

# Establishing a national priority list of pediatric drugs in Canada : A case for close collaboration with regulators

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## Background

In 2020, Health Canada (HC) developed the Pediatric drug action plan (PDAP) to address the many barriers to accessing medicines for children with the ultimate vision that children and youth in Canada be consistently treated with the medicines they need in age-appropriate formulations.

## Objective

To develop a National Priority List of Pediatric Drugs (NPLPD) for Canada



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## Methods

### 1. Establishment of a pediatric external reference group (PERG)

- For guidance and recommendations to HC
- Comprised of 10 pediatricians and pharmacists from across Canada, and two observers from Health Technology Assessment (HTA) organizations

### 2. Nomination and screening process

- 5-question form on a HC-hosted website opened for 60 days (March to May 2023)
- Pediatric healthcare community invited to nominate drugs that address diseases/ disease areas with high unmet medical need(s) and/or drugs lacking commercial child-friendly formulations
- Drug product eligibility criteria
- The nominated drug product lacks:
  - Approval for sale in Canada OR
  - A pediatric indication AND/OR
  - A child-friendly formulation
- The nominated drug is approved for sale in a trusted foreign jurisdiction with an established pediatric indication and/or child-friendly formulation
- Screening process by HC and GPFC
- Four (4) drug categories identified (Figure 1)

### 3. Prioritization of eligible drugs

#### Objective criteria

- Separately defined for each drug category
- Certain criteria applied to all categories, while others were category-specific
- Each scored using a binary scoring system as follows: yes=1 / no=0
- Calculation of objective score:
  - Sum of scores per category = maximum score
  - New drug=4, new indication=5, new formulation=12, new formulation and indication=12.
  - Maximum score per category (%) calculated using category-specific denominator: for example for a new formulation a drug scoring of  $8/12 = 67\%$

#### Subjective criteria:

- Drugs in each therapeutic area (TA) ranked by pharmacists and subspecialists based on perceived priority/urgency of access:
  - Extremely urgent=3 (score=100%);
  - Very urgent=2 (score=67%)
  - Urgent=1 (score=33%).
- Top 2 priority drugs per TA were identified by both groups.

#### Final weighted total score

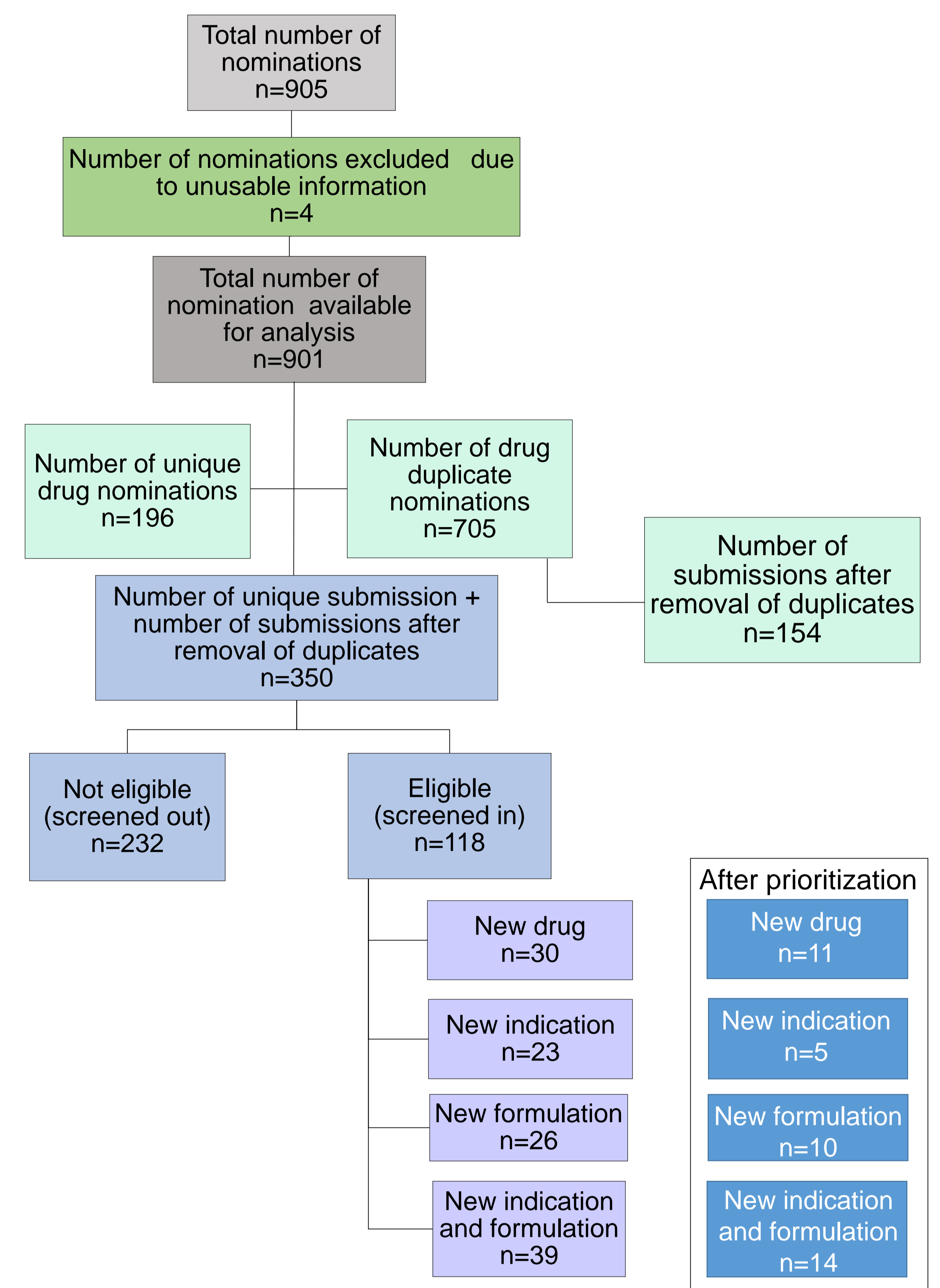
- = 40% objective score + 30% subspecialist subjective score + 30% pharmacist subjective score

