Willingness to Pay More: A Review of Paediatric Medicines Assessed Between 2018 and 2023

Poster No. HTA314

Karinou E, PhD[1]; Beugre-Guyot M, MSc[1];Bungey G, MSc[1]; Vilu HL, MBiochem[1]

[1]Evidera Inc., a business unit of PPD, part of Thermo Fisher Scientific, London, UK

Background

- Health economic evaluations for paediatric diseases are often characterised by high uncertainty, in part due to small study sizes, and the lack of long-term clinical effectiveness data, age specific utility values and accurate indirect cost.[1]
- The National Institute for Health and Care Excellence (NICE) in England and Wales is responsible for the fair and equal distribution of healthcare resources within society and may take into consideration factors other than relative costs and benefits.[2]
- NICE provides little guidance around the methods for generating paediatric-specific data in cost-effectiveness analyses, particularly paediatric-specific health state utility values.[2,3]

Objectives

- This study reviewed the evidence submitted to NICE for paediatric indications between 2018 and 2023, and how this evidence was received by the committee.
- Factors that contributed towards a positive outcome for the examined health technology assessment (HTA) submissions were identified.

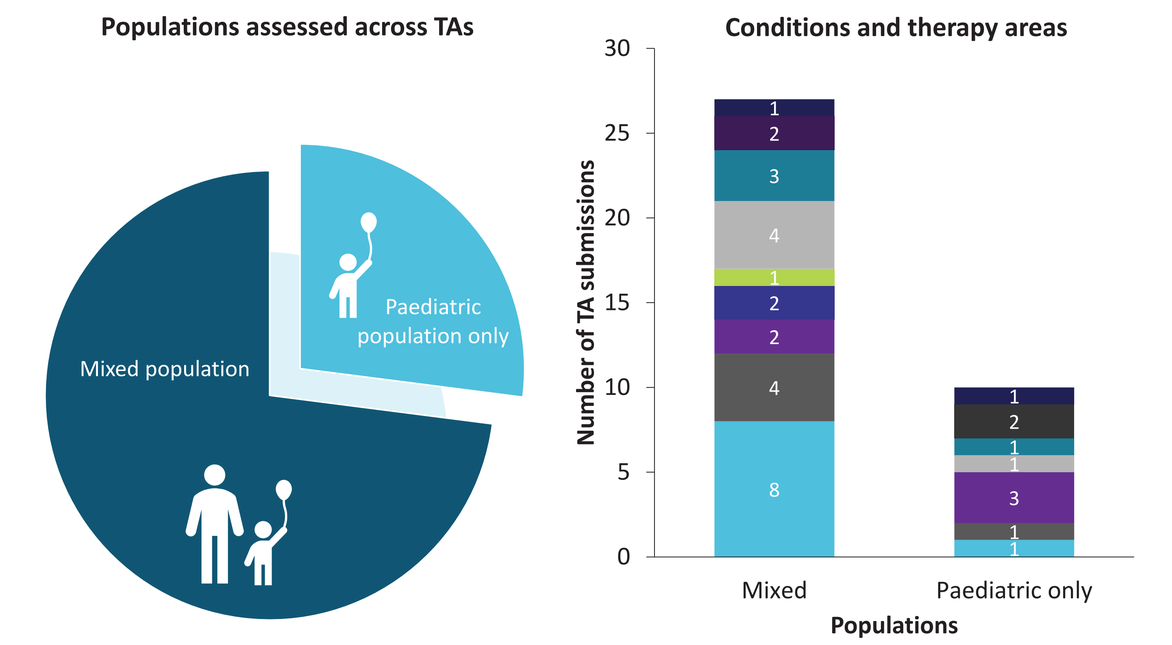
Methods

- Publicly available technology appraisal (TA) documents were reviewed, including highly specialised technology (HST) evaluations, for medicines with indications for paediatric populations (<18 years of age) assessed by NICE between June 2018 and June 2023.
- The included TA documents were reviewed with regards to submission type (single or multiple TA, HST), therapy area, population, evidence submitted, incremental cost-effectiveness ratio (ICER), health-related quality of life (HRQoL) tool used, assessment outcome, and committee comments on the evaluation and decision outcomes.

