Drug Repurposing Guidebook

Building Block U229

This document defines the content of the FACT SHEET to be created for each identified tool, incentives, initiative or practice (the Building Block) introduced by public bodies or used by developers to expedite drug repurposing in Rare Diseases (RDs).

ITEM	DESCRIPTION
Building Block (BB) Title	**Initiative updating old labels – BPCA + PREA**
References	https://www.fda.gov/science-research/pediatrics/pediatric-labeling-changes https://www.fda.gov/media/122694/download https://www.accessdata.fda.gov/scripts/cderworld/index.cfm?action=newdrugs:main&unit=4&lesson=1&topic=7 https://www.nichd.nih.gov/research/supported/bpca https://www.federalregister.gov/documents/2019/04/23/2019-08167/best-pharmaceuticals-for-children-act-bpca-priority-list-of-needs-in-pediatric-therapeutics
Description	This building block describes the Best Pharmaceuticals for Children's Act (BPCA)/Pediatric Research Equity Act (PREA) legislative initiatives by which FDA drug labels can be updated (irrespective of original sponsor's willingness to engage).
Category	Regulatory and HTA engagement
Type of Building Block	Regulatory
Geographical scope	US

Availability	BPCA/PREA uses drug sponsors, NICHD/NIH, and the Pediatric Trials Network (PTN) to conduct the studies in collaboration with FDA. The program only apply to FDA-approved prescription drug labels.
Scope of use	- BPCA can be used in select settings when trying to update an FDA drug label for safety, efficacy, PK/PD, dosing, etc. for a drug used in a pediatric population.
Stakeholders involved	FDA, Sponsors (drug and biological product application holders), NIH (administers BPCA), Pediatric Trials Network (PTN) (conducts trials for BPCA), Pediatricians/Parents (BPCA)
Enablers/ Requirements	- Drug must be prescribed to pediatric populations/neonates - Drug must be off-patent (if BPCA off-patent clause is to be used) - The original sponsor/NDA holder has the right of first refusal to expand the label themselves. If they decline, and the drug is off-patent, NIH can decide to support someone else to conduct the studies to do it, and to serve as the sponsor to FDA or get the company to agree to submit the data package that was developed, typically by the Pediatric Trials Network (PTN) with NICHD support. - Funding is needed to conduct the studies, which are funded by NIH (but it is an unfunded congressional mandate, so the funding primarily comes from NICHD) and the trials are run primarily by the Pediatric Trials Network. - The FDA has to be in agreement with the trial design/data to be used/etc. - The group conducting the trial typically needs to be willing to serve as the sponsor or needs to be able to work with the original sponsor/NDA holder or another manufacturer to serve this role. - There needs to be access to the drug product still. - If sponsor replies to the Written Request themselves and is willing to conduct the study, they can receive 6 additional months of marketing exclusivity for their product. -
Output	BPCA:

	- New dosing, PK/PD, safety, or efficacy data added to FDA labels of drugs (including those that are off-patent) -
Best time to apply and time window	Can apply at any time; BPCA – multiyear process. For full approvals of entirely new indication in pediatrics, it has taken close to 10 years to get those approvals (and there have only been 2). The BPCA network goes through a rigorous prioritization process to identify disease/therapeutic areas of interest. Drugs or studies best positioned to meet those priorities are most encouraged to apply. BPCA engages with the FDA Review Divisions to determine what sort of trials would be necessary.
Expert tips	BPCA: Contact Perdita Taylor-Zapata, MD from NICHD about BPCA-related topics (taylorpe@mail.nih.gov) https://www.nichd.nih.gov/about/org/der/branches/opptb/taylor-zapata Interested parties can also participate in the BPCA prioritization process.