



National Kidney Foundation®



Participating in the Upcoming Externally-Led Patient-Focused Drug Development Meeting with FDA on APOL1 Kidney Disease

Speaker:

James Valentine, JD, MHS



An EL-PFDD meeting in partnership with:





Overview of Today's Webinar

- Background on EL-PFDD meetings
- Background on Food and Drug Administration (FDA)
 - FDA's role in drug development
- Introduction to Patient-Focused Drug Development
- Participating in the EL-PFDD Meeting
- Other Important Information



Background on EL-PFDD Meetings



Purpose of the EL-PFDD Meeting on APOL1 Kidney Disease

To inform the FDA, Pharma, and other interested parties:

- What it is like to live with APOL1 kidney disease
- Your concerns as the disease progresses
- Your perspectives on clinical trials for APOL1 kidney disease
- How you currently manage APOL1 kidney disease
- What meaningful new treatments would look like



Why Should I Participate?

- Opportunity for *Your Voices* to be heard
- The FDA will approve all treatments for APOL1 kidney disease and needs to know what is important to *you*
- Your input can impact FDA's *decision-making*, and lead to *better treatments* and potentially *faster approvals* for APOL1 kidney disease treatments
- This EL-PFDD meeting is your chance to **talk *directly* to the FDA**



Why Have an EL-PFDD Meeting Now?

- The **Patient Voice** has historically been absent from the drug development process
- **In the past, patient input has been solicited only when ...**
 - A drug was already approved by the FDA (sometimes even with endpoints that are not meaningful for patients), or
 - A clinical trial was failing and the drug company or FDA wanted input from patients to understand the problem
- Your voices should be heard at the *beginning* of the process and *throughout*
- Patient participation in drug development = FDA Priority
 - **So, FDA initiated a *Patient Focused Drug Development Program* to learn from people living with a disease and their care partners about the impact of disease on their lives.**



Meeting Logistics-1

☐ Who?

- All people living with APOL1 kidney disease and their care partners

☐ When?

- November 6, 2026, from 10am – 3:30pm

☐ Where?

- College Park Marriott Hotel and Conference Center, Hyattsville, MD and
- Virtual



Meeting Logistics-2

- Most of meeting is about hearing from patients and care partners
 - Only patients, care partners, and moderator speak during audience discussion
 - Everyone else is in “listening mode” *because ...*
 - FDA wants to hear experiences from only people for whom medicines are developed
- Moderator will guide meeting discussion



James Valentine, JD, MHS

Hyman, Phelps & McNamara

Washington, DC



Background on FDA and Drug Development



Drug Discovery

- Typically, researchers discover new drugs through:
 - New insights into a disease process that allow researchers to design a product to stop or reverse the effects of the disease
 - Many tests of molecular compounds to find possible beneficial effects against any of a large number of diseases
 - Existing treatments that have unanticipated effects
 - New technologies, such as those that provide new ways to target medical products to specific sites within the body or to manipulate genetic material
- Once researchers identify a promising compound, the development of drugs follows a well-established path to make sure that they are safe and effective when they reach the public

Preclinical Development

- Preclinical work occurs before a new drug or biologic is tested in humans
- Primary goals are to determine whether the product is
 - Reasonably safe for initial use in humans
 - Sufficiently effective against a disease target in chemical assay tests or animal models
- The end results of the preclinical stage of development is an Investigational New Drug





