http://www.fda.gov/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDE R/ucm221864.htm)
    - CDER Professional Affairs and Stakeholder Engagement (PASE): cderpase@fda.hhs.gov
Advisory Committee Process

• What are Advisory Committees (AC)?

- Committees of external, independent experts to advise FDA on a variety of issues (e.g., product approvals)

- Experts include clinicians, academicians, consumer reps, industry reps, patients and/or care givers

- All meetings include an open public hearing
Advisory Committee Process

• How can you participate in the AC process?
  - Request to provide oral comments during the open public hearing session
  - Provide a written submission
  - Serve as a patient representative on an AC
  - For info about ACs, meeting schedule, meeting materials, etc.: http://www.fda.gov/advisorycommittees/default.htm

• What is a Patient Representative and how can you become one?
  - Patient reps serve as temporary voting members on an AC and can also consult directly with the scientific review staff and sponsor
  - Go through an application process (with a conflict of interest review) and training process
  - http://www.fda.gov/ForPatients/About/ucm412709.htm
Public Meetings, Workshops, Webinars

• FDA routinely conducts public meetings, workshops, and webinars on a broad range of issues intended to receive input from and/or educate the public, e.g.,
  – Mar. 16 - Public Meeting on Patient-Focused Drug Development for Psoriasis

• How can I learn about these meetings?
  – Patient-specific webpage: http://www.fda.gov/forpatients/
  – General announcement in the Federal Register:
  • Go to “Today’s Federal Register” and scroll for “Food and Drug Administration”
Proposed Rules & Guidance Documents

• FDA publishes proposed rules and guidance documents implementing laws, describing policies and procedures, and providing recommendations on a wide range of regulatory and scientific issues, e.g.,
  – FDA Draft Guidance – Rare Diseases: Common Issues in Drug Development (issued August 2015)
  – FDA Draft Guidance – Rare Pediatric Disease Priority Review Vouchers (issued November 2014)
Proposed Rules & Guidance Documents

• Patients can provide written comments to proposed rules and guidance documents
  – *For a proposed rule*: Generally must submit comments either 60 or 90 days from date of issuance
  – *For a guidance document*: Can submit comments at any time but to ensure that comments on a draft guidance are considered before work on a final guidance commences, submit comments before the close date

• **How do I know when proposed rules or guidances are issued for which I can comment?**
  – Patient-specific webpage: [http://www.fda.gov/ForPatients/CommentonGuidance/default.htm](http://www.fda.gov/ForPatients/CommentonGuidance/default.htm)
    • Go to “Today’s Federal Register” and scroll for “Food and Drug Administration”
  – Search under [www.regulations.gov](http://www.regulations.gov)
Apply for natural history grant funding!

- FDA’s OOPD just launched the Orphan Products Natural History Grants Program (www.fda.gov/orphan)
  - Fund ~$2M to award 2-4 grants in FY2017
  - Patient advocacy groups are eligible to apply
  - Important dates:
    - Request for applications (RFA) – March 2016
    - Receipt date for Applications – October 14, 2016
    - Anticipated earliest award date – March 2017

- FDA’s video discussion on natural history studies featuring patient advocate perspectives: https://www.youtube.com/watch?v=2GDU7f75MVI
Questions?

Office of Orphan Products Development

www.fda.gov/orphan

orphan@fda.gov

301-796-8660
The Promise of Agency Partnership

Prescription Drug User Fee Act (PDUFA):

Eric Gascho
Vice President of Government Affairs, National Health Council (NHC)
The Promise of Agency Partnership

PDUFA

Eric Gascho
Vice President, Government Affairs

@EricGascho
The Birth of PDUFA – The Power of Patients

Back to Basics: HIV/AIDS Advocacy as a Model for Catalyzing Change
Evolution

Initial patient group engagement

Shift to consumer engagement

Patient groups re-engage

1993
PDUFA I: Backlog reduction

1998
PDUFA II: FDAMA Reduce review times

2003
PDUFA III: BTP

2008
PDUFA IV: FDAAA
- Unintended consequences
- Process for engagement

2013
PDUFA V: FDASIA
- Benefit/Risk framework
- Biomarkers/PROs
- PFDD
- Rare diseases

2018
PDUFA VI
Patient engagement throughout the development lifecycle
PDUFA V Success

- Development of an objective, qualitative benefit-risk framework that includes robust patient input
- Expanded use of biomarkers, patient reported outcomes in clinical trials, and companion diagnostics
- Increased resources for Rare Disease Program
- Patient Focused Drug Development Program
PDUFA VI Asks