Results

- A total of 368 TAs published by NICE between June 2018 and 2023 were screened for paediatric indications.
- Thirty-seven (10%) submissions included medicines indicated for infants, children and/or adolescents and were selected for review: 2 (5.4%) multiple TA, 30 (81.0%) single TA and 5 (13.5%) HST evaluations.
- The majority (27/37; 72.9%) of the TAs were for medicines indicated for mixed populations including both adult and paediatric patients, with 27% (10/37) of TAs in exclusively paediatric indications (**Figure 1**), including all five HST evaluations.
- Blood and immune system conditions (9/37; 24.3%), cancer (5/37; 12.5%), musculoskeletal conditions (5/37; 12.5%), and diabetes and other endocrinal, nutritional and metabolic conditions (5/37; 12.5%) were the most common therapy areas assessed (**Figure 1**).

Figure 1. TAs in paediatric indications published by NICE between 2018 and 2023



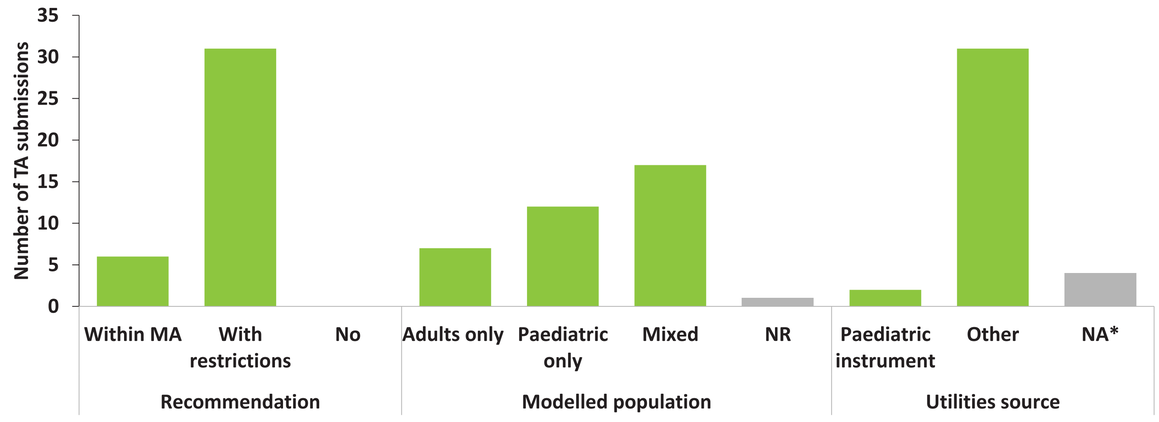
IS = immune system
* Diabetes and other endocrinal, nutritional and metabolic conditions

- All 37 (100%) TAs were recommended for reimbursement by NICE (**Figure 2**).
- Six TAs (6/37; 16.2%) were approved within their marketing authorisation (MA), while the remaining 31 (83.7%) were recommended with restrictions.
- Two (2/37; 5.4%) TAs had a commercial access agreement, seven (7/37; 18.9%) TAs had a managed access agreement and 25 (25/37; 67.5%) TAs had a simple discount patient access scheme.
- Twenty-one (21/37; 56.8%) TAs had restrictions on the population compared with their MA.
- Utility values were derived from clinical trial data (12/37; 32.4%), vignette studies (12/37; 32.4%), observational studies (8/37; 21.6%) and discrete choice experiments (1/37; 2.7%).
- Two TAs (2/37; 5.4%) used trial data directly measuring HRQoL in children to generate utilities using the EQ-5D-Y and PedsQL instruments, respectively; however, the mapping algorithms used in both cases resulted in adult tariffs.

Results (Cont'd)

- The majority of TAs (24/37; 64.8%) used data from mixed or adult-only populations in their economic models, with 12 (32.4%) using paediatric-specific inputs.

