OBJECTIVE
To assess the extent that the Best Pharmaceuticals for Children Act (BPCA) encourages pediatric inclusions in clinical trials (CTs) and availability of pediatric information.

BACKGROUND
Best Pharmaceuticals for Children Act (BPCA)
Legislation passed in 2002 that permitted the U.S. Food and Drug Administration (FDA) to request manufacturers to conduct pediatric CTs for previously approved drugs.
- Manufacturer’s participation is voluntary, not mandatory.
- Incentivized by additional marketing exclusivity.

Pediatric Research Equity Act (PREA)
Legislation passed in 2003 that provided FDA with authority to require pediatric studies in drugs and biologics currently undergoing review.

Food and Drug Administration Safety and Innovation Act (FDASIA)
Identified the need for the development of CT designs for small populations and better oral dosage forms.

2016 Report to Congress: BPCA and PREA Status
Identified the need for the development of CT designs for small populations and better oral dosage forms.

2019 Report to Congress: Pediatric Labeling of Orphan Drugs
Out of 221 pediatric indications, 127 lacked pediatric information or missing information for specific pediatric age groups.

Pediatric COVID-19 Vaccine Studies as of Feb. 2021
- Pfizer has enrolled > 2,000 participants ages 12 to 15.
- Moderna is currently recruiting ages 12 to 18, aims to enroll approximately 3,000 participants.

METHODS

1. BPCA Drugs
   - Searched Clinicaltrials.gov for CTs relating to drugs approved under BPCA in 2016 to 2018 period.
   - Categorized by study eligibility:
     - Pediatric and Adult
     - Pediatric Only
     - Specific Pediatric Populations Only
   - Calculated number of pediatric participants from age data and assessed for trends.

2. Non-BPCA Drugs
   - Searched FDA.gov for New Drug Applications (NDA) in 2016 to 2018 period.
   - Collected from approved drug labels and Drug Trials Snapshots.
   - Examined data for similarities and trends.

FINDINGS

Eligibility Criteria for BPCA Drugs Studies (2016-2018)

<table>
<thead>
<tr>
<th>Year</th>
<th>Both Groups</th>
<th>Pediatric Only</th>
<th>Sub-Populations</th>
</tr>
</thead>
<tbody>
<tr>
<td>2016</td>
<td>8 Studies</td>
<td>0 Studies</td>
<td>16 Studies</td>
</tr>
<tr>
<td>2017</td>
<td>37 Studies</td>
<td>0 Studies</td>
<td>27 Studies</td>
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<tr>
<td>2018</td>
<td>75 Studies</td>
<td>10 Studies</td>
<td>7 Studies</td>
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</tbody>
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54% of studies open to adults and pediatrics had <1% of pediatric representation (n=120).

Pediatrics in Non-BPCA Drugs Studies (2016-2018)

- 4 of 6 drugs in 2016 had ≥ 50% of pediatric subjects in ≥ 1 studies conducted.
- 6 of 8 drugs in 2017 had ≥ 50% of pediatric subjects in ≥ 1 studies conducted.
- 8 of 17 drugs in 2018 had ≥ 50% of pediatric subjects in ≥ 1 studies conducted.

CONCLUSIONS
CT data for BPCA and non-BPCA drugs demonstrates:
- Low levels of pediatric subjects for studies completed under the BPCA, especially those open to adults and pediatrics.
- An increasing trend of conducting CTs, where adults and pediatrics are eligible, compared to pediatric only and pediatric subpopulations for both BPCA and non-BPCA drugs.
- A correlation between drugs initially seeking a pediatric indication via the NDA pathway (aka non-BPCA drugs) with higher pediatric subject enrollment and retention vs. BPCA drug studies.