Child AdVOCacy

"Giving children a **voice** in healthcare"

Unheard Children's Voices in Health Care

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Introduction

As long as children, young people, and their parents do not have a structural voice in the organisation of federal and regional health care in Belgium, it is our duty as paediatricians (and as advocates of children's rights, especially in health care) to give them this voice.

A children's rights reflex focuses on recognising and respecting the individual rights of children, as outlined in the Convention on the Rights of the Child. This concept differs from other approaches, such as child friendliness, which are more discretionary and based on what is deemed good for children without ensuring their rights. A children's rights reflex ensures an automatic and structural attitude that approaches situations from a children's rights and participation perspective.

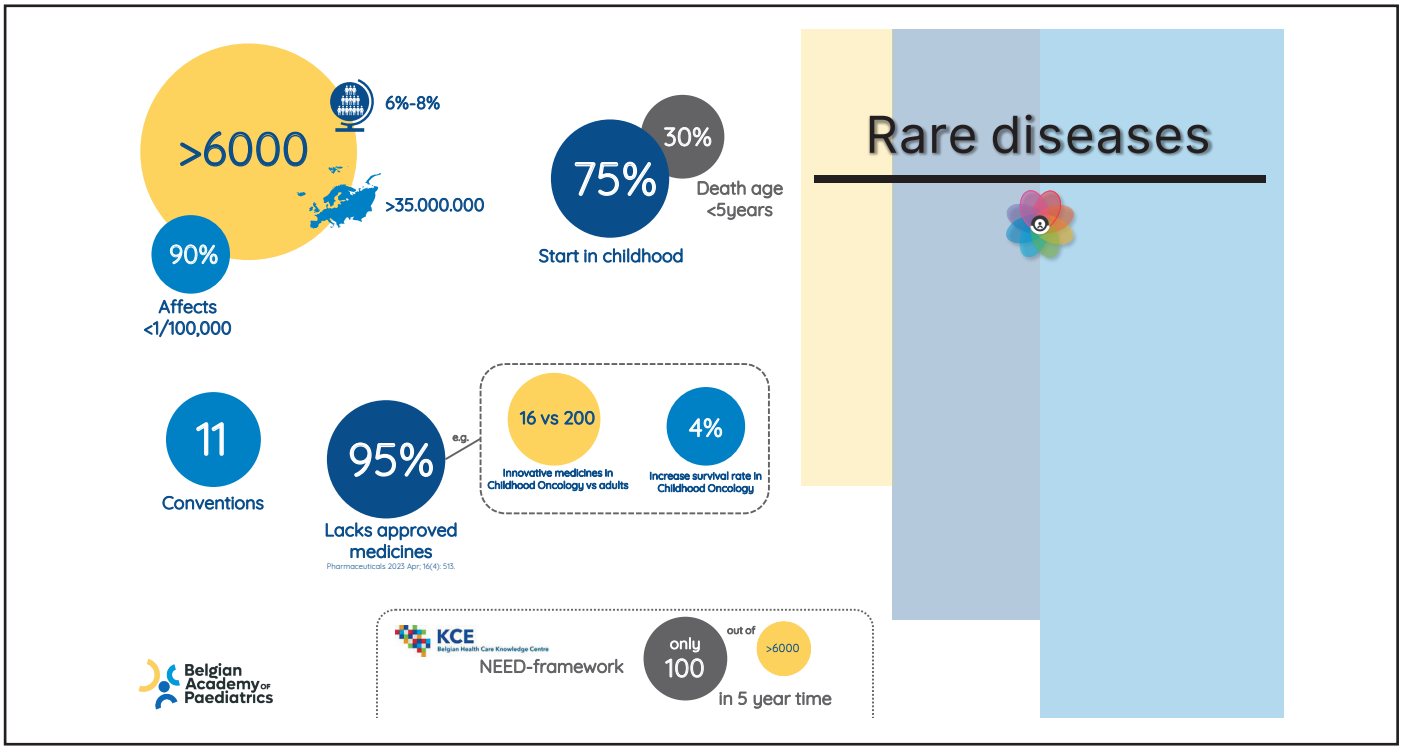
We aim to strengthen the position of children and young people by integrating a children's rights reflex into policy, research, and practice.

In this section, we provide them with that voice by backing up our figures with a statement or story that has appeared on social media or in the news in the last 3 months indicating children's health-care 'inequity' and 'inequality'.

