C R I T
collaboration for research integrity and transparency

Collaboration for Research Integrity and Transparency

A PROGRAM OF YALE LAW SCHOOL, YALE SCHOOL OF MEDICINE AND THE YALE SCHOOL OF PUBLIC HEALTH

February 15, 2018

Via Electronic Submission

Division of Dockets Management
Food and Drug Administration
5630 Fishers Lane, Room 1061, HFA-305
Rockville, MD 20852

Docket FDA-2015-N-2002

Yale Collaboration for Research Integrity and Transparency
Comments on FDA's Draft Guidance on the "Clarification of Orphan Designation of Drugs and Biologics for Pediatric Subpopulations of Common Diseases"

The Yale Collaboration for Research Integrity and Transparency (CRIT) supports the changes to orphan designation described in the FDA proposed guidance entitled "Clarification of Orphan Designation of Drugs and Biologics for Pediatric Subpopulations of Common Diseases."

The Pediatric Research Equity Act (PREA) has contributed significantly to the generation of new evidence on dosing, safety, and efficacy on the pediatric use of approved therapeutics, leading to the addition of pediatric labeling information for over 487 drugs and biologics.[1] However, the interplay of the pediatric-subpopulation orphan designation and the PREA orphan exemption has created the unintended consequence of

[1] US Food and Drug Administration (FDA) [Accessed February 12, 2018]; New pediatric labeling information database. www.accessdata.fda.gov/scripts/sda/sdNavigation.cfm?sd=labelingdatabase.

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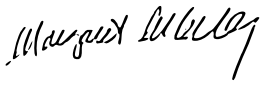
exempting drugs granted pediatric-subpopulation orphan designation from PREA requirements. The proposed changes outlined in the guidance will close this loophole. We agree with the FDA's assessment that the Rare Pediatric Disease (RPD) Priority Review Voucher (PRV) program provides appropriate incentives for rare pediatric disease drug development, so that the pediatric-subpopulation orphan designation is no longer needed.

In addition, we urge the FDA to publish a list of drugs that were previously granted pediatric-subpopulation orphan designation, that have not undergone the pediatric studies contemplated in PREA due to this loophole, and to work with sponsors of these drugs to encourage voluntary compliance with PREA requirements.

We urge FDA to finalize the draft guidance document, and to take the steps described above to encourage the completion of pediatric studies for drugs that were previously granted pediatric-subpopulation orphan designation, thereby generating additional evidence on dosing, safety, and efficacy on the pediatric use of these approved therapeutics, improving care for pediatric patients.

We appreciate the opportunity to comment.

Sincerely,



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