Clinical Development – Phase 1

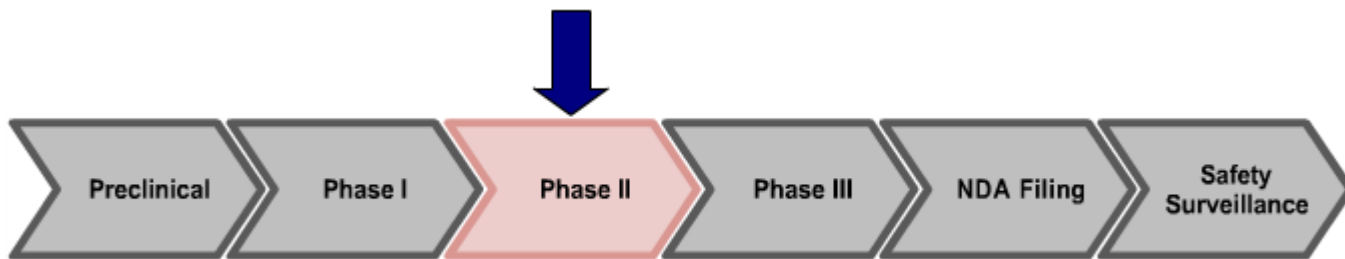
- IND submission
 - Pharmacology/Toxicology Studies
 - Manufacturing Information
 - Clinical Protocols and Investigator Information
- Phase 1 primary goals
 - Place emphasis on a drug's safety
 - Determine the most common side effects of a drug
 - Determine how a drug is metabolized and excreted





Clinical Development – Phase 2

- Primary goals
 - Place emphasis on a drug's effectiveness
 - Determine if the drug works in people who have a certain disease
 - Compare the drug against placebo
 - Continue to monitor short-term side effects and other safety issues





Clinical Development – Phase 3

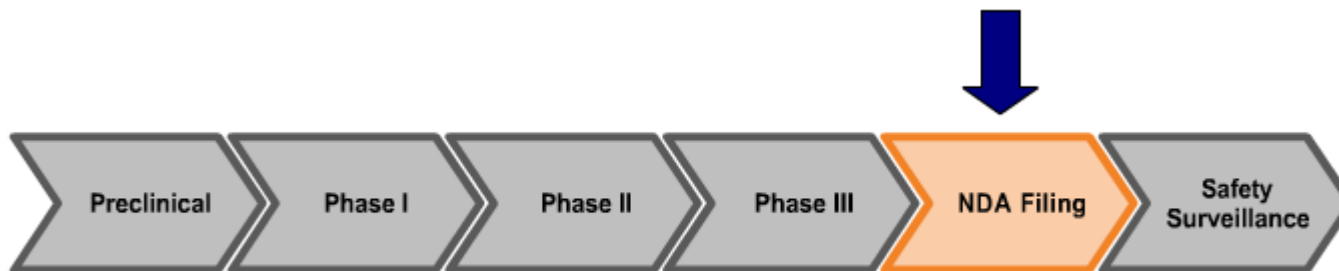
- Primary goals
 - Traditionally large-scale, randomized, placebo-controlled trials
 - Continued assessment of effectiveness, duration of effect, effect in different populations, varying dosages
 - Safety evaluation continues, including potential drug-drug interactions





NDA/BLA Submission

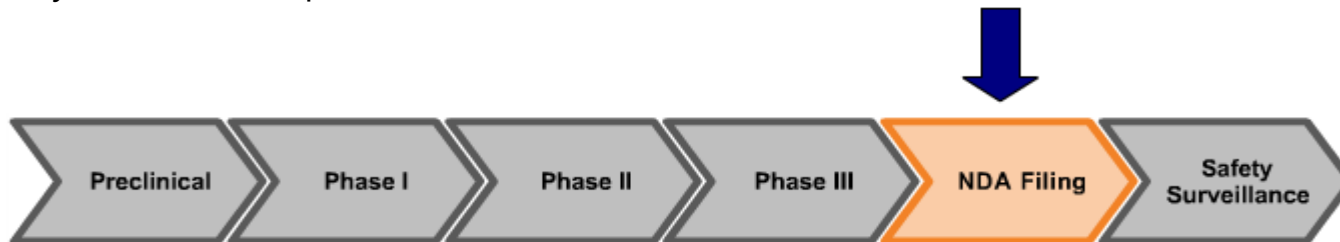
- FDA holds Pre-NDA meeting
 - Identify pivotal studies
 - Discuss methods of statistical analysis
 - Uncover major unresolved issues
- NDA includes all animal and human data from the development program





