HLAA
Hearing Loss Association of America

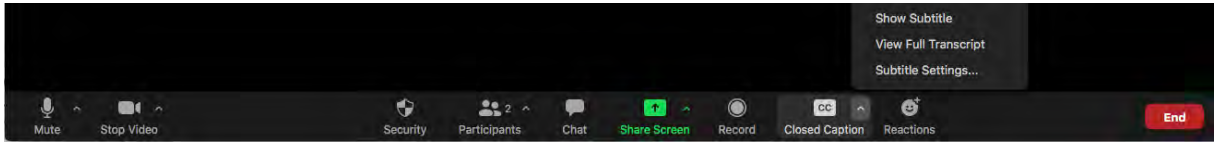
Welcome to HLAA Webinars

HLAA WEBINAR

Externally-Led Patient-Focused Drug Development Meeting for People and Families Living with Sensorineural Hearing Loss

Guest Speakers: Larry Bauer, Hyman, Phelps, & McNamara

Captions



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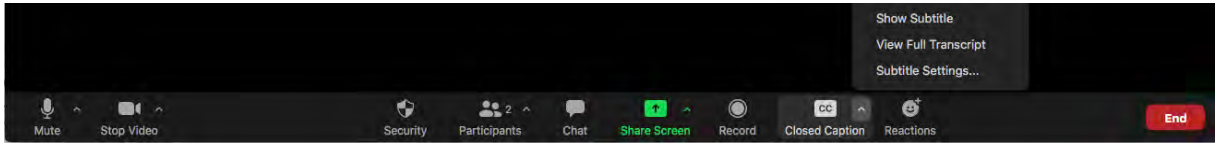
Increase Caption Font Size



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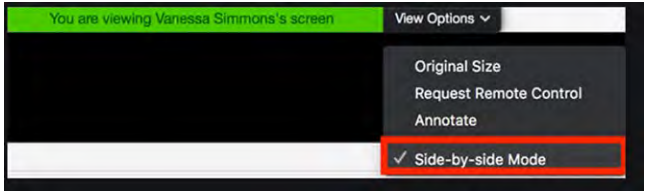
Chat and Q & A

- Open Chat and move that box on your screen to avoid overlap of captions and chat messages. Please use chat for technical issues and panelists only.

- Click on Q & A icon to ask questions. We will be using this feature to facilitate questions after the presentation.

Adjust Side-by-side view



- Your screen should be in Side-by-side mode if you have joined by computer.
- There should be a shared screen with presentation on the left and the speakers will appear on the right in gallery view.
- You can adjust the size of your Side-by-side view by hovering your pointer between the shared screen and panelist videos and click on grey line and slide to adjust to your desired view.

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- American Academy of Otolaryngology–Head and Neck Surgery (AAO-HNS)
- American Speech-Language-Hearing Association (ASHA)
- American Tinnitus Association
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- Association of Late-Deafened Adults (ALDA)
- Bionics Institute
- British Cochlear Implant Group
- Canadian HARD of HEARING Association (CHHA-Hamilton)
- CaptionCall
- Cochlear
- Cochlear Implant International Community of Action (CIICA)
- Deafness Forum of Australia
- Deafness Foundation Australia
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- European Association of Cochlear Implant Users (EURO-CIU)
- European Federation of Hard of Hearing People (EFHOH)
- The Global Alliance of Speech-to-Text Captioning
- Hands & Voices
- Hearing Health Foundation (HHF)

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- Hearing Industries Association
- Hearing Matters Australia
- Hearing Rehabilitation Foundation
- Hearing Tracker
- Ida Institute
- International Federation of Hard of Hearing People (IFHOH)
- International Hearing Society (IHS)
- MED-EL Corporation
- NIHR University College London Hospitals Biomedical Research Centre
- OTOjOY
- Phonak
- Pindrop Foundation
- RNID-UK
- Sharon L. King Foundation
- Starkey
- Tennessee Captioning
- Tinnitus Hub
- U.S. Department of Veterans Affairs
- University of Iowa Department of Otolaryngology Cochlear Implant Clinical Research Center
- Usher Syndrome Coalition

Today's Presenter

Larry Bauer

Welcome!

