

Enpr-EMA off-label working group

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Update

- Paper on use of existing data
- Level of evidence of off-label drug use
- Next steps

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Paper on use of existing data

Off-label is not always off-evidence:

**Proposal to include more paediatric data in the medicine labels,
a proposition by the off-label evidence working group of the European Network of
Paediatric Research at the European Medicines Agency (Enpr-EMA)**

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Status: pre-final, ready to submit for publication

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Project Dutch Pediatric Formulary

> Clin Pharmacol Ther. 2022 Sep 7. doi: 10.1002/cpt.2736. Online ahead of print.

Off-Label, but on-Evidence? A Review of the Level of Evidence for Pediatric Pharmacotherapy

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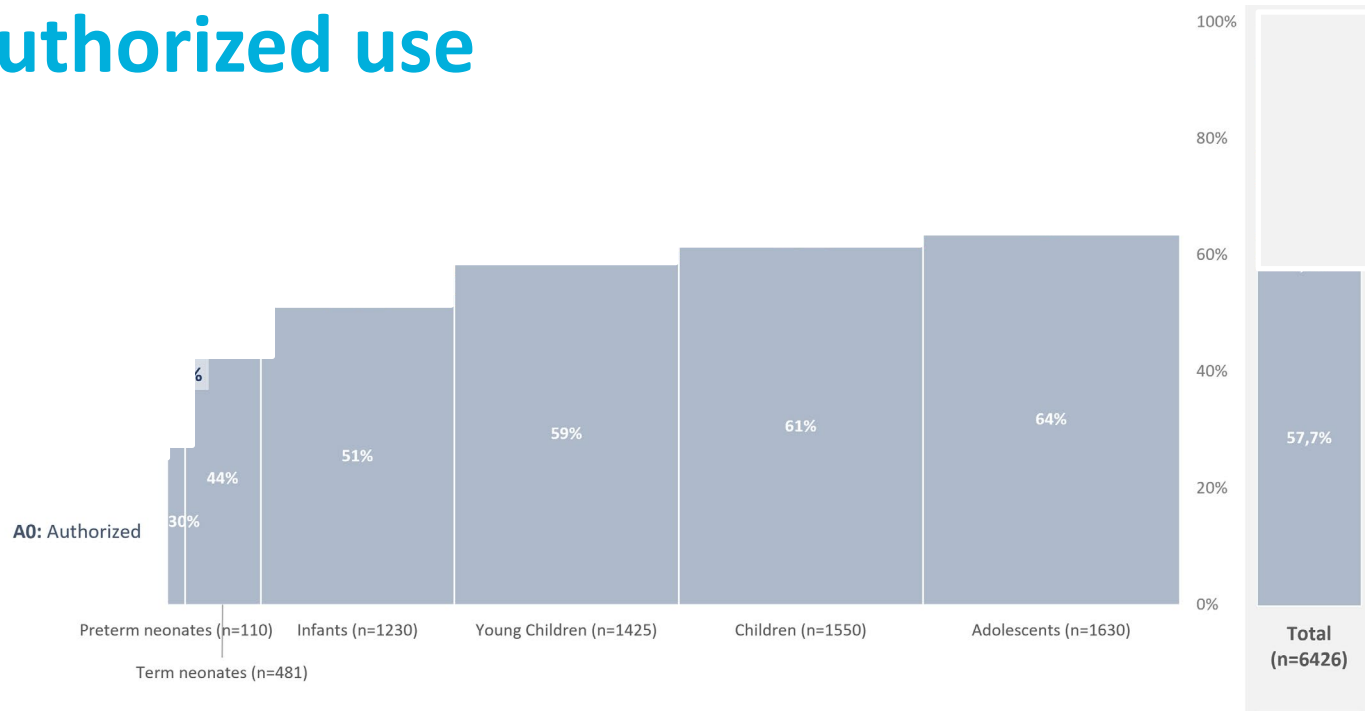
Affiliations + expand

