MINUTES
STRATEGIC ADVISORY GROUP MEETINGS | JAN/FEB 2022
RECORDING LINK
PRESENTATION

VIRTUAL | January 20, 2022
15.00 – 16.00 CET

MEDICINES FOR CHILDREN

Chair: Mark Turner

1. **Introductions (10 min)**

   Please see the list of attendees attached

2. **Overview of paediatric medicines in Europe (10 min)**

Burning issues:

- The correct use of medicine in children (formulation and the dosage).
- The lack of medicine that this appropriate for and specifically aimed at children.
- Excessive prices of some of the orphan drugs for rare diseases / high expense low volume medications.
- The remaining large number of prescriptions of off-label drugs in our patients.
- Another issue is the lack of Investment in drugs for specific paediatric indications/needs
- Insufficient infrastructure/networks to perform multicentric paediatric trials across Europe.
- Some medications are approved by EMA, But are not approved or reimbursed by national authorities, which brings inequities in access to care in Europe. This leads to a host of new problems and lack of investment.
- Despite EMA approval of drugs, authorities can still block them, which leads to inequities in the different European countries.
- Because of the lack of infrastructure, there is a lack of investment and therefore still a high number of off-label drugs.
- This in turn, creates mistrust and unwillingness to give consent to in parents (for example, if young doctors have to explain the off-label medications).
- medicines security
- Supply chains issues for children specifically: medical equipment getting to children (for example suppling children with ventilators at home) – these are sometimes also self-inflicted by the country's policies.
- Shortages for some of the medicines that we've relied on (greater risk of shortages in the next few decades)

Further comments:

The process of drug development: several groups have suggested that access and pricing of medicines for children would be improved by knowing how much it costs to develop and supply medicines so that rational pricing incentives can be offered to companies.
Paediatric drug development needs to match the need for, and impact of, medicines.

Pricing: when it comes to pricing this is down to health technology assessments and currently each country does that in a different way, so it's very difficult. For any company trying to sell a medicine, gathering the information in all countries is a massive challenge. People who oversee pricing say the companies don't provide the information that is needed to set a fair price.
It was suggested that if a company has a marketing authorization through the European Medicines Agency they should market the product across the EU.
Information that is used to price needs to be included in the same studies that we do to test the medicines. Pricing should be governed much better and there should be much more pricing transparency. Most drugs (also vaccines) are produced with public money.

Data collection: Sometimes we need to target neonates or critical care or other groups we need data about medicines before and after marketing authorisation.
We need data on safety when the medicine is being used to routinely and things like real world data platform trials should be included.

3. **Policy opportunities and suggestions for future work (15 mins)**

The chair mentioned that the European Commission has published a pharmaceutical strategy for Europe approximately 1 year ago and it is moving towards a legislative phase to address many of the issues raised.

The strategy aims at creating a future proof regulatory framework and at supporting industry in promoting research and technologies that reach patients to fulfil their therapeutic needs while addressing market failures.
It will also consider the weaknesses exposed by the coronavirus pandemic and take appropriate actions to strengthen the system.

The working group may choose to work with the process outlined in the strategy, but amendments may be necessary on a case-by-case basis.

The four pillars of the Strategy are below:

- ensuring access to affordable medicines for patients, and addressing **unmet medical needs** (in the areas of antimicrobial resistance and rare diseases, for example)
- supporting **competitiveness, innovation and sustainability** of the EU's pharmaceutical industry and the development of high quality, safe, effective and greener medicines
- enhancing **crisis preparedness and response** mechanisms, diversified and secure supply chains, address medicines shortages
- ensuring a **strong EU voice in the world**, by promoting a high level of quality, efficacy and safety standards

The first two pillars are directly relevant to the points about access to medicines and about making sure that medicine has a marketing authorization, that it available in each country and that it is affordable.

Affordability is a critical issue, as many countries must pay for healthcare from limited budgets.

With respect to research infrastructure for paediatric drug development, c4c is a pan-European research network in each country represented on the call with the exception of Belarus and Ukraine. Apart from those two countries, a national hub for doing paediatric trials is active or under development and can be found here: https://conect4children.org/national-hubs-2/ or by contacting spoc@conect4children.org

By the end of 2022 c4c will be launching an organisation that will be able to provide this infrastructure to work across multiple countries.

Currently, academic studies can ask for advice about study design by contacting spoc@conect4children.org

4. Practical opportunities (15 mins)

- Produce a statement or consensus paper
- Include the issues on pricing (and related) in the statement to highlight the unfairness and the need to make medicines available in an equitable way.
- From the statement there should be a distinct action plan / practical items to execute. We need to step back and think about to achieve something after the paper is produced.

Some further ideas:

- Address people who are in a position to make change or to affect (European bodies, the Commission, individual member states / governments)
- Recruit active volunteers.
- Speak to legislators once a policy comes out but
- Pay attention to patient groups, clinical teams, public health alliances, European Medicines Agency and others : talking to people before the legislation comes into influence.
- We need to be working about education in general not just about legislation and policy, but helping to explain (for example, produce and spread leaflets about off-label medicine, translated into local contexts/ local language so that that information can continue to be spread)
- This could be done by developing a communication campaign with a strong core message to spread to all possible groups
- We need to look for the key messages and we can practise by putting something into literature, but we then need to have some, some sound bites, some justifications that go to the legislators (or the people that influence legislators).
- We need to have some targets in mind, for example :
  - → We could attend or organised conferences.
  - → We can make presentations
  - → We could write papers and go to meetings
  - → We can spread the word on social media
  - → Put up a stakeholder meeting

5. **Next steps (10 mins)**

Draft legislation is expected late 2022
There is likely to be an integrated approach to general pharmaceutical legislation and updates to the paediatric and rare disease legislation
National and international lobbying about draft
National and international campaigning about issues

- Legislators
- Professionals
- Public

The key thing is to have a message and to choose how you're going to share it and then have a plan. This plan needs to be presented to the right people to show it with the right people who can have the influence that we want.
We expect legislation to appear at the end of 2022, so we have a few months to get a message out there to begin to influence the people in the European Parliament and the Commission and nationally.

The suggestion is to meet again in six weeks' time. The group is encouraged to begin to think about the message that we want to put across some examples and then ways to work on those messages.

List of Attendees:

First Name	Last Name
Mark	Turner
Liesbeth	Siderius
Barbara	Rath
Ülle	Einberg
Anna	Lastovka
Karin	Geitmann
Nora	Karara
Risto	Lapatto
Lars	Gelander
Chris	Pruunsild
Marina	Mamenko
Jordi	Antón
Mark	Turner
Adamos	Hadjipanayis
Artur	Mazur
Laura	Reali
Joe	Brierley
Berthold	Koletzko
Stefano	Del Torso