HLAA's EL-PFDD meeting is a chance to make your voice heard to shape future treatments.

It doesn't matter where you are in your journey…
What matters is your story.

Purpose of EL-PFDD Meeting

To educate the U.S. Food & Drug Administration about:

- What it's like to live with hearing loss
- Concerns related to hearing loss
- Your experiences in managing hearing loss
- What meaningful treatments would look like to you

Why Should I Participate?

- The FDA approves all new treatments for hearing loss and needs to know what is important to you
- You can impact FDA decision-making, leading to better treatments and potentially faster approvals.
- Industry and nonprofit stakeholders should join to hear vital perspectives from our community.

Why Now?

- Stakeholder engagement is an FDA priority
- Hearing loss trials are gaining momentum
- Elevating the perspective of people with hearing loss
- Chance to ensure that emergent treatments meet your needs and those of the next families facing hearing loss

Housekeeping

- **Today's session is scheduled to last approximately one hour**
- **All participants will be muted**
- **The webinar will be recorded and available for later viewing on**

- **To ask questions, please use your Q&A box at the bottom of your screen -- we will answer them at the end of the webinar.**

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

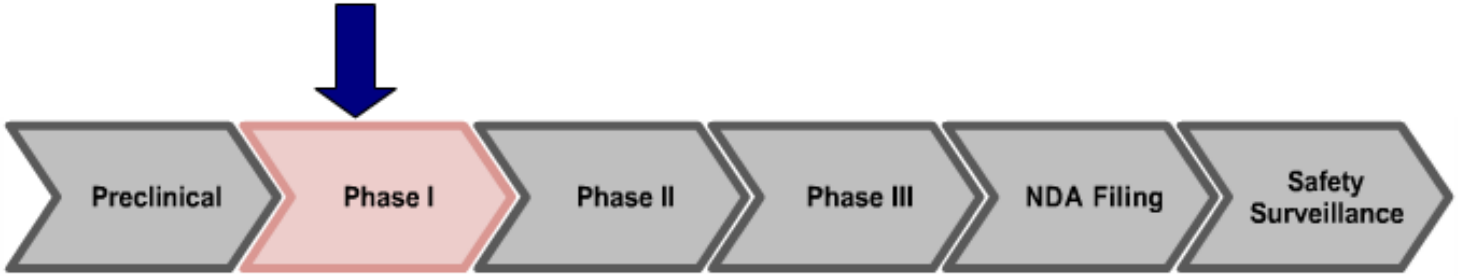
- Typically, researchers discover new drugs through:
  - Learning new insights into a disease process that allows researchers to design a product to stop or reverse the effects of the disease
  - Conducting many tests of molecular compounds to find possible beneficial effects against any of a large number of diseases
  - Learning from existing treatments that have unanticipated effects
  - Developing 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

