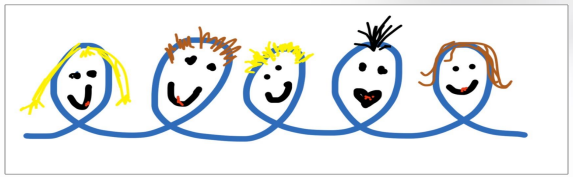
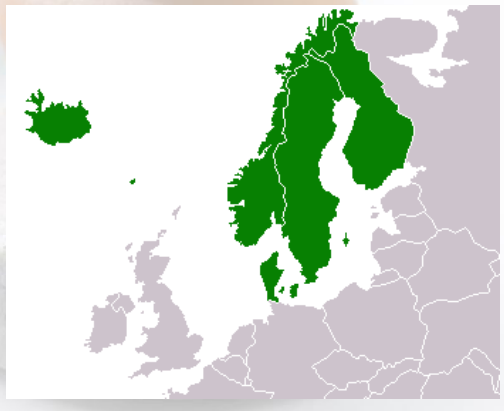
Access to new paediatric medicines in the Nordic countries

4th Nordic Conference on Paediatric Medicines, Helsinki, Sep22

Siri Wang
Scientific Director, NoMA, Norway





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RESS REPORT ON 10 YEARS OF EU
EDIATRIC REGULATION

edicines for children were
ween 2007 and 2016.

eu/health/human-use/paediatric-medicines_en

??

Do we have these products available for children in Nordic countries??

(DK, FI, NO, SE)

«- since the implementation o… …edicines for use in children and 43 new pharmaceutical forms appr… …the EU.»

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What we did

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

15 November 2016
EMA/35987/2016

Annex
10-year Report to the European Commission

Table of content

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)[4]

Annex table number*	Tables in the Annex of EMA'S 10-year report	Number of medicinal products listed
1	New medicines (CAPs, initial MAs, including a paediatric indication (product group A)†	82
3	New pharmaceutical forms (or routes of administration) of paediatric relevance (CAPs, line extensions of existing MAs) (product group B)	27
6	New pharmaceutical forms (or routes of administration) of paediatric relevance (NAPs, line extensions of existing MAs) (product group B)	16

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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?

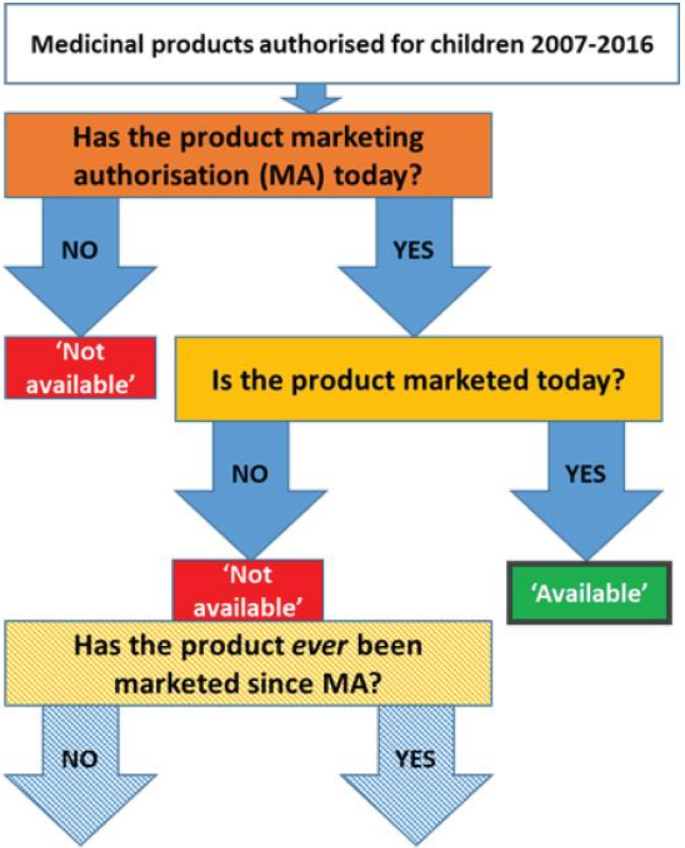

Figure 1 Flow chart illustrating how marketing status was assessed for the individual products. MA, marketing authorisation.

