COVID19 Rare Diseases
FDA Listening Session
Welcome from NORD

Debbie Drell
NORD
Director of Membership
Agenda

Welcome and Overview
Debbie Drell, Membership Director, NORD
Janet Maynard M.D., M.H.S. Director, Office of Orphan Products Development, FDA

Speakers
Kristi Ouimet, Laura Bonnell, and Renie Moss, rare disease community representatives
Kathleen Donohue, M.D., Director (Acting), Division of Rare Disease and Medical Genetics, Center for Drug Evaluation Research
M. Khair ElZarrad Ph.D., M.P.H., Deputy Director, Office of Medical Policy, Center for Drug Evaluation and Research
Aftin Ross, M.S.E., Ph.D., Senior Project Manager/Senior Science Health Advisor All-Hazards Readiness Response and Cybersecurity (ARC) OST, CDRH

Closing
NORD, an independent nonprofit, is leading the fight to improve the lives of rare disease patients and families.

We do this by supporting patients and organizations, accelerating research, providing education, disseminating information and driving public policy.
NORD’s COVID Response: Surveying the Community

Goal: Understand the Experience of Patients with Rare Diseases Dealing with the Impact of COVID

Activities: NORD Surveyed the community twice. Once in April and then again in June

Respondents: A total of 1,600+ responses were collected. 36% of participants contributed to both surveys.

Outcome: Our analyses have been published on NORD’s website, shared by the FDA, and cited in a statement sent to the World Health Organization by international patient advocacy organizations.
Expectations for this Listening Session

Interest in and Scope of Meeting

• Interest in Listening Session
  • 620 registered
  • 116 interested in speaking
  • ~100 submitted questions to FDA

• Focus on three major topics:
  • drug shortages
  • personal protective equipment (PPE) shortages
  • access to clinical trials
Welcome from FDA

Janet Maynard M.D., M.H.S.
Director, Office of Orphan Products Development, FDA
A Family’s Experience with Drug Shortages during the Pandemic: Kristi Ouimet

Matthew, 9  Molly, 17
Primary Hyperoxaluria Type 1
FDA’s Response to Drug Shortages and the Oimet Family’s Experience and Rare Disease Community Questions

M. Khair ElZarrad Ph.D., M.P.H., Deputy Director, Office of Medical Policy Center for Drug Evaluation and Research

Kathleen Donohue, M.D., Director (Acting), Division of Rare Disease and Medical Genetics, Center for Drug Evaluation and Research
Talk to your doctor about the type of therapy you are on and whether it is a biological product (CBER) or a more common drug (CDER).
A Family’s Experience with PPE Shortages during the Pandemic: Laura Bonnell

Cystic Fibrosis
FDA’s Response to Drug Shortages and the Bonnell Family’s Experience

Aftin Ross, M.S.E., Ph.D.,
Senior Project Manager/
Senior Science Health Advisor
All-Hazards Readiness
Response and Cybersecurity
(ARG) OST, CDRH
Online References

Coronavirus Disease 2019 (COVID-19), Resources for Patients

Personal Protective Equipment for Infection Control Questions
www.fda.gov/medical-devices/personal-protective-equipment-infection-control/questions-about-personal-protective-equipment-ppe

Personal Protective Equipment Emergency Use Authorizations

FAQs on Shortages of Surgical Masks and Gowns During the COVID-19 Pandemic

How to Protect Yourself (CDC)
A Family’s Experience with Clinical Trials during the Pandemic: Renie Moss

Neurofibromatosis type 1

Philip, 15
Helen, 12
FDA’s Response to: Clinical Trials, the Moss’ Family’s Experience, and the Rare Disease Community Questions

M. Khair ElZarrad Ph.D., M.P.H., Deputy Director, Office of Medical Policy Center for Drug Evaluation and Research

Kathleen Donohue, M.D., Director (Acting), Division of Rare Disease and Medical Genetics, Center for Drug Evaluation and Research

Conduct of Clinical Trials of Medical Products during COVID-19 Public Health Emergency

General Qs on the conduct of clinical trials during COVID-19
Clinicaltrialconduct-COVID19@fda.hhs.gov
Questions for FDA?

http://www.fda.gov/PatientsAskFDA
How NORD Supports You

COVID-19 Resource Center

The health and safety of those with rare diseases and their caregivers are always our top priority. The National Organization for Rare Disorders® (NORD) has always been in solidarity with the rare disease community. In light of the COVID-19 pandemic, we would like to extend support for those impacted by COVID-19, directly and indirectly. Our focus is on how to be prepared and seek proper care under these unusual circumstances, we aim to empower the community during this unprecedented time.

Alone we are rare. Together we are strong.

NORD COVID-19 Resources

- On-Demand Videos
- On-Demand Webinars
- Advocacy Opportunities and Tools
- Disease-Specific Resources for Patients
- Informational Materials
- NORD COVID-19 Policy Statements
- Resources for Nonprofits
Concluding Thoughts
Thank you.