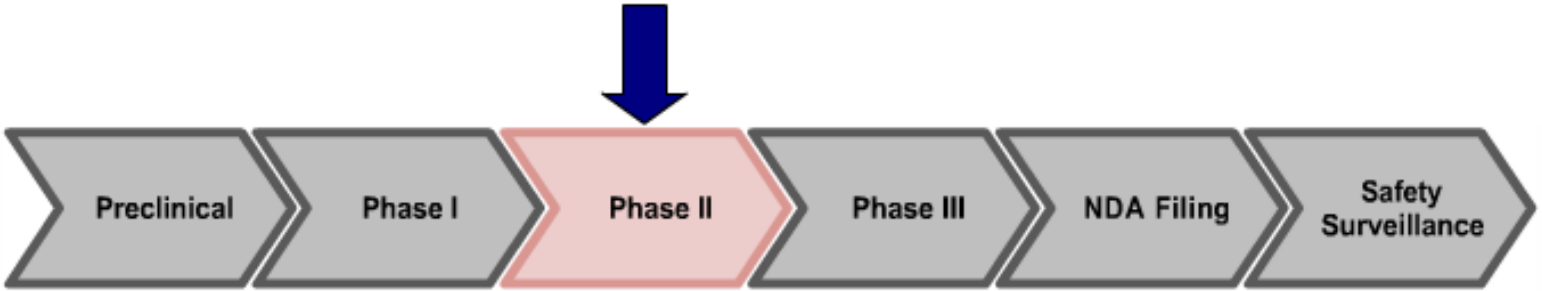
Clinical Development – Phase 1

- Investigational New Drug (IND) submission
- 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



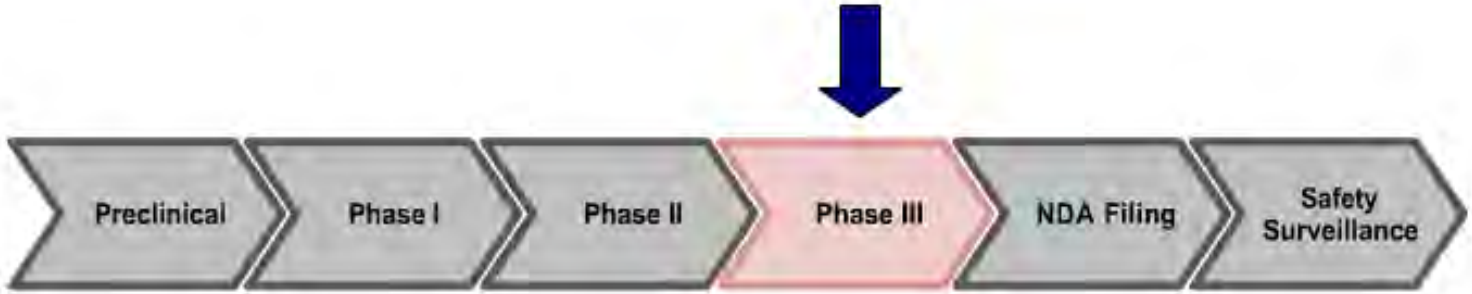
Clinical Development – Phase 2

- 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



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

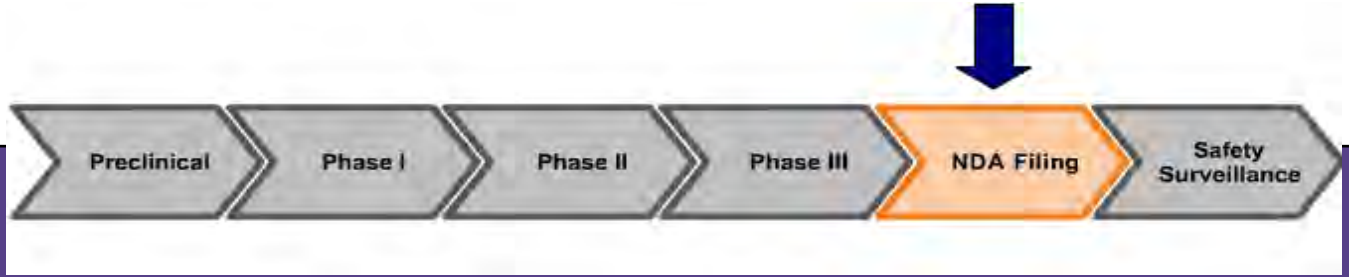


NDA/BLA Submission

- 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 cohort
  - New safety information often emerges after a product is used in a wider 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
  - However, researchers conducting trials must seek FDA approval before beginning to give a new drug to human beings

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

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 lived 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
- Input from people and families affected 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

EL-PFDD Meeting on hearing loss: Your Story Matters

- 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 hearing loss
- FDA emphasizes that:
  - ✔ **ACTIVE INVOLVEMENT & PARTICIPATION FROM PEOPLE AND FAMILIES AFFECTED IS THE KEY TO THE SUCCESS OF THESE MEETINGS!**