PMID: 36069288 DOI: 10.1002/cpt.2736

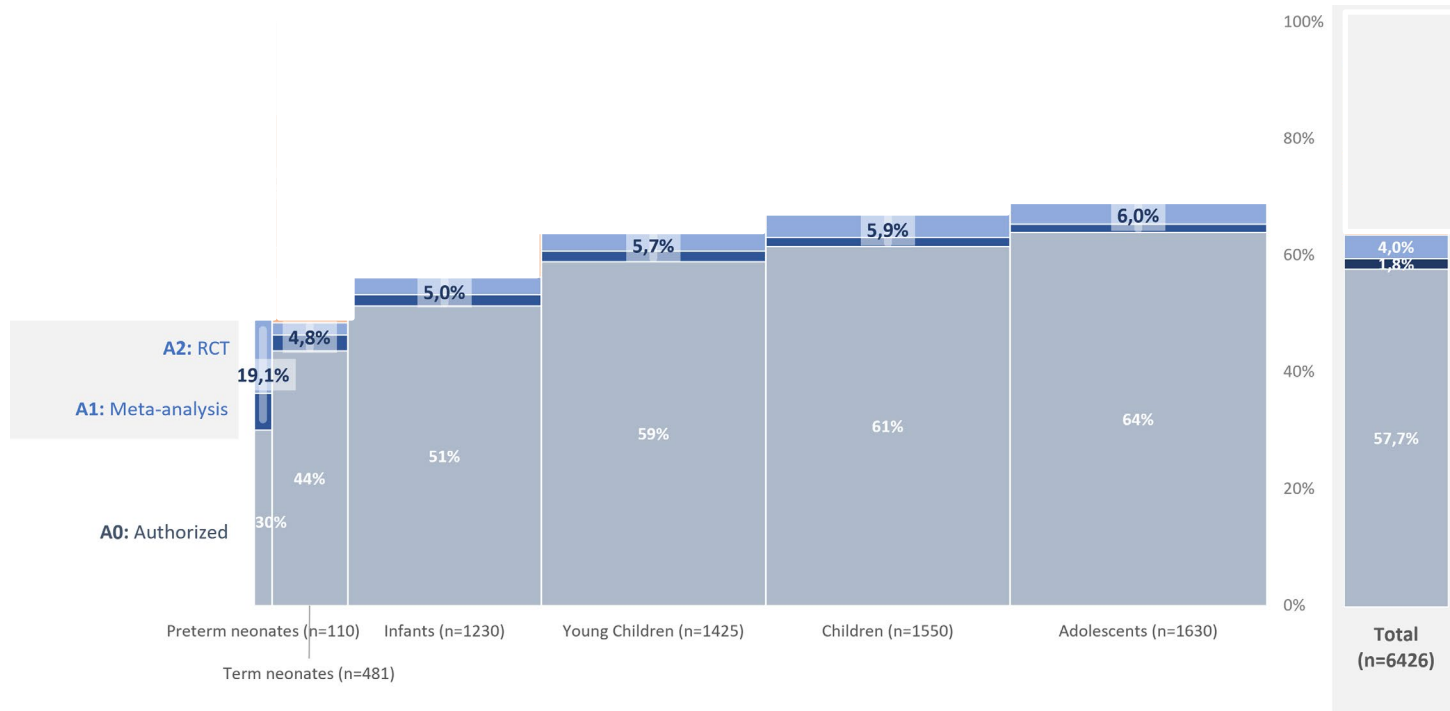
For every drug-indication-age group in the formulary (800 drugs),
highest level of evidence assessed