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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

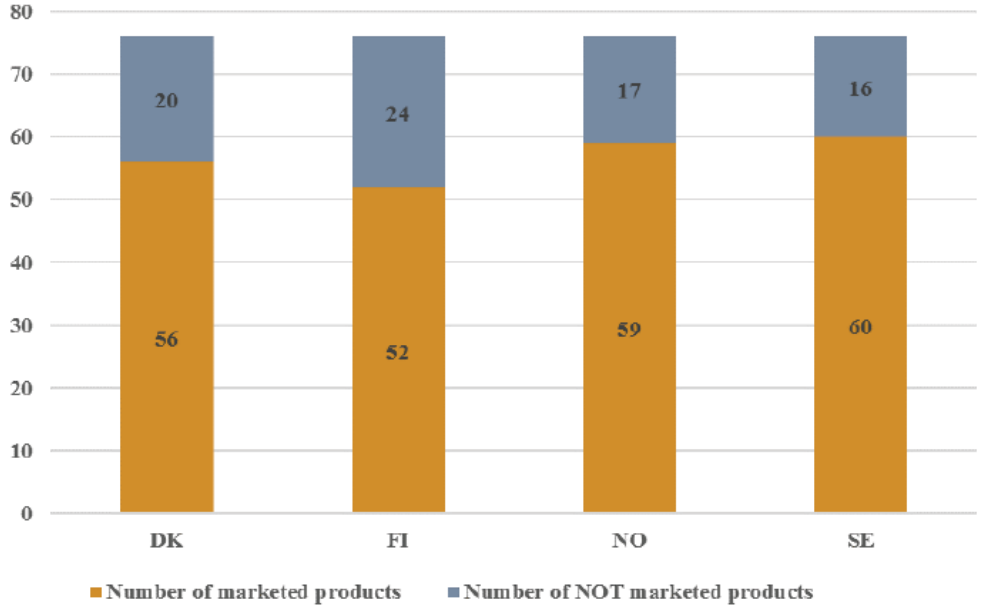

Figure 2 Number of new medicinal products marketed (or not) in DK, FI, NO and SE. DK, Denmark; FI, Finland; NO, Norway; SE, Sweden.

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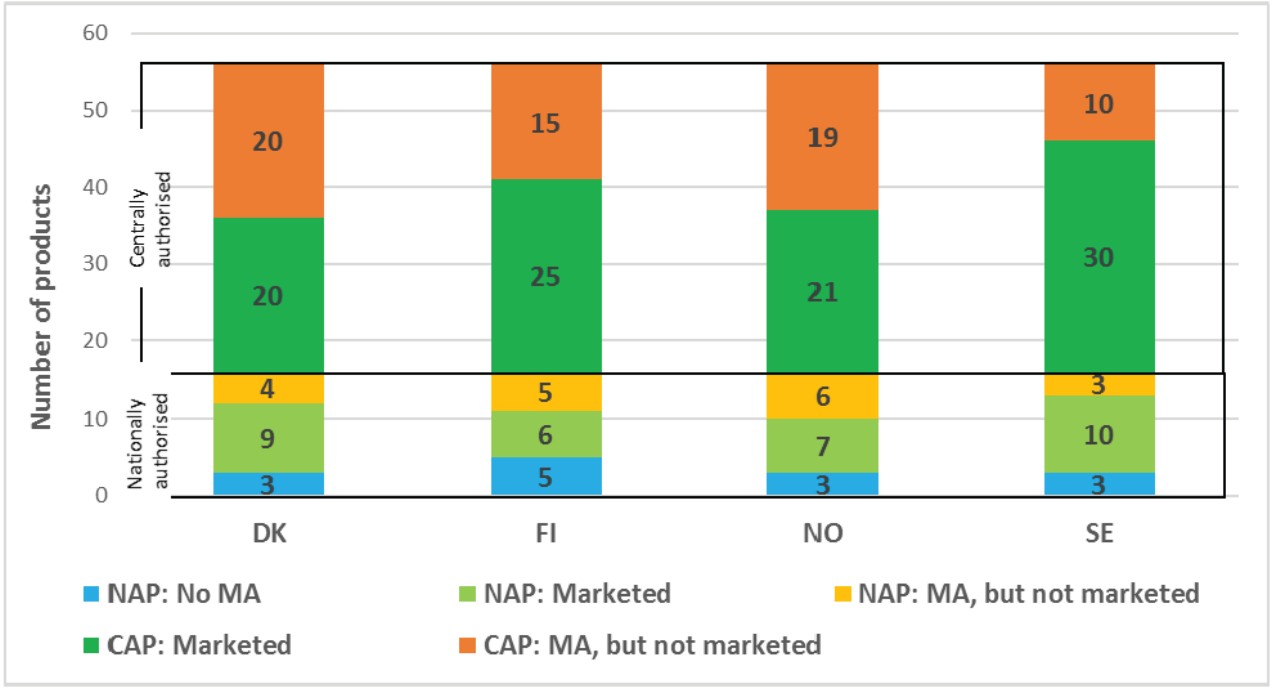
New formulations (forms/strengths)