FDA Review

- FDA determines the application's completeness and assigns a review team to evaluate the application
- FDA assesses
 - Whether effectiveness has been demonstrated for the drug's proposed use
 - Whether the safety assessment is adequate to conclude that the drug is safe (i.e., the benefits of the drug outweigh the risks)
 - Whether the manufacturing methods and the controls used to maintain the product quality are adequate
- Advisory Committee input





Post-Market Safety Surveillance

- Knowledge about a product will always be limited at the time of approval
 - Clinical studies are short and involve a limited patient population
 - New safety information often emerges after a product is used in a wider patient population
- FDA maintains an active program in post-market safety surveillance to monitor adverse events





So What Exactly Is FDA's Role?



Drug Development & Clinical Trials

- ❑ FDA does not develop drugs
 - Researchers, pharmaceutical companies, and nonprofit groups conduct disease research and drug development
- ❑ FDA does not test drugs in clinical trials
 - FDA does not run clinical trials
 - FDA staff are not onsite for clinical trials
 - Researchers conducting trials must seek FDA approval before beginning



Practice of Medicine & Drug Costs

- FDA does not have authority to regulate the practice of medicine
 - **FDA regulates the development and marketing of medical products**
 - Doctors are free to prescribe FDA-approved drug for other conditions (known as “off-label” use)
- FDA does not regulate the price of medicine
 - Consideration of drug prices is not part of FDA’s mandate
 - FDA cannot base an approval decision based on drug price
 - FDA does not even receive pricing information during a review



FDA's Role in Facilitating Drug Development

- FDA's mission includes "Promoting Public Health"
- FDA reviewers have expert knowledge on drug development and clinical trials
 - FDA provides technical help to researchers and drug developers
 - FDA helps companies design clinical trials and studies
 - FDA has numerous meeting with companies throughout the drug development process
- Manufacturers pay user fees under the Prescription Drug User Fee Act (PDUFA)
 - Fees support FDA involvement in drug development and the agency's review of marketing applications



For more information on FDA regulation of medical products, as well as other ways to get involved, visit <http://www.fda.gov/ForPatients/>



Introduction to Patient-Focused Drug Development



Purpose of the Meetings?

- FDA wanted a more systematic way to gather the patient perspective about the condition and available treatment options
- Helps inform their understanding of the context for benefit/risk assessment and decision making for new drugs
- Patient input helps the FDA during drug development and their review of an application for marketing a new drug



Benefit-Risk Assessment

- Analysis of Condition
- Current Treatment Options



Provides regulators with the clinical context for weighing benefits and risks

- Benefit
- Risk
- Risk Management



Incorporates expert judgments based on evaluation of the efficacy and safety data and the expected impact of efforts to reduce and further characterize risks



APOL1 kidney disease EL-PFDD Meeting

- Framework for discussion questions came from FDA's benefit-risk framework; represents important considerations in their decision making
- We reviewed this & other disease area questions and tailored our agenda/panel questions to communicate the most important information related to APOL1 kidney disease
- FDA emphasizes that:
 - ACTIVE PATIENT INVOLVEMENT & PARTICIPATION IS THE KEY TO THE SUCCESS OF THESE MEETINGS!



“Voice of the Patient” Report

- NKF to prepare a written “Voice of the Patient” report after the meeting
 - Summary of patient & audience testimony, polling data & submitted written comments
- Key communication to FDA review staff & the regulated industry about what patients most want to see in a treatment
- FDA wants this information; informs them about ways to develop meaningful treatments for APOL1 kidney disease



Participating in the EL-PFDD Meeting



Meeting Logistics

☐ Who?

- APOL1 kidney disease patients and caregivers, FDA staff, and pharma and biotech companies

☐ When?

- November 6, from 10am – 3:30pm

☐ Where?