Highest level of evidence per drug

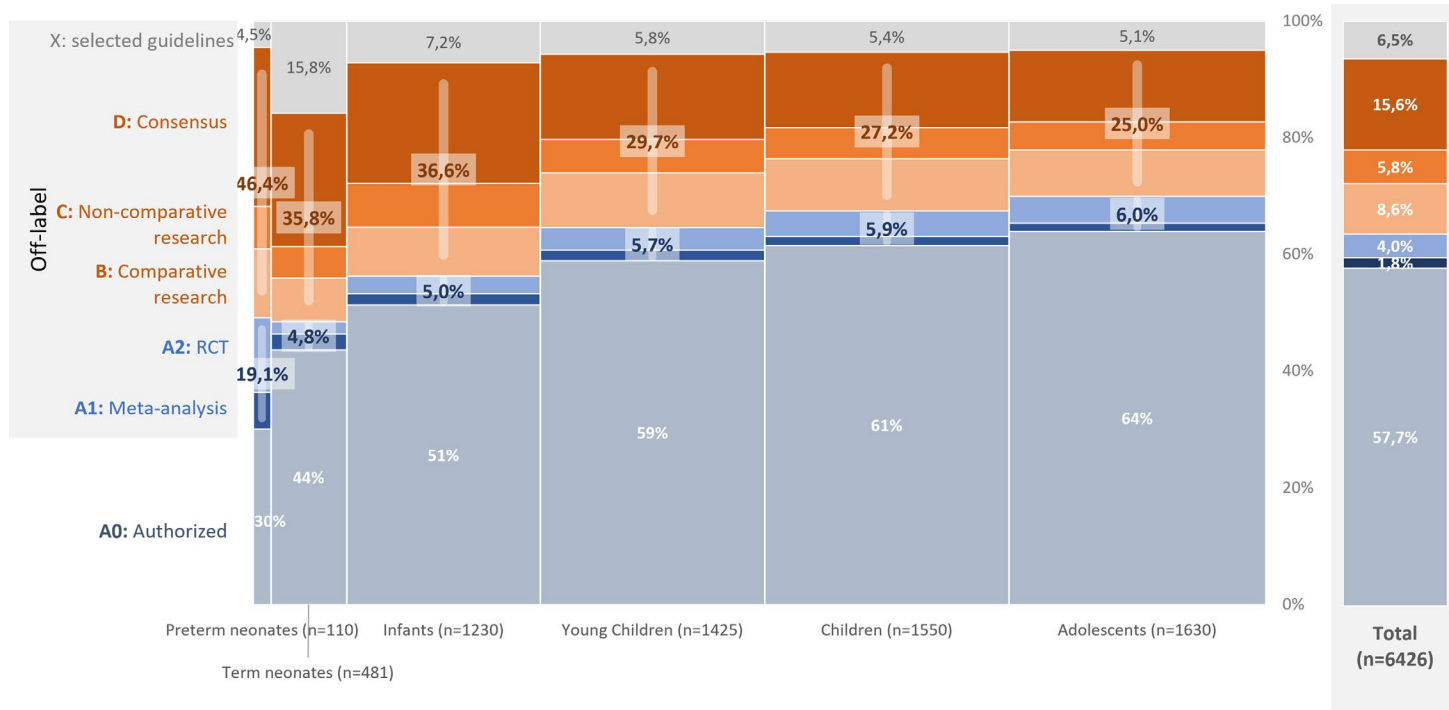
Authorized use



Off-label: High quality evidence



Off-label: Low quality evidence



Conclusions

- First comprehensive overview of the quality of available evidence supporting off-label drug use in the paediatric population
- Only 14% of off-label records supported by high quality studies.
- This puts children at an increased risk of therapy failure and toxicity.

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• Next steps?

1. Continue efforts to include high level of evidence data in label?

2. Prioritize need for evidence

Which off-label, medium-low level of evidence drugs need more research?

based on: Burden of disease, epidemiology, risk of failure, risk of toxicity?

Which studies are needed?

RTCs, other trial designs, RWD studies, extrapolation with modeling and simulation?

Who: International pediatric community with regulators, patients/parents and industry