This issue focuses on the entire journey from symptom onset to treatment for rare diseases, highlighting how the paediatric population is disproportionately affected.

The figures

To date, there are more than 6000 rare diseases affecting between 6% and 8% of the global population and more than 35 million people in the



European Union. These diseases often manifest early in life, either at birth or during childhood, have lifelong symptoms and can progressively worsen, become chronic or relapsing and lead to life-threatening conditions. Alarmingly, 75% of rare diseases have an exclusive onset in childhood, and approximately 30% of these children die before reaching the age of five.

Over 90% of rare diseases affect less than 1/100,000 patients, which contributes significantly to the limited knowledge of most rare diseases among healthcare workers (1). In order to recognise early symptoms and understand the complexity of the individual needs of patients and their families, a qualitative educational program and significant exposure is necessary for all healthcare workers involved in paediatric care. However, today the Belgian healthcare system lacks a solid quality framework and competency requirements, leading to potential delays in diagnosis and mismanagement. Only 11 (rare) diseases are supported by affordable paediatric multidisciplinary care.

Furthermore, only 5% of rare diseases have effective treatments available, meaning that 95% lack approved medicines (2). Thus, it is crucial to incentivise the development of medications for this significantly affected population. Since the European Orphan Regulation on Medicinal Products and the European Paediatric Regulation were enacted, there has been an increase in the development of paediatric appropriate medicines, but much more work remains to be done, in particular to promote development in diseases that predominantly affect children and to develop a framework that facilitates drug development based on mechanism of action studies. Currently, knowledge about rare diseases is still limited, and the lack of approved treatments has led to the harmful off-label use of medicines by physicians and pharmacists.

Exemplative, every type of childhood cancer is a rare disease. The most significant advancements in oncology have been made for adults. Since 2007, more than 200 cancer drugs for adults have been approved. In the same period, only 16 drugs have been approved for paediatric cancers. This disparity is also evident in young patients' access to innovative clinical trials: access remains an exception in paediatric cancer. As a result, the average survival rate for childhood cancer has stagnated. Since the year 2000, the survival rate has increased by only about 4% (3).

The voice

RaDiOrg (Rare Diseases Organisation Belgium) has serious concerns about the current NEED framework developed by KCE (Belgian Health Care Knowledge Centre) to address unmet needs in rare diseases. The methodology, aiming to analyse 100 diseases over five years, is inadequate given that there are over 6100 rare diseases, of which only 6% have effective treatments. The slow pace of the framework needs to be reconsidered as it relies heavily on data and spokespersons with essential information that is lacking for many rare diseases. Furthermore, while the framework focuses on disease-level analyses, it fails to reflect individual patient needs, the diverse manifestations of diseases, and cross-cutting medical needs. RaDiOrg suggests that different methodological choices could better address the vast, often invisible, unmet needs of patients, fostering greater equity in health care.

Conclusion

Industry often claims that the problem of drug development gaps goes beyond a lack of commercial potential. Factors such as the state of knowledge, access to research populations and an existing infrastructure for collaboration play a key role. Existing research infrastructure that connects physicians, patients and caregivers, such as the Belgian Paediatric Clinical Research Network (BPCRN), has shown to tackle many of these challenges 1) by the facilitation of paediatric studies and increasing Belgium's attractiveness for clinical trials, 2) by developing well-educated trial sites and promote education among all stakeholders, and 3) by identifying patients' needs and engaging patients in every phase of research. The benefits of this model have been clearly validated in an international context (e.g., within the IMI2-funded conect4children project) and the BPCRN is now seeking – together with its stakeholders – for a sustainable future. That such future is viable has been clearly demonstrated in paediatric oncology, where national connectedness is formally embedded in a European context, where academic and industry research organisations are co-developing supported by governments, patient organisations and a critical amount of structural funding. Future investments in both oncology and non-oncology research, should clearly focus on paediatrics and rare diseases, and leverage existing research infrastructure in order to provide a maximal benefit for patients and families.

REFERENCE

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The content of this article aligns with recommendations number

in the Plan Care for the Child

May we invite all paediatricians who are active on LinkedIn, Instagram, etc., to support your feeds and posts with the following hashtags:

#unmetneeds #invisibleneeds #rarediseases #needfordata #needforvisibility #needforspokespersons #needforresearch #childrensrights