- **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*

Open access Original research

BMJ Paediatrics Open

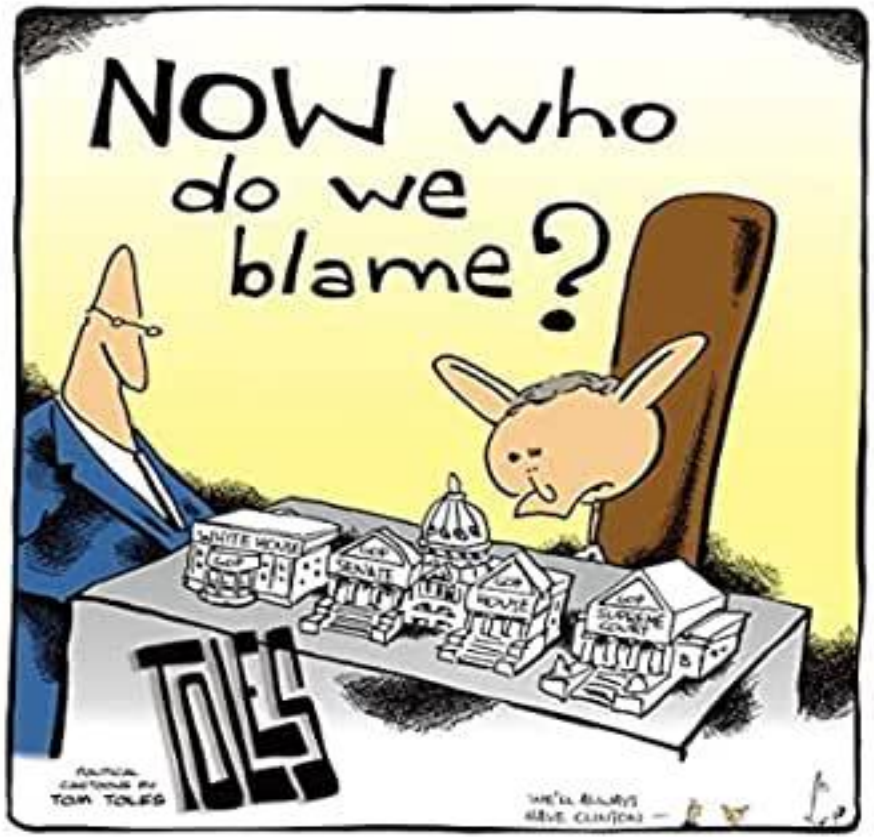
Does the EU's Paediatric Regulation work for new medicines for children in Denmark, Finland, Norway and Sweden? A cross-sectional study

Pirkko Lepola ,[1] Siri Wang,[2] Ann Marie Tötterman,[3] Ninna Gullberg,[4] Kirstine Moll Harboe,[5] Elin Kimland[4]

https://bmjpaedsopen.bmj.com/content/bmjpo/4/1/e000880.full.pdf

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

https://www.amazon.com/NOW-Who-Do-Blame-Political-ebook/dp/B005D720ZI



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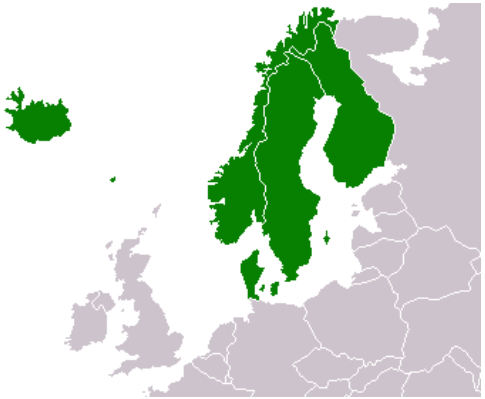
Our ‘regulatory tool box’ in NO

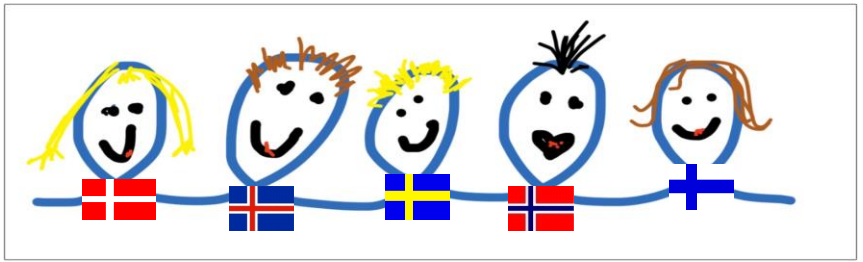
- Fast and efficient regulatory handling; recognise assessments from other MSs
- Promote and enable multilingual packages (within or outside Nordic area)
- Agree higher maximum price than normally accepted
- Fee waiver/reduction at time of MAA or at later procedures
- Particular attention to withdrawal 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/strenghts 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

Dalrymple H and Sorrentino M: Journal for Clinical Studies, vol 13;3 www.jforsc.com

