Inclusion of Pediatric Participants in Clinical Trials: Which Regulatory Process is Better, BPCA or NDA?

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Objective

To determine whether clinical trials (CTs) are inclusive of the pediatric population through a comparison of CT data for drugs approved under the Best Pharmaceuticals for Children Act (BPCA) versus drugs approved via New Drug Approvals (NDA).

Background

Key Pediatric Legislation and Policies

2002
Best Pharmaceuticals for Children Act (BPCA)
- Authorized use of Food and Drug Administration (FDA) to require manufacturers of pediatric CTs for previously approved drugs
- Manufacturer’s participation is voluntary
- Incentivized by extended marketing exclusivity

2003
Pediatric Research Equity Act (PREA)
- Provides FDA with authority to require pediatric studies of drugs and biologics reviewed by FDA

2012
Food and Drug Administration Safety and Innovation Act (FDASIA)
- Made BPCA and PREA legislations permanent

2016
Report to Congress: BPCA and PREA Status
- Identified that CTs and product development do not adequately address the health needs of neonates and pediatric cancers

2019
Report to Congress: Pediatric Labeling of Orphan Drugs
- Of 221 pediatric indications, 127 lacked or had no information for specific pediatric age groups

2021
Pediatric COVID-19 Vaccines
- Vaccines approved for those 12+ years old
- Pediatric studies ongoing completed by Pfizer (enrolled 12,000 subjects ages 12-18) and Moderna (Aiming to enroll ~3,000 subjects ages 12-18)

Methods

1. From FDA.gov, compiled list of drug approvals under BPCA from 2016 to 2018
2. From clinicaltrials.gov, categorized CT information related to BPCA drugs by study eligibility, Pediatric and Adult, Pediatric only, and Specific Pediatric Populations Only
3. From FDA.gov, compiled list of pediatric-related NDAs from 2016 to 2018
4. Collected from approved drug labels and Drug Trials Snapshots, Age data, Pediatric indications, and Pediatric studies
5. Calculated number of pediatric participants from age data and assessed for trends
6. Examined data for similarities and trends

Results

Findings for BPCA Drugs from 2016 to 2018

- 22 BPCA Drugs
- 196 Studies Conducted
- 18 BPCA Drugs with Approved Pediatric Indications
- Of 117 Relevant Studies Open to Pediatrics and Adults had less than <1% of pediatric participants

Findings for Pediatric-Related NDAs from 2016 to 2018

- 36 Pediatric-Related NDAs
- 25 of 31 NDAs mentioned clinical studies involving some number of pediatric participants
- 3 of 31 NDAs mentioned inclusion of studies with adult subjects to support drug safety for pediatrics
- 2 of 31 NDAs mentioned additional pediatric safety studies but no study details on label
- Of these 31 NDAs had 50% pediatric participants in one or more studies conducted

Conclusions

This comparison of pediatric-related NDAs and BPCA drugs from 2016 to 2018 demonstrates:

- A lack of standardization regarding which ages constitute a specific pediatric subpopulation
- An increasing trend of conducting studies, where both adults and pediatrics are eligible
- A lack of pediatric representation in BPCA studies open to both adults and pediatric
- A correlation between drugs initially seeking a pediatric indication via the NDA pathway (aka non-BPCA drugs) with higher pediatric subject enrollment and retention versus BPCA drug studies

Contact Information

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References


References