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January 11, 2018

**Comments RE: Draft Guidance on Clarification of Orphan Designation of Drugs and Biologics for Pediatric Subpopulations of Common Diseases (Docket No. FDA-2017-D-6380)**

Public Citizen is a consumer advocacy organization with more than 400,000 members and supporters nationwide. We advocate in an array of issue areas to advance the public interest, including ensuring prescription drugs meet high safety and efficacy standards and are made more affordable both in the U.S. and abroad.

We submit the following comments in support of the Food and Drug Administration (FDA) draft guidance to no longer grant orphan drug designation to drugs for use in pediatric subpopulations of common diseases or conditions unless: 1) The disease in the pediatric population constitutes a valid orphan subset, or 2) the disease in the pediatric subpopulation is a different disease from the disease in the adult population.

Although the rationale provided in the draft guidance focuses on closing a loophole that has allowed drug manufacturers to evade a requirement to conduct pediatric studies, the draft guidance also may enable pediatric patients to gain earlier access to low-cost generic versions of certain drugs.

Manufacturers of products that are granted FDA marketing approval for an orphan designation receive special rewards from the government, including fee waivers, research and development tax credits, and seven years of marketing exclusivity for that orphan indication.

Given that, for any chronic health condition, there are almost invariably fewer than 200,000 pediatric patients,<sup>1</sup> granting orphan designation for pediatric subgroups of common adult diseases makes orphan designations possible for a wide range of drugs that treat common diseases.

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<sup>1</sup> The total population of children in the U.S. is 74 million. (Source: Childstats.gov. POP1 Child Population: Number of Children (in millions) Ages 0-17 in the United States by Age. <http://www.childstats.gov/americaschildren/tables/pop1.asp>. See 2016 figure.) To reach the 200,000 patient threshold, the prevalence of a condition would have to be above 0.3 percent among children. Only a few very common conditions exceed this 0.3 percent threshold. Rezaee ME, Pollock M. Multiple chronic conditions among outpatient pediatric patients, southeastern Michigan, 2008-2013. *Prev Chronic Dis.* 2015;12:E18.

No longer granting orphan designation to drugs for use in pediatric subpopulations when they are also safe and effective in a much broader population of adults will prevent cost-lowering generic competition for such orphan indications from being delayed by this seven-year exclusivity period.

Moreover, although this draft guidance is a good first step, numerous other problems and misaligned incentives of the Orphan Drug Act are in need of remedy, as outlined in Public Citizen's report, "House Orphan Drug Proposal: A Windfall for Pharma, False 'Cure' for Patients."<sup>2</sup> These remedies include encouraging the development of truly novel compounds instead of repurposing and making minor changes to old drugs, setting more rigorous standards for clinical trials conducted on drugs for orphan diseases, and preventing companies from receiving orphan drug benefits for products that are ultimately used in much larger patient populations.

Although further reforms to the Orphan Drug Act are necessary, this draft guidance is a positive development. We urge the FDA to finalize the draft guidance as soon as possible.

Thank you for taking our comments into consideration.

Sincerely,

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<sup>2</sup> Almashat, S. Sorscher, S., Knievel S. (2015, December). House Orphan Drug Proposal: Windfall for Pharma, False 'Cure' for Patients. *Public Citizen*. Retrieved December 27, 2017, from <https://www.citizen.org/sites/default/files/2289.pdf>