“Voice of the Patient” Report

- HLAA will prepare a written “Voice of the Patient” report after the meeting
  - ✔ Summary of testimony and input from people with hearing loss, caregivers/care partners, polling data & submitted written comments
- Key communication to FDA review staff & the regulated industry about what people with hearing loss and care partners/caregivers most want to see in a treatment
- FDA wants this information; informs them about ways to develop meaningful treatments for hearing loss

Participating in the Meeting

Meeting Logistics

- Who?
  - ✓ People living with hearing loss, care partners, caregivers and family members, people at risk of hearing loss, FDA staff, pharma and biotech companies, and nonprofit partners
- When?
  - ✓ May 25th, 2021 from **10 a.m. – 3 p.m. U.S. Eastern Time**
- Where?
  - ✓ Virtual - Anywhere
- How?
  - ✓ Interactive Live Stream

Overview of the Agenda

- Welcome and introductory comments from HLAA & FDA
- Background on hearing loss and clinical trials by hearing loss expert
- Broken into two topics:

  Panel 1: How hearing loss symptoms affect your life

  Panel 2: How you manage symptoms and current & future approaches to treatment
- Topics will include pre-recorded panels and will include polling questions and a moderated discussion with the audience
- Closing comments

Discussion Questions

Topic 1: *Living with hearing loss: Symptoms and Daily Impacts*

1. Of all the symptoms of hearing loss, which 1 to 3 have had the most significant impact on you?

a) Which symptoms most affect you now?

b) Which symptoms were the most significant at other times in your life?

2. How has hearing loss affected you on your best and on worst days? Describe the best days and the worst days.

Discussion Questions (cont.)

3. Are there specific, personally-significant activities that you cannot do at all, or not do as fully because of hearing loss?

a) How does this affect relationships/friendships with others?

b) How does it affect life activities (school/work, abilities; relationships, self-sufficiency, living situation, activities, etc.)?

c) If you could do one activity that you currently are unable to do, what would it be?

4. What worries you most about living with hearing loss?

Discussion Questions (cont.)

Topic 2: *Perspective on Current and Future Treatments for hearing loss*

1. What are you currently (or recently) doing to manage hearing loss symptoms?

a) Which specific hearing loss symptoms do the treatments address?

b) How has this treatment regime changed over time and why?

2. What are the most significant downsides to these hearing loss treatments and how do they affect daily life?

Discussion Questions (cont.)

3. How well have these treatments helped your hearing loss overall?

4. Along the pathway to a cure, what specific things would you look for in an ideal treatment for hearing loss? What factors do you consider when making decisions about selecting a course of treatment?

Discussion Format

1. There will be a group of people with hearing loss 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
2. People with hearing loss and care partners/caregivers in the audience 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
3. Then move to a discussion with people with hearing loss and caregivers in the audience
   - The purpose is to build on the experiences shared by the panel
   - A moderator will ask questions and invite you to submit responses in writing or **preferably, to call in to provide live comments**

Tips for Effective Participation

- Remember FDA's role & the purpose of the meeting
- Review the Discussion Questions in advance
- If you have something important to share, relate it to the most appropriate topic/panel question and **call or write in! (number provided during meeting)**
- 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
- You can always send in additional comments after the meeting

Participating in the Discussion

Register for the meeting:

HLAA Registration Link: https://www.hearingloss.org/hlaa-pfdd/

Participate in the meeting:

- By webcast (remotely; polling questions, call in comments & written submissions)
- Responses will be kept confidential
- Comments with answers to the questions may be submitted for up to 30 days after the meeting to HLAA
- Comments will be included in the “Voice of the Patient” report to be submitted to FDA

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 hearing loss & the lived perspective
- HELP SHAPE THE FUTURE OF HEARING LOSS TREATMENT!!

Questions?

Please use Q & A icon on meeting bar

to ask a question.

Thank you.

Thank you for joining HLAA Webinars

For more educational resources
on hearing loss and recorded webinars,
please visit
hearingloss.org