VOICE OF THE PATIENT FORUM YOUR ROLE IN DRUG DEVELOPMENT

April 21, 2020

What is it, and how can you participate?

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FSHD SOCIETY MDA® OPTUM® friends of FSH research In Pursuit of a Cure

Purpose of Meeting?

To educate the Food & Drug Administration (FDA)

- ✓ What it is like to live with FSHD
- ✓ Your concerns as the disease progresses
- ✓ How you are currently managing FSHD
- ✓ What meaningful treatments look like

Why Should I Participate?

- Opportunity to have Your Voices heard
- The FDA approves all treatments for FSHD and needs to know what is important to you
- This knowledge will impact their decision-making, and lead to better treatments and potentially faster approvals for FSHD

Why Now?

- The **Patient's Voice** has historically been absent from the drug development process
  - ✓ Until a drug was approved by the FDA (sometimes with non meaningful endpoints for patients), or
  - ✓ A clinical trial was failing and the drug company or FDA wanted input from actual patients to understand the problem
- Want your Voices heard at the beginning of the process and throughout
- Stakeholder engagement = FDA Priority
  - ✓ Initiated a *Patient Focused Drug Development Program* to learn about disease impact on patient's lives both from patients and their caregivers

Overview

- Background on FDA & Drug Development
- Introduction to FDA's Patient-Focused Drug Development
- Participating in the Meeting
  - Logistics, Format, and Tips
- Other Important Information

Background on FDA and Drug Development

Drug Discovery

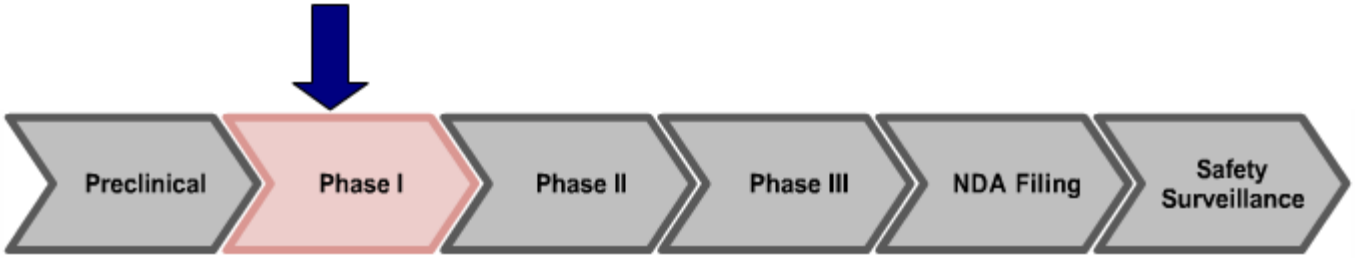
- Typically, researchers discover new drugs through:
  - New insights into a disease process that allow researchers to design a product to stop or reverse the effects of the disease
  - Many tests of molecular compounds to find possible beneficial effects against any of a large number of diseases
  - 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 Application (IND)

