Access to new paediatric medicines in the Nordic countries

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since the implementation of the Regulation, from 2007 until 2016, 267 new medicines for children were authorised in the EU. Do we have these products available for children in Nordic countries? (DK, FI, NO, SE)
What we did

Table 1. The source data for this study, from the Annex of the 10-year report to the EC (EMA/35987/2016), listing new authorised medicines (Annex ‘chapter 1’).

<table>
<thead>
<tr>
<th>Annex table number*</th>
<th>Tables in the Annex of EMA’s 10-year report</th>
<th>Number of medicinal products listed</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>New medicines (CAPs, initial MAs, including a paediatric indication (product group A))†</td>
<td>82</td>
</tr>
<tr>
<td>3</td>
<td>New pharmaceutical forms (or routes of administration) of paediatric relevance (CAPs, line extensions of existing MAs) (product group B)</td>
<td>27</td>
</tr>
<tr>
<td>6</td>
<td>New pharmaceutical forms (or routes of administration) of paediatric relevance (NAPs, line extensions of existing MAs) (product group B)</td>
<td>16</td>
</tr>
</tbody>
</table>
What we did

Pr 1Q 2019, based on data from individual NCA’s databases:

- Marketing authorisation status?
- - and marketed?

**Having a MA + being marketed as a ‘proxy’ of availability**

- If not marketed: has it ever been marketed?

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**Figure 1** Flow chart illustrating how marketing status was assessed for the individual products. MA, marketing authorisation.
Main results

- 21%–32% (16/76–24/76) of the new medicines initially authorised for children between 2007 and 2016 were not marketed across the four Nordic countries
- 29%–50% (16/56–28/56) of the new paediatric formulations were not marketed and a significant proportion had never been marketed
New medicines (first time MA) for children

- 21%–32% (16/76–24/76) of new medicines initially authorised for children not marketed
- Minor variations between countries
- Therapeutic areas:
  - Variations between nations
  - Nearly half of the antineoplastic and immunosuppressive agents were not marketed in any of the Nordic countries
- 6 products withdrawn

Figure 2 Number of new medicinal products marketed (or not) in DK, FI, NO and SE. DK, Denmark; FI, Finland; NO, Norway; SE, Sweden.
New formulations (forms/strengths)

- 29%–50% (16/56–28/56) of the new formulations were not marketed.
- Vast majority of them had never been marketed.

**Figure 3** Marketing status for new formulations of medicinal products (whether the product still had MA and was still marketed) per country (DK, FI, NO and SE). CAP, centrally authorised product; DK, Denmark; FI, Finland; MA, marketing authorisation; NAP, nationally authorised product; NO, Norway; SE, Sweden.
New formulations: Some observations

• The formulations not marketed in any country: often the paediatric specific ones, e.g. the lower strength formulations (5/14), the oral liquid/powder/granules (5/14) and the chewable tablets (2/14)

• Approximately half of the products marketed in all countries were products for which the new formulation seemed to have replaced the old one (eg, prefilled syringe replacing vials, tablets replacing capsules and ‘ready-to-use solution for injection’ replacing ‘powder and solvent for solution for injection, i.e. overall product strategies, not necessarily paed specific
PIP requirements on formulation – not a driver for availability (in the Nordic area)

• 35/56 of the reviewed new formulations represented products with agreed-upon PIPs where the specific formulation was part of the PIP obligations

• Only 6 of these 35 formulations were marketed in all countries

• Of the 14 formulations not marketed in any of the countries, nine were listed as specific requirements in the PIP

• In contrast, the majority (10/16) of the formulations available in all countries did not have a PIP / were not specific PIP formulation requirements
What we did not do.....

- Price and reimbursement status
- Reasons for ‘non-marketing’
- Availability regardless of marketing status
- ‘Clinical impact’ assessment
Conclusion?

• Majority of new medicines with paed indication (centrally authorised) were marketed in the Nordic countries
  
  • 21%–32% (16/76–24/76) of the new medicines initially authorised for children between 2007 and 2016 were not marketed across the four Nordic countries

• New forms/strengths – both for CAPs and NAPs – are more prone to not be marketed – particular concern re ‘lower age formulations’
  
  • 29%–50% (16/56–28/56) of the new paediatric formulations were not marketed and a significant proportion had never been marketed

• Despite the intentions of the EU’s Paediatric Regulation, medicines targeted at children are not all marketed, risking limitations in availability and accessibility for patients
Does the EU’s Paediatric Regulation work for new medicines for children in Denmark, Finland, Norway and Sweden? A cross-sectional study


https://bmjpaedsopen.bmj.com/content/bmjpo/4/1/e000880.full.pdf
NOW who do we blame?

Pharma Industry
Regulatory requirements
Paediatricians
Pricing system
HTA principles
Wholesalers/supply org

https://www.amazon.com/NOW-Who-Do-Blame-Political-ebook/dp/B005D720ZI
Our ‘regulatory tool box’ in NO

- Fast and efficient regulatory handling; recognise assessments from other MSs
- Promote and enable multilingual packages (within or outside Nordic area)
- Agree higher maximum price than normally accepted
- Fee waiver/reduction at time of MAA or at later procedures
- Particular attention to withdrawal notifications for products particularly relevant for children => discussion with MAH on potential continued marketing
- Public notification of products in need (list of calls)
- Multistakeholder working group (NoMA/Industry/Paediatric community)
- Direct requests and dialogue with industry on relevant products

But... 😊
Can we have more paediatric medicines/formulations/strengths into the Nordic market if we collaborate more closely?

Medicines for children in the Nordic Area - closer collaboration?
Project under Nordic Council of Ministers (NMR) - 2022

https://projektdb.norden.org/details/0e5b3a6c-f7bc-415a-9ea0-78fa321fe268

• Can something be gained by closer Nordic collaboration within meds authorities?
• Sharing experiences?
• Potential ‘joint policies/strategies’?

Aim: Explore the possibilities for closer Nordic collaboration to have more authorised products marketed and available for all children in the Nordic area
**Project elements**

- **Survey** of current regulatory ‘status’: current practices, possibilities, framework/regulations, in the Nordic countries / NCAs

- Arrange a **Workshop 20.- 21. oktober 2022**, and based on the survey:
  - Share experiences and practices
  - Identify possible areas of collaboration
  - Identify any limitations (regulatory) at national (or EU?) level
  - Specify any potential further collaboration, if relevant
  - Regulatory discussions mainly – input from paediatricians, industry (EFPIA/MfE), public health
Paediatric formulations are being developed

Paediatric formulations are being authorised

Paediatric formulations are being marketed

Paediatric formulations are being kept on the market
From Little Acorns: A Scandinavian Study’s Implications for Paediatric Development

In December 2020, a paper was published in the journal BMJ Paediatrics Open, a respected journal on child health. The study described the status of new paediatric medicines in Scandinavia (Norway, Sweden, Finland and Denmark). The study described that regulatory obligations in addition to national pricing and reimbursement systems may impact such decisions, together postulate that regulatory obligations in addition to national pricing and reimbursement systems may impact such decisions, together.

The study described in the article contained extremely informative data, so enlightening that it could—and should—trigger a wide-reaching review of our approach to the development of and access to new paediatric medicines. Lepola and her colleagues examined the marketing

- 21%–32% (65/304–24/76) of the new medicines were not marketed,
- Of the new formulations relevant to children, 29%–50% (65/66–26/50) were not marketed,
- A significant proportion of these products had never been marketed.

The authors conclude that similar data from other countries are needed to evaluate the overall European status of marketed paediatric approvals. How true!
Thank you for your attention!