- College Park Marriott Hotel and Conference Center, Hyattsville, MD *and*
- Virtual



Overview of the Agenda

- ❑ Welcome and introductory comments from NKF & FDA
- ❑ Background on APOL1 kidney disease and clinical trials by: Drs. Keisha Gibson and Kirk Campbell
- ❑ Broken into three topics:
 1. What are APOL1 kidney disease symptoms and how do they impact your life?
 2. Factors that would influence your decision to participate in a clinical trial for APOL1 kidney disease
 3. How you manage symptoms and current & future approaches to treatment?
- ❑ Topics 1. and 3. will include a patient panel; all topics will include polling questions, and a moderated discussion with the audience
- ❑ Closing comments by NephCure



Discussion Questions: Topic 1

Living with APOL1 kidney disease: Disease Symptoms and Daily Impacts

1. Of all the symptoms that you experience because of your condition, which 1-3 symptoms have the most significant impact on your life?
2. Are there specific activities that are important to you but that you cannot do at all, or as fully as you would like, because of your condition?
 - a. How do your symptoms and their negative impacts affect your daily life on the best days? On the worst days?
3. How has your condition and its symptoms changed over time?
4. What worries you most about your condition?



Discussion Questions: Topic 2

Current Challenges to Treating APOL1 kidney disease

1. What are you currently doing to help treat your condition or its symptoms?
 - a. How has your treatment regimen changed over time, and why?
2. How well does your current treatment regimen treat the most significant symptoms of your disease?
 - a. How well do your treatments address specific symptoms?
 - b. Which symptoms are not addressed as well?
3. What are the most significant downsides to your current treatments and how do they affect your daily life?
4. Assuming there is no complete cure for your condition, what specific things would you look for in an ideal treatment for your condition?



Discussion Format

... and how to make your voices heard

- A panel of people living with APOL1 kidney disease will speak first
 - The purpose is to set the foundation for the broader audience discussion to follow
 - Panelists were selected to reflect a range of experiences with APOL1 kidney disease
 - Polling questions
 - Only patients and caregivers (in-person and virtual audience) can answer
 - Purpose: starting point for the audience discussion
 - Use your cell phone, tablet, or laptop to respond
- Then move to audience discussion with patients and care partners
 - In-person
 - Virtual audience
 - Speak or chat (we will read it) your experience
 - Purpose of audience discussion: build on the experiences shared by the panel
 - James will pose Discussion Questions and invite in-person and virtual audiences to respond



Tips for Effective Participation

- ❑ Remember FDA's role and the purpose of the meeting
- ❑ Review Discussion Questions in advance (to be sent)
- ❑ If you have something to share, relate it to the most appropriate topic being discussed
- ❑ It is okay to reiterate a feeling/experience already voiced by someone that is similar to your own, but give it a personal or unique perspective
- ❑ **Keep your comments concise & focused;** there are many voices to be heard about this emotional topic
- ❑ You can send additional comments after the meeting



Additional Information

- Pre-meeting survey:
 - Meeting website (*Google APOL1 kidney disease– PFDD – NKF*)
 - Responses will be kept confidential
- Comments may be submitted for up to 30 days after the EL-PFDD meeting
 - Email responses to david.feldman@kidney.org
 - These comments will be included in the “Voice of the Patient” report
- Voice of the Patient Report
 - Comprehensive report of the meeting
 - To be submitted to FDA
- College Park Marriott and Conference Center, Hyattsville, MD
 - Discount rate for hotel room may still be available



Questions? Need Assistance?

- ☐ This webinar will be recorded and available upon request.
- ☐ Questions about participating in the EL-PFDD meeting?

David Feldman at david.feldman@kidney.org



Summary

- This is YOUR OPPORTUNITY to be part of the process
- You can have a meaningful impact on drug development & clinical trial design
- Your collective voices must be heard at the beginning of the process to help:
 - FDA assess risks & benefits of a new drug while fully understanding the impact of APOL1 kidney disease and the patient perspective
 - Companies design trials that meet your needs
- MAKE A DIFFERENCE!!



THANK YOU

Let us answer your questions