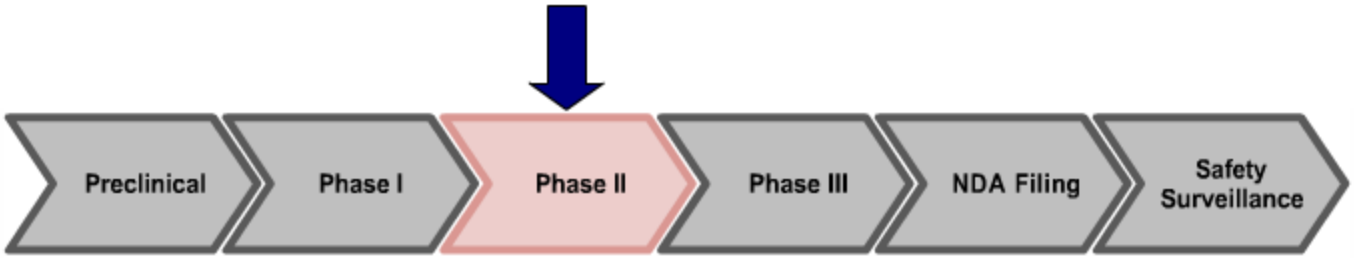
Clinical Development – Phase 1

- 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



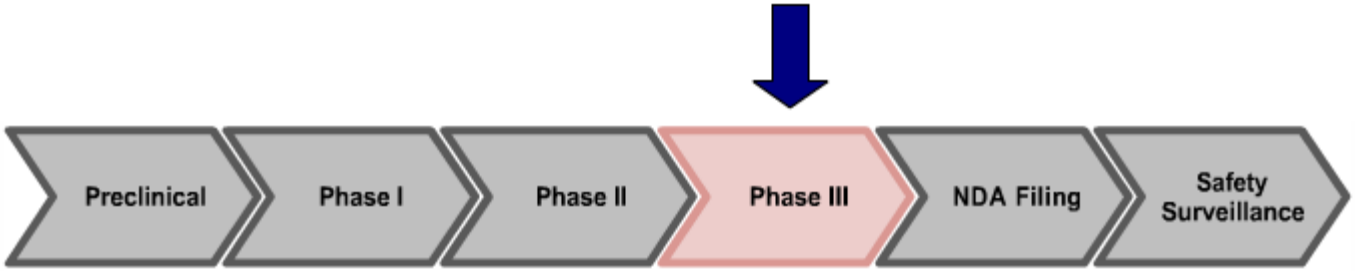
Clinical Development – Phase 2

- 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



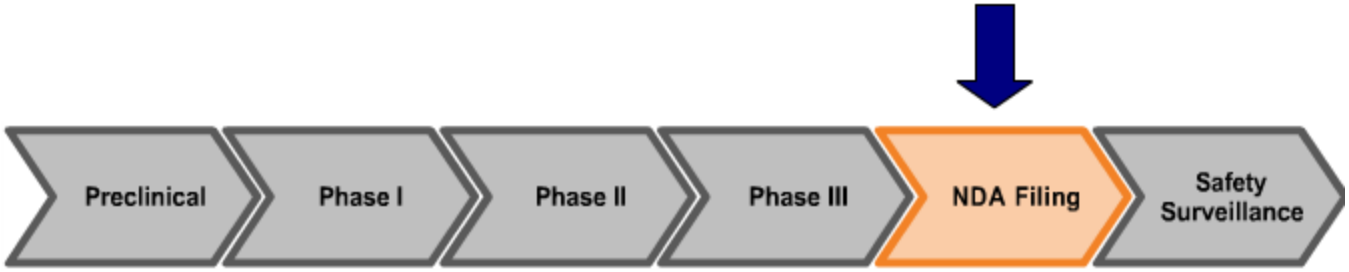
Clinical Development – Phase 3

- 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



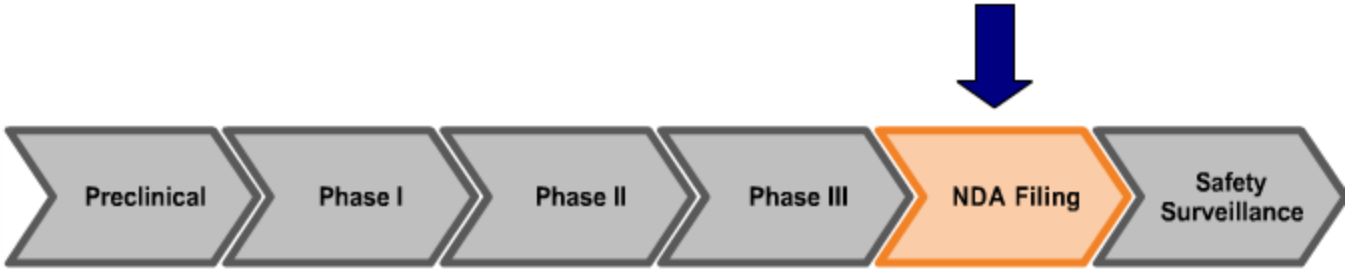
NDA/BLA Submission

- 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



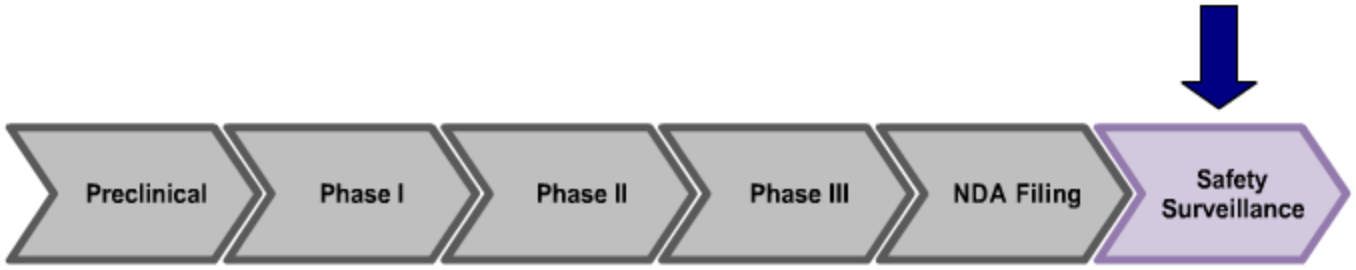
FDA Review

- 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 brief in duration 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

Image used with permission of the FDA

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

FSHD "Voice of the Patient" 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 FSHD
- FDA emphasizes that:
  - ✓ ACTIVE PATIENT INVOLVEMENT & PARTICIPATION IS THE KEY TO THE SUCCESS OF THESE MEETINGS!