- Adopt FDA guidance to integrate patient voice into drug development and approval
- Increase resources for qualifying biomarkers and patient-reported outcomes
- Pilot adaptive clinical designs
- Minimize barriers to FDA hiring
- Clarify oversight of combination products that have cross-center jurisdiction
What’s next?
Eric Gascho  
Vice President, Government Affairs  
National Health Council  
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@EricGascho
The Promise of Agency Partnership

Patient Focused Drug Development:

Johanna Gray
Senior Vice President, CRD Associates
FDA’S PATIENT-FOCUSED DRUG DEVELOPMENT: AN ALPHA-1 CASE STUDY

Community Engagement/Public Policy/Grassroots

Johanna Gray
Senior Vice President, CRD Associates
Federal Policy Advisor, Alpha-1 Foundation
My Experience with PFDD Meetings

- I have worked with three patient advocacy group clients to prepare for and participate in the process for PFDD meetings:
  - Hemophilia A, Hemophilia B, von Willebrand disease, and other heritable bleeding disorders – Sept 2014
  - Breast cancer – April 2015
  - Alpha-1 Antitrypsin Deficiency – Sept 2015
The Goal – Make the Alpha-1 Voice Heard

• Fill every open space
  • 300 in room
    • We registered over 310
  • 1000 online spots
    • Over 600 registered

• Develop survey for patients too sick or unable to travel
  • Goal of 500
    • 1655 responded

• Follow-on Activities
  • Statistical analysis and summary report of survey
  • Thank you letters and request to submit comments to CBER
  • Submit policy ask from A1F Leadership
How?

• Prioritization
• Preparation
• Communication
• Include the entire community
• Collaboration
• Follow-up
Prioritization

- Resources diverted to PFDD activities
- Full Network Support – Strategic Alliances
- A1F Programs Utilized
  - Research Registry – letter to liver affected
  - Clinical Resource Centers
  - Support Groups
  - AlphaNet Coordinators
- Made financial commitment
  - Patient safety – oxygen provider locked in
  - Committed to foster attendance
- Logistics: Planes, Trains and Automobiles!
Preparation

• We have a “readied” community and network

• Continuous education and engagement
  • Patients are knowledgeable
  • Set up for success with “How to tell my story” training

• Had a point person to answer questions

• Leadership on hand at the venue (A1F and Public Policy Partners)
Communication

- Recruitment buckets – panel, participants, liver, lung, Alpha-1 parents/children
- Phone calls
- In field events
- Social Media Campaign
- Video Messages from Leadership
- Registration/Information page on website
- FDA PFDD Recruitment banner on www.alpha1.org
- Splash Page on PFDD took over our website
  - Countdown ticker
  - Easy links for in-person and webinar registration
  - Link to survey
Communication Examples

Splash Page with Countdown
(High jacked our Site)

Leadership Ask

Action Center/FAQs

Frequently Asked Questions

- When and where will the FDA public meeting take place?
- How do I sign up to attend the meeting in person?
- How do I sign up to watch the live webcast?
- I registered to attend in person. Is there travel assistance?
- Can I submit a question to the panel if I am watching online?
Inclusion

• It was vital to let the agency know that many patients were unable to travel due to the impact of Alpha-1
  • Illness
  • Disability
  • Financial

• Survey Monkey survey created so more voices could be heard
  • Based on the FDA's PFDD questions
  • Broke out paths for patient audiences
    • Alpha-1 Kids
    • Liver
    • Lung
    • Liver and Lung
Collaboration

• Continuous Collaboration with the FDA
  • Logistics
  • Timing
  • Security
  • Bringing Oxygen in
  • Rest Areas
  • Meals/water
  • Distance to room

• Developed messages for FDA to distribute to registrants
  • Hotel and travel stipend availability
  • A1F contact info if registrant required medical support