## Selection of top 40 drugs via sequential approach

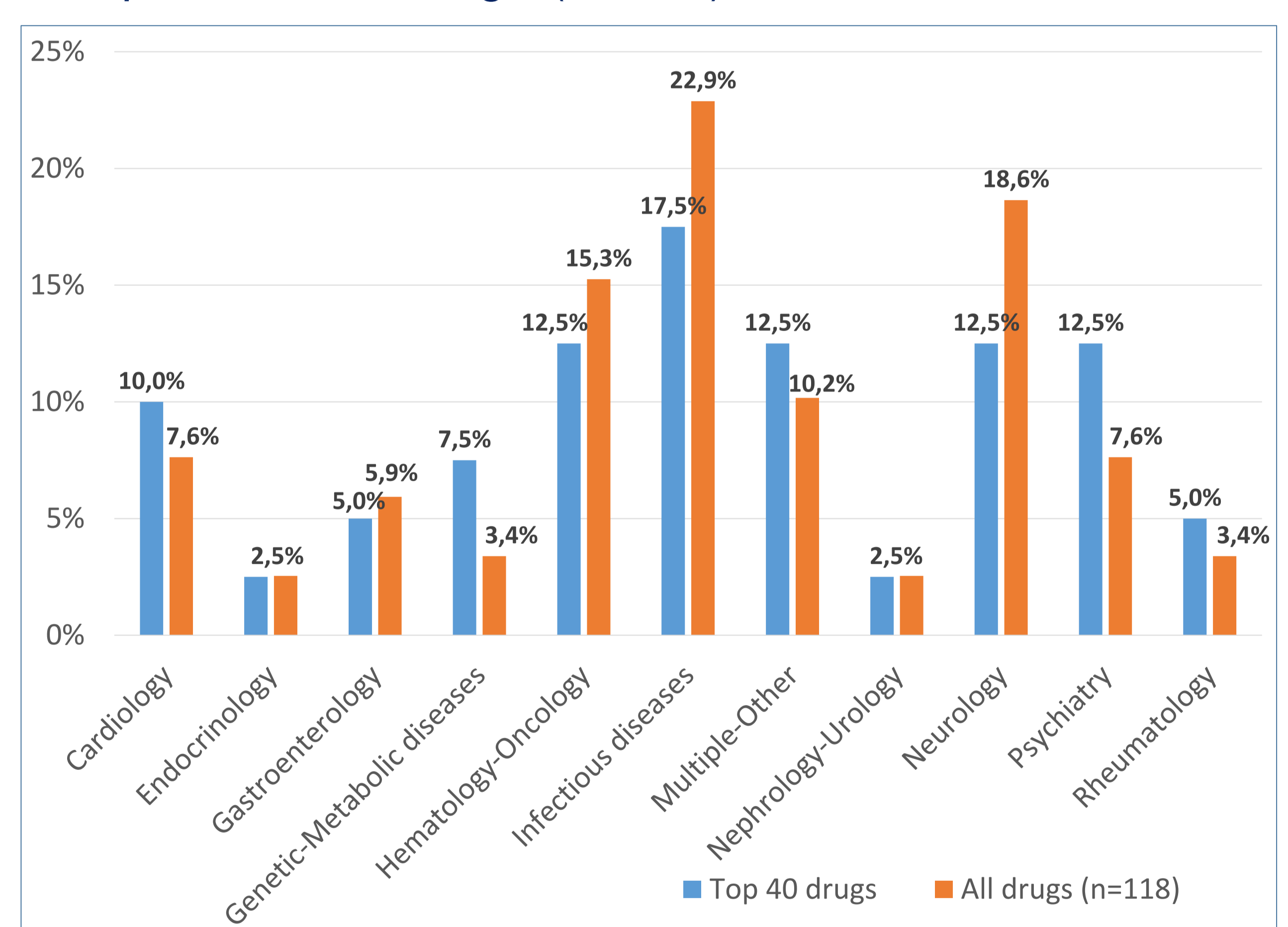
- Step 1: Drugs selected as top priorities in each TA by subspecialists and pharmacists (n=32) were automatically included on the draft list of 40 drugs.
- Step 2: The remaining 8/40 included drugs were selected based exclusively on final combined weighted total score irrespective of TA.

## Results

Figure 1: NPLPD screening process flowchart



Graph 1: Percentage of drugs by TA for top 40 drugs compared to all drugs (n=118)



## Conclusion

The NPLPD initiative—the first of its scale in Canada—represents the culmination of years of dedicated effort and direct collaboration between the government of Canada and the pediatric medical community, a truly exceptional partnership for a mid-sized pharmaceutical market such as Canada. The list is currently undergoing review by HC. Following this review, HC will lead a public as well as targeted consultations on the list with industry, provincial and territorial governments, and Health Technology Assessment organizations in fall of 2024.