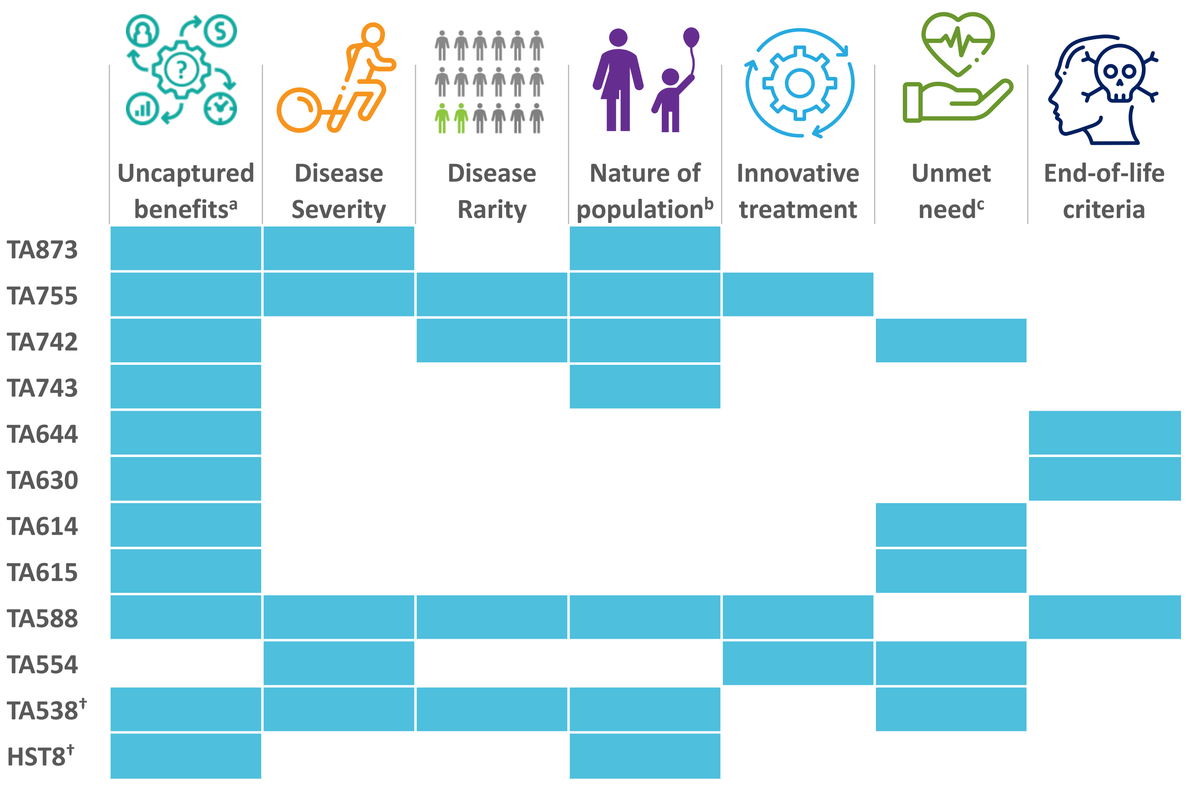
Figure 2. Lack of data specific to paediatrics



NA = not applicable; NR = not reported
* Cost-comparison analyses

- Although all 37 TAs with paediatric indications were recommended by the committee for reimbursement, 12 of them (32.4%) were not considered an acceptable use of National Health Service (NHS) resources, with ICERs above the NICE willingness-to-pay (WTP) threshold.
  - Common issues identified by the committee were: uncertainties due to inappropriate model assumptions, immature or insufficient clinical data, lack of appropriate utilities for patients and caregivers, and unclear technology implementation costs.
  - The committee took into consideration additional factors for their final decision making, outside relative costs and benefits, which resulted in recommendation for reimbursement for these medicines (**Figure 3**).

Figure 3. Additional factors considered by NICE in decision-making, contributing to recommendation of health technologies with ICER above WTP threshold



[a] Uncaptured benefits included health-related and non-health-related benefits such as benefits to NHS, benefits of treatment to carers and patient's family, children's school attendance and their work attendance as adults;
[b] Nature of population includes children and young people, people facing inequalities (e.g., learning difficulties), hereditary diseases and diseases disproportionally affecting people of certain ethnic backgrounds;
[c] Unmet need due to lack of treatment or current treatments lacking efficacy or having negative HRQoL impact
† Evaluations for paediatric indication only.

Conclusions

- All 37 NICE TAs for paediatric indications in the past 5 years resulted in a recommendation for reimbursement, regardless of whether paediatric evidence was included in the economic model.
- A third (12/37) of the treatments assessed exceeded the NICE WTP threshold but were still recommended.
- High uncertainty regarding the cost effectiveness of paediatric treatments is often mitigated by factors such as rarity and severity of disease, and additional benefits like impact on carers or school attendance, which are not easily captured by health economic evaluations.

References

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