• Update on Survey Participation
  • Resulted in FDA’s approval to present data and third topic added to the agenda on clinical trial design
The Meeting

• Full room (213)
  • Attendees in both overflow rooms
  • Varying reports of online participation: 600 vs. 273
• Two incredible panels with facilitated Q&A–
  • Effects of Alpha-1 that matter most: John Walsh, Henry Moehring, Charlotte Mattison, Richard Johnson, Jim Quill, Roger Alquist
  • Perceptions of current treatment: Fred Walsh, Jesse Young, Ken Richmond, Jean McCathern, Marcie Heitzman, Karen Erickson
• Third topic added – Clinical trial design
• Opportunity to report survey data on each topic: Liz Johnson, Gordon Cadwgan, John Walsh
• Audience Participation all day
• Public comments (15) - 2 minutes each
• Dr. Sandhaus concluding remarks from Foundation
Who participated?

- PATIENTS!!!
- FDA: Center for Biologics Evaluation and Research; Center for Drugs Evaluation and Research including Office of Rare Diseases
- Industry partners
- Research partners including academic institutions
- Other government agencies (NIH, etc.)
- Other Foundation partners:
  - COPD Foundation; NORD; ViiMED; global Alpha-1 partners;
Message Heard Loud and Clear

• We are compliant
• We are engaged
• We manage our disease to the best of our ability
• Lung-affected: we are grateful for our therapy, but are ready for advances
• Liver-affected: support network is the best therapy they have
• We will step up for trials, as long as therapy is not interrupted
• We need Alpha-1 cohorts in all Lung/Liver trials
Follow-up

- Thank you letters to Agency members

- Submission of Survey Results
  - Summary Report
  - Statistical Analysis (for each target audience)
  - IMPORTANT - Added Patient Comments

- Prepared and Submitted A1F Leadership Policy Requests
Result thus far…

• Meeting held with FDA/CDER on Liver Studies and Trial Design

• Discussion Topics:
  • Prevalence of alpha-1 antitrypsin deficiency and liver disease in children and adults
  • Features and natural course of liver disease in children and adults
  • Patient recruitment for interventional clinical trials
  • Outcomes and duration of studies

• Recommended use of Alpha-1 as a Model of Rare Disease
The Message Was Heard.
The Promise of Agency Partnership

How Patients Can Engage with National Institutes of Health (NIH):

Petra Kaufmann, M.D., M.Sc.
Director of the Office of Rare Diseases Research and Division of Clinical Innovation, National Center for Advancing Translational Sciences, NIH
How Patients Can Engage with the National Institutes of Health

PETRA KAUFMANN, M.D., M.Sc.
OFFICE OF RARE DISEASES RESEARCH
DIVISION OF CLINICAL INNOVATION

RARE DISEASE WEEK ON CAPITOL HILL
MARCH 1, 2016
National Institutes of Health

• **27** different Institutes and Centers (ICs), **24** of which award grants

• Each one has:
  • Different missions
  • Different funding priorities
  • Different budgets
  • Different types of grants they support
  • Different procedures for making funding decisions
  • Different funding strategies
NIH grant cycle
(9 months)

PI develops research proposal

Institution/Organization submits the application

Plan

NIH Institute Staff

Council

Institute Director

Funding Decision

Fund

Peer Review

• Score
• Summary Statement

Division of Receipt and Referral (CSR*) assigns applications to:

• Review group
• NIH Institute

Award made!
### Search Results

**Matching Records:** 693

**Show:** Active Only

**Release Date:** August 31, 2014

**Include Notices:** Yes • No

**Include Expired:** Yes • No

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Peer review

*What* is being proposed?

*Why* is it important?

*Can* the applicants do it?

Looking at

- Overall impact
- “Core” Criteria:
  - Significance
  - Investigators
  - Innovation
  - Approach
  - Environment
- Additional issues (e.g., Human Subjects Protection)
What determines …

If awards are made?

- Scientific merit (Peer review score)
- Program considerations
- Availability of funds

Paylines?

