REGULATORY SCIENCE INITIATIVES SPECIFIC TO GENERIC DRUGS

OFF-LABEL USE OF GENERIC DRUGS IN PEDIATRIC POPULATIONS

Ilene Harris, PharmD, PhD, IMPAQ International, LLC
Zippora Kiptanui, Bpharm, MPH, IMPAQ International, LLC
Paul Dowell, PharmD, PhD, IMPAQ International, LLC
Jingjing Qian, PhD, Auburn University
OFF-LABEL USE OF GENERIC DRUGS IN PEDIATRIC POPULATIONS

OUTLINE

▸ Background
▸ Research Priorities
▸ Recommended Methods
▸ IMPAQ International’s Relevant Experience
BACKGROUND

- Many drugs commonly prescribed for children are not labeled for use in the pediatric population
  - 80% of hospitalized children have reported off-label drug use
  - 25% of children in a pediatric emergency department have reported off-label use
- Off-label drug use in children generally accepted as standard medical practice, but some evidence of increased risk of adverse drug events (ADEs)
- Generic drugs may be used off-label for indications that are carved out relative to the reference listed drug

OFF-LABEL USE OF GENERIC DRUGS IN PEDIATRIC POPULATIONS

PROPOSED RESEARCH PRIORITIES

▸ Determine which generic drugs, clinical specialties, and ADEs are most prevalent with off-label drug use in children

▸ Determine the information sources used by healthcare providers as clinical guidance when using generic drugs off-label in pediatric populations

Provide insight into off-label generic drug use in children, thereby improving the FDA’s ability to assess post-marketing use and safety in pediatric populations
RECOMMENDED METHODS FOR ANSWERING RESEARCH QUESTIONS

▸ Priority 1: Determine which generic drugs, clinical specialties, and ADEs are most prevalent with off-label drug use in children

▸ Mixed methods design: Qualitative
  ▸ Literature review and environmental scan to identify the most prevalent drugs used off-label in children
  ▸ Drug label review to confirm that the drugs identified are indeed off-label per the FDA-approved label
RECOMMENDED METHODS FOR ANSWERING RESEARCH QUESTIONS

- Priority 1: Determine which generic drugs, clinical specialties, and ADEs are most prevalent with off-label drug use in children

  - Mixed methods design: Quantitative
    - Medication utilization analysis using administrative claims
      - Estimate use by age ranges (infants, children, and adolescents)
      - ADEs for off-label medication use
        - FDA’s Adverse Event Reporting System (FAERS) database
RECOMMENDED METHODS FOR ANSWERING RESEARCH QUESTIONS

▸ Priority 2: Determine the information sources used by healthcare providers as clinical guidance when using generic drugs off-label in pediatric populations

▸ Qualitative

▸ Environmental scan of sources of information used by healthcare providers

▸ Key informant interviews to determine how clinicians across settings obtain information for off-label prescribing
IMPAQ’S COLLABORATION WITH FDA

- Generic Drug Substitution in Special Populations (U01FD005875)
- Educating Groups Influencing Generic Drug Use (U01FD005486)
- Post-Marketing Surveillance of Generic Drugs (U01FD004855)
- Evaluation, Development, and Utilization of Statistical and Mathematical Methods IDIQ (HHSF223201510023I)
OFF-LABEL USE OF GENERIC DRUGS IN PEDIATRIC POPULATIONS

IMPAQ’S PRIOR RESEARCH EXPERIENCE

- Expertise in pharmaceutical health services research, especially with regard to generic drugs
- Published research using the proposed analysis techniques: literature review, environmental scan, drug label review, FAERS database analysis, and claims analysis and integrating results