From Little Acorns: A Scandinavian Study's Implications for Paediatric Development

In December 2020, a paper[1] was published in the journal *BMJ Paediatrics Open*, a respected journal modest impact factor of 2.496, regarding pae product availability in Scandinavia (N Finland and Denmark). The study describ contained extremely informative data, so e it could – and should – trigger a wide-reach approach to the development of and access t medicines. Lepola and her colleagues examin status of new paediatric medicines listed Commission's ten-year report on the imple Paediatric Regulation, as initially authorise and 2016[2], and assessed the products' availab countries. The main findings of this study w

- 21%–32% (16/76–24/76) of the new me were not marketed,
- Of the new formulations relevant to children, 29%–50% (16/56–28/56) were not marketed,
- A significant proportion of these products had never been marketed.

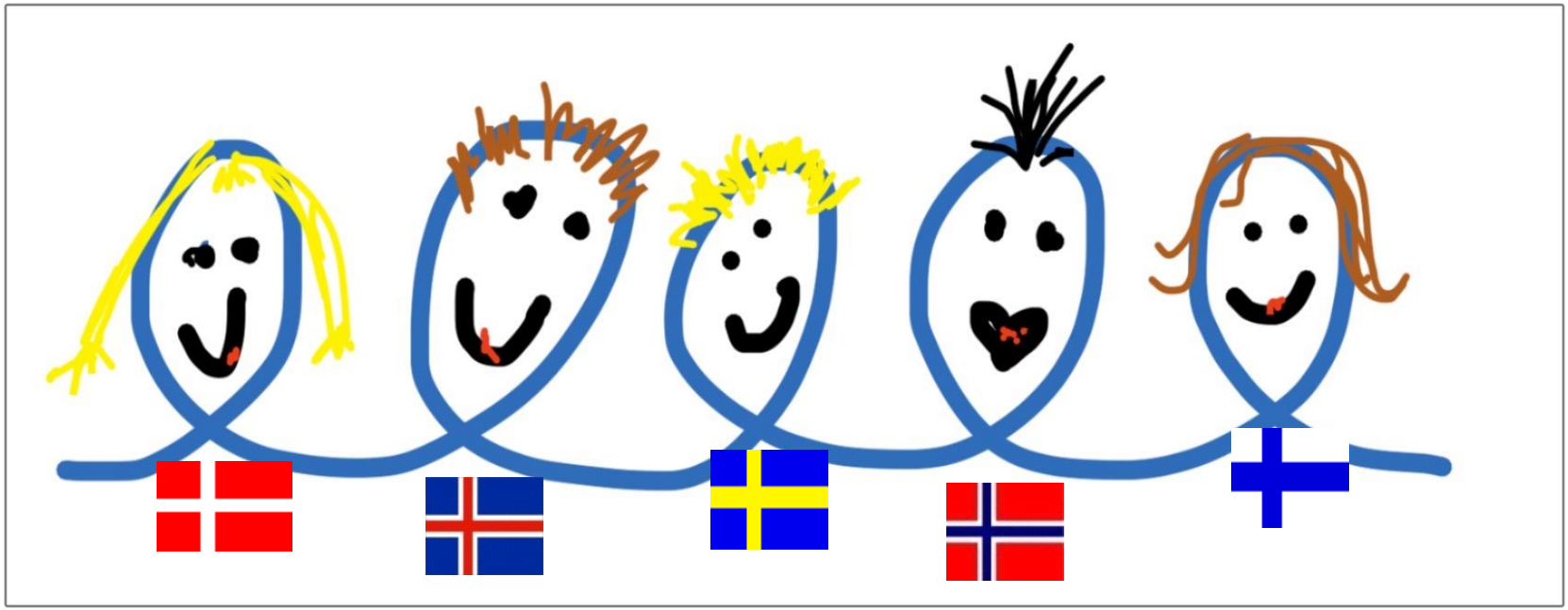
The authors conclude that similar data from other countries are needed to evaluate the overall European status of marketed paediatric approvals. How true!

postulate that regulatory obligations in addition to national pricing and reimbursement systems may impact such decisions, together

product availability in Scandinavia (Norway, Sweden, Finland and Denmark). 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

fulfilling a specific paediatric need. The authors also point out, correctly, that the marketing authorisations for paediatric products in Europe *allows* manufacturers to market the drug in all EU member states but does not *compel* them to do so. Marketing is, therefore, not specifically encouraged, and potential rewards, e.g., extended patent protection, are granted nationally, rather than on a pan-EU basis, for the whole product rather than any specific formulation.

Do Lepola's findings raise a potential ethical concern? Lepola's paper lists the new medicinal products and the new pharmaceutical forms and strengths which have marketing authorisation but have not



Thank you for your attention!

noma.no

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