“Voice of the Patient” Report

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

Participating in the Meeting

Meeting Logistics

- Who?
  - ✓ FSHD patients and caregivers, FDA staff, and pharma and biotech companies
- When?
  - ✓ April 21, 2020 from 10:00am – 3:30 pm
- Where?
  - ✓ College Park Marriott Hotel & Conference Center in Hyattsville, Maryland
- How?
  - ✓ **In-person** or online via a **live webcast**

Overview of the Agenda

- Welcome and introductory comments from FSHD Society & FDA
- Background on FSHD and clinical trials by FSHD expert
- Broken into two topics:
  1. How FSHD symptoms affect your life
  2. How you manage symptoms and current & future approaches to treatment
- Topics will include patient panels and will include polling questions and a moderated discussion with the audience
- Closing comments by FSHD Society

Discussion Questions

Topic 1: *Living with FSHD: Disease Symptoms and Daily Impacts*

- Of all the symptoms of FSHD which 1-3 symptoms have the most significant impact on your life?
- How does FSHD affect you on best and on worst days? Describe your best days and your worst days.
- Are there specific activities that are important to you that you cannot do at all or as fully as you would like because of FSHD?

Discussion Questions (cont.)

Topic 1: *Living with FSHD: Disease Symptoms and Daily Impacts*

- How have your symptoms changed over time?
  - How has your ability to cope with the symptoms changed over time?
- What do you fear the most as you get older?
  - What worries you most about your condition?
  - What frustrates you most about your condition?

Discussion Questions (cont.)

Topic 2: *Current Challenges to Treating FSHD*

- What are you currently doing to manage your FSHD symptoms?
- How well do these treatments treat the most significant symptoms of your FSHD?
- What are the most significant downsides to your current treatments and how do they affect your daily life?
- Short of a complete cure, what specific things would you look for in an ideal treatment for FSHD?

Discussion Format

- There will be a panel of patients for each panel
  - The purpose is to set the foundation for the broader audience discussion
  - Panelists are selected to reflect a range of experiences with the condition
- Patients in the audience and online will have a chance to answer “polling” questions
  - Their purpose is as a starting off point for the discussion
  - Participants will use cell phones, tablets, or laptops to respond
- Then move to a discussion with patients in the audience
  - The purpose is to build on the experiences shared by the panel
  - A moderator will ask questions and invite you to raise your hand to respond

Tips for Effective Participation

- Remember FDA's role & the purpose of the meeting
- Review each Discussion Question in advance
- If you have something important to share, relate it to the most appropriate topic/panel question
- 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 always send in additional comments after the meeting

Preparatory Surveys

Please open your emails from the FSHD Society!

From January 28-March 31, every two weeks you will get a new survey

April 21 Meeting & Live Poll

Voice of the Patient Report

Participating in the Discussion

- Two ways to participate in the meeting:
  - In person (conference center in Hyattsville, MD)
  - By webcast (remotely; polling questions & written submissions)
  - Responses will be kept confidential
- Register for meeting:
  - **fshdsociety.org/fsh-events/vopf/**
  - Comments with answers to the questions may be submitted for up to 30 days after the meeting
  - Email responses to **vopf@fshdsociety.org**
  - These comments will be included in the "Voice of the Patient" report to be submitted to FDA

To attend the Live Webcast on April 21

- **Please register in advance.**
- Virtual “room capacity” is limited so we need a headcount to make sure everyone will be accommodated.
- Register here:
  - **fshdsociety.org/fsh-events/vopf/**
- You will receive a log-in link on the day of the live webcast.

Would you like to attend in person?

- **Space is limited.** Register ASAP. www.fshdmeetings.org/VOPF
- A block of rooms at the College Park Marriott is being held for the meeting. Travel and hotel is the responsibility of the attendee.
- Your participation is invaluable!
- Deadline to register to attend is April 6.
- If for any reason you have to cancel, please notify the Society so a person on the waitlist can go.

Travel Information

- A limited number of discounted rooms will be blocked at the College Park Marriott Hotel
  - The rate is $179; after registering to attend, you will receive a link to reserve from the room block.
  - Or call Marriott Reservations at 1(800) 228-9290 or (301) 985-7300 and identify yourself as a guest attending the FSHD Society's VOPF meeting.
  - The deadline to receive this rate is Tuesday, March 24, 2020.
- Travel information:
  - By air: Fly into Reagan National or BWI airport
  - By train: Union Station (Washington, DC)
  - Ground travel: Instructions will be provided by email

Questions? Need Assistance?

This webinar is being recorded and will be available online at: youtube.com/FSHDSociety

If you have questions about participating, contact: **vopf@fshdsociety.org**

Summary

- This is YOUR OPPORTUNITY to be part of the process
- You can have a meaningful impact on clinical trial design & drug development
- Your (collective) voices must be heard at the *beginning* of the process to help:
  - ✓ companies design trials that meet your needs
  - ✓ FDA assess risks & benefits with a full understanding of the impact of FSHD & the patient perspective
- MAKE A DIFFERENCE!!

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Please visit the meeting landing page to register:

fshdsociety.org/fsh-events/vopf/