



DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2021-N-0689]

Public Workshop: Analgesic Clinical Trial Designs, Extrapolation, and Endpoints in Patients from Birth to Less Than Two Years of Age

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of public workshop.

SUMMARY: The Food and Drug Administration (FDA) is announcing a public workshop entitled “Analgesic Clinical Trial Designs, Extrapolation, and Endpoints in Patients from Birth to Less Than Two Years of Age.” The purpose of the public workshop is to discuss the state of science, data gaps, and challenges in drug development for drugs intended to treat acute pain in patients less than 2 years of age.

DATES: The public workshop will be held virtually on October 13 and 14, 2021, from 10 a.m. to 2 p.m. Eastern Standard Time. See the SUPPLEMENTARY INFORMATION section for registration date and information.

ADDRESSES: The public workshop will be held in virtual format only and will not be held at a specific location. Please note that due to the impact of the COVID-19 pandemic, all meeting participants will be joining this public meeting via an online teleconferencing platform. The public workshop will be held at <https://go.umd.edu/analgesic-clinical-trial>.

FOR FURTHER INFORMATION CONTACT: Heather Buck at Heather.Buck@fda.hhs.gov, 301-796-1413 or Kerri-Ann Jennings at Kerri-Ann.Jennings@fda.hhs.gov, 301-796-2919, Food and Drug Administration, Center for Drug Evaluation and Research, 10903 New Hampshire Ave., Bldg. 22, Rm. 6467, Silver Spring, MD 20903-0002.

SUPPLEMENTARY INFORMATION:

I. Background

In 2009, FDA convened a scientific workshop with experts in pediatric pain, pediatric clinical trial design, pediatric ethics, and pediatric drug development¹. Based on the available data at the time, the expert panel recommended extrapolation of efficacy in patients 2 years and older, relying on matching effective drug exposures in adults. The current approach to study drugs with well-established mechanisms of action, such as opioids, non-steroidal anti-inflammatory drugs (NSAIDs), acetaminophen, and local anesthetics, relies on matching safe and effective drug exposures in adults to support the efficacy of drugs used to treat acute pain in pediatric patients at least 2 years of age. Controlled efficacy trials are only required in patients from birth to less than 2 years of age. When controlled efficacy trials are needed, FDA has recommended an “add-on” design using opioid-sparing calculation rather than the change in pain intensity used in efficacy trials of analgesics in adults.

Despite these advances in clinical trial design, there continues to be unmet needs in the availability of products to treat acute pain, especially in patients less than 2 years of age. There is currently only one analgesic labeled for use in patients less than 2 years of age: ibuprofen is approved for the treatment of pain in children 6 months of age and older. Furthermore, controlled trials in patients less than 2 years of age have been difficult to complete and the data obtained from completed trials have often been difficult to interpret.

The purpose of the public workshop is to discuss the current state of therapies to treat acute pain in children, identify data gaps, and consider methods to improve the current drug development paradigm for acute pain in patients less than 2 years of age (e.g., use of pediatric extrapolation, and novel clinical trial designs). The workshop is intended to focus on drugs with well-established mechanisms of action (NSAIDs, acetaminophen, local anesthetics, opioids), rather than drugs with novel mechanisms of action.

¹ Berde, CB, et.al., Pediatrics 2012 Feb;129(2):354-64.
<https://www.fda.gov/advisory-committees/advisory-committee-calendar/april-12-2016-pediatric-advisory-committee-meeting-announcement-04122016-04122016>

II. Topics for Discussion at the Public Workshop

The main objective of the “Analgesic Clinical Trial Designs, Extrapolation, and Endpoints in Patients from Birth to Less Than Two Years of Age” workshop is to discuss the current state of therapies to treat acute pain in children, identify data gaps, and discuss feasible trial designs and methods (e.g., use of pediatric extrapolation) to improve the current drug development paradigm for acute pain in patients less than 2 years of age. The workshop will include regulators, industry, academia, and patient organizations to optimize the discussion of the selected topics.

III. Participating in the Public Workshop

Registration: Please visit the following website to register for this public workshop: <https://go.umd.edu/analgesic-clinical-trial>. Please provide complete contact information for each attendee, including name, title, affiliation, address, email, and telephone.

Streaming Webcast of the Public Workshop: This public workshop will also be webcast at the following site: <https://collaboration.fda.gov/rz3mubd491lo/>.

If you have never attended a Connect Pro event before, test your connection at https://collaboration.fda.gov/common/help/en/support/meeting_test.htm. For an overview of the Connect Pro program, visit https://www.adobe.com/go/connectpro_overview. FDA has verified the website addresses in this document, as of the date this document publishes in the *Federal Register*, but websites are subject to change over time.

Dated: September 22, 2021.

Lauren K. Roth,

Acting Principal Associate Commissioner for Policy.