**Examination of FDA Pediatric Regulations: Inclusion of Pediatric Participants in Clinical Trials**

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**OBJECTIVE**
Assess the impact of the Best Pharmaceuticals for Children Act (BPCA)

**BACKGROUND**

- **2002** Best Pharmaceuticals for Children Act (BPCA)
  - Allowed FDA to send written requests to drug manufacturers, requiring pediatric clinical trials for previously approved drugs

- **2003** Pediatric Research Equity (PREA)
  - Provided FDA the authority to require pediatric studies in drugs and biologics currently undergoing review

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

- **2016** Report to Congress: BPCA and PREA Status
  - There remains a need for development of new clinical trial designs for small populations and better oral dosage forms

- **2019** Report to Congress: Pediatric Labeling of Orphan Drugs
  - Of 221 indications relevant to pediatrics, 127 lacked pediatric information or missing information for certain pediatric age ranges.

**METHODOLOGY**

- **Names of Drugs Approved under BPCA in 2016, 2017, and 2018**
- **Each drug was searched for in clinicaltrials.gov**
- **Studies were categorized as follows:**
  - Pediatric and Adult Eligible Studies
  - Pediatric Only Eligible Studies
  - Specific Pediatric Population Only Eligible Studies
- **Studies were examined for similarities and trends.**

**FINDINGS**

**11 Drugs Approved in 2017**

- **n = 38 total studies eligible for adult and pediatrics**
- **Trials with <1% Pediatric Representation**
  - n = 26 trials

**13 Drugs Approved in 2018**

- **n = 89 total studies eligible for adult and pediatrics**
- **Trials with <1% Pediatric Representation**
  - n = 54 trials

**CONCLUSIONS AND IMPLICATIONS**

For the 26 drugs approved under the BPCA from 2016 to 2018 where both pediatric and adult populations were eligible, 154 studies were conducted. 19 studies (12.3%) were excluded due to ambiguous data presentation. Examining the remaining 135 studies revealed:

- There is a lack of standardization regarding which ages constitute a specific pediatric sub-population although guidelines exist (Table 1, Figure 2).
- There is a lack of pediatric representation in clinical trials involving both adult and pediatric population (Figures 3-5).
- These findings are consistent with 2019 Report to Congress (Figure 1).

Pediatric drug labeling guides doctors, other healthcare providers, and caretakers of children on how to use a drug. Better pediatric representation in clinical trials will provide further safety, efficacy, and dosage information for pediatric labeling.

**CONTACT INFO**

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**Table 1. Pediatric Age Breakdown from CDER**

<table>
<thead>
<tr>
<th>Age Group</th>
<th>Approximate Age Ranges</th>
</tr>
</thead>
<tbody>
<tr>
<td>Neonates</td>
<td>Birth to &lt;1 Month</td>
</tr>
<tr>
<td>Infants</td>
<td>1 Month to &lt;2 Years</td>
</tr>
<tr>
<td>Children</td>
<td>2 to &lt;12 Years</td>
</tr>
<tr>
<td>Adolescents</td>
<td>12 to &lt;17 Years</td>
</tr>
</tbody>
</table>

**Example of Drug Approved under BPCA in 2016: Studies Breakdown**

- **Drug: Acetaminophen**
- **n = 7 studies eligible for adult and pediatrics**
  - **Trials (Combined %)**
    - 57.1% n = 4 trials
  - Of these trials, n = 0 had <1% pediatric representation.

- **n = 8 total studies eligible for adult and pediatrics**
  - **Trials with <1% Pediatric Representation**
    - 25% n = 2 trials

**Figure 1. Timeline of Key Pediatric Laws, Regulations, and Reports**

**Figure 2. Example of Drug Approved under BPCA in 2016: Studies Breakdown**

**Figure 3. Pediatric Representation in Drugs Approved under BPCA in 2016**

**Figure 4. Pediatric Representation in Drugs Approved under BPCA in 2017**

**Figure 5. Pediatric Representation in Drugs Approved under BPCA in 2018**