ACCELERATE Annual Conference 2021

Topic: How to prioritize developments in specific areas of pressing unmet pediatric needs?

Executive summary

During the 2021 Annual ACCELERATE conference, participants were split in small groups to work on five topics related to the development of innovative therapies for children and adolescents with cancer in order to identify issues and propose solutions. Proposals were discussed thereafter in plenary sessions to define actions. An executive summary has been prepared for each topic.

Disclaimer: The views expressed in this Executive Summary are the personal views of the participants of the Breakout Session and may not be understood or quoted as being made on behalf of or reflecting the position of the agencies or organizations, including ACCELERATE, with which the authors are affiliated.

Chairs: Lia Gore (Children's Hospital Colorado), Nicole Scobie (Zoe4life), Leona Knox (Solving Kids' Cancer UK), Dominik Karres (EMA)

1. Rationale

- A future revision of the EU paediatric regulation may consider having specific rewards/incentives to direct the development of medicines in specific areas where there is a pressing unmet need for children.
- The main objectives of this session were to discuss 1) How in the field of paediatric oncology unmet needs could be defined – what are unmet needs, who should define them? 2) How does prioritisation relate to defining unmet needs?
- Identifying the unmet needs of paediatric cancer patients and prioritizing drug development in order to address them is crucial but complex, with many factors to be considered:

![Flowchart Diagram](chart.png)

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2. Topics discussed

a. Definition of Unmet Needs

Defining unmet needs for children and adolescents with cancer is highly complex, as it is a “moving target” and hence unmet needs are constantly evolving over time. Furthermore, there are several subjective factors influencing their definition, with not one size fitting it all. Regional issues and heterogeneity need also to be considered, e.g., poor access to existing drugs which varies across continents and countries. Defining unmet needs is highly challenging and limitless.

Several categories of unmet needs were suggested – the extent to which these are weighed is influenced by disease type:

<table>
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<tr>
<th>Examples of Drivers of Unmet Needs</th>
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<tr>
<td>- Survival rates</td>
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<td>- Lack of available therapy for the indication</td>
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<td>- Treatment sequelae that seriously affect the quality of life of survivors</td>
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<td>- Lack of access to a clinical trial for patient(s)</td>
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<td>- Relapsed and refractory disease (especially relevant for solid tumours)</td>
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<td>- Inclusion of &gt;12 years old patients in adult trials</td>
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<td>- Unless the survival rate is 100% in any disease type and children are still dying, with sequelae that seriously affect the quality of life of survivors the disease remains arguably an unmet need</td>
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b. Who should identify unmet needs?

- The crucial importance of multi-stakeholder collaboration to define and address the unmet needs of children and adolescents with cancer was highlighted.
- There is a distinction between what are the needs and what are assets available – however both aspects are relevant.
- Working in silos is ineffective.
- The ACCELERATE platform ethos needs to be developed across other disease types.

c. How to prioritize? Assuming it is a dynamic process – how to set it up?

- The question of whether the focus should be on prioritization rather than on defining unmet needs was debated. Prioritization is difficult but necessary, as there are no limitless resources. The impact should always be considered.
- The importance of companies taking low priority assets forward was considered. If a company has defined an asset as a low priority, then it is very difficult to prioritize it even where the paediatric need is high.
- Prioritization should be considered in each stage of the product lifecycle – taking into account the different levels of evidence in the context of the emerging R&D landscape.
3. Conclusions

- It was concluded that currently the whole area of paediatric cancers represents "an unmet need"
- Defining unmet needs in paediatric oncology is exceptionally challenging, as it is a “moving target” and hence unmet needs are constantly evolving over time. It is proposed that there is a unified and interrelated process where unmet needs are identified, the underlying scientific rationale is reviewed and then medicinal products are prioritised, as exemplified by the Paediatric Strategy Forums

4. Next steps & Output

- ACCELERATE will produce a statement regarding unmet needs