- Congressional appropriation
- Commitment base vs. competing dollars
- Administrative cuts to grants
- Funds reserved for high priority areas
Advisory Council

- Serves as second (broader) level of grant review
- Performs concept clearance of proposed solicitations
- Advises the institute on overall goals and priorities
- 12-18 members:
  - 1/3 basic scientists
  - 1/3 medical scientists
  - 1/3 public members
- Balanced for ethnic, racial, gender, disability, geographic representation
NIH grant cycle
(9 months)

Plan:

- PI develops research proposal
- Institution/Organization submits the application

Review:

- Division of Receipt and Referral (CSR*) assigns applications to:
  - Review group
  - NIH Institute

Peer Review:

- Score
- Summary Statement

Funding Decision:

- Institute Staff
- Council
- Institute Director
- Funding Decision

Award made!
CSR is developing a registry of experienced senior scientists who would make good reviewers based on recommendations from scientific societies and institutions. We hope this registry will be a key tool for Scientific Review Officers to (1) more quickly identify experienced, volunteer reviewers and (2) provide societies and institutions with additional input into the peer review process.

To date, **155 scientific organizations** have identified over 5,000 volunteer reviewers. CSR thanks all of these societies for helping us recruit highly qualified reviewers.

CSR is looking for potential reviewers who—

- Have substantial and broad independent research experience
- Have received major peer-reviewed grants either from NIH or an equivalent agency
- Understand the review process
- Are willing to consider serving for four years as study section members

**How Scientific Societies and Institutions Can Help**

We are asking societies, scientific groups and academic institutions to help us identify qualified volunteers by recommending experts in the field who indicate a willingness to serve.

Society representatives interested in participating are asked to contact CSR by sending an e-mail to **RecruitReviewers@csr.nih.gov**. CSR will provide the appropriate form for the societies to distribute to their members to complete.

**How You Can Help**

If you are interested in serving, contact your professional society to inform it of your interest. If your society has not yet participated, please encourage its leaders to contact CSR.
NCATS Office of Rare Diseases Research

Rare Diseases Clinical Research Network (RDCRN) Program
- 22 consortia at 250 institutions worldwide
- Studying 282 rare diseases with more than 90 active protocols
- More than 130 patient advocacy groups participating

Genetic and Rare Diseases (GARD) Information Center Program
- Provides up-to-date information to patients, families, researchers and the public about rare or genetic diseases.
- Information provided by information specialists and on the NCATS/GARD website https://rarediseases.info.nih.gov

Global Rare Diseases Patient Registry Data Repository/GRDR® Program
- Will provide a “one-stop shop” for rare diseases data from registries in the USA and elsewhere
- Will provide ability to conduct cross-disease analysis and recruitment

NCATS Scientific Conferences Program
- ORDR manages a committee to identify scientific opportunities for rare and common diseases and evaluate applications
NCATS Genetic and Rare Diseases Information Center (GARD)

https://rarediseases.info.nih.gov

Online resource with:

- Up-to-date, reliable and easy-to-understand information on rare or genetic diseases
- In English or Spanish
- For people with rare or genetic diseases, their families, friends, care providers and wider communities
- Contact information for telephone and email queries
NIH/NCATS Global Rare Diseases Patient Registry Data Repository (GRDR®) Program

Develop a web-based resource to aggregate, secure & store de-identified patient information from multiple sources.

Features:
- Common Data Elements (CDEs) for collecting data
- Informed consent templates
- Central IRB services
- Access to GRDR Global Unique Identifier (GUID)
- Map patient data to GRDR CDEs & national standards
- Information and tools
NCATS Toolkit Project

- Create online “toolkit” for patient groups
- Promote effective engagement along the therapy development process
- Offer workshops so that patient groups can exchange best practices and strategies
- Bring community together to share resources and identify gaps
Take-home message

- Patient groups can partner with NIH by
  - Providing information
  - Applying for funding
  - Partnering with applicants
  - Serve as peer reviewers or Council members
- Patient groups can partner with ORDR
  - Rare Disease Clinical Research Networks
  - Promoting data standards for registries
  - GARD
  - Conference programs
  - Toolkit initiative
Learn more about NCATS

Website: www.ncats.nih.gov

facebook: facebook.com/ncats.nih.gov

Twitter: twitter.com/ncats_nih_gov

YouTube: youtube.com/user/ncatsmedia


Email us! info@ncats.nih.gov