



# *IDENTIFICATION OF PAEDIATRIC MEDICAL NEEDS – METHODOLOGY*

## **Patients perspective**

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# Defining unmet paediatric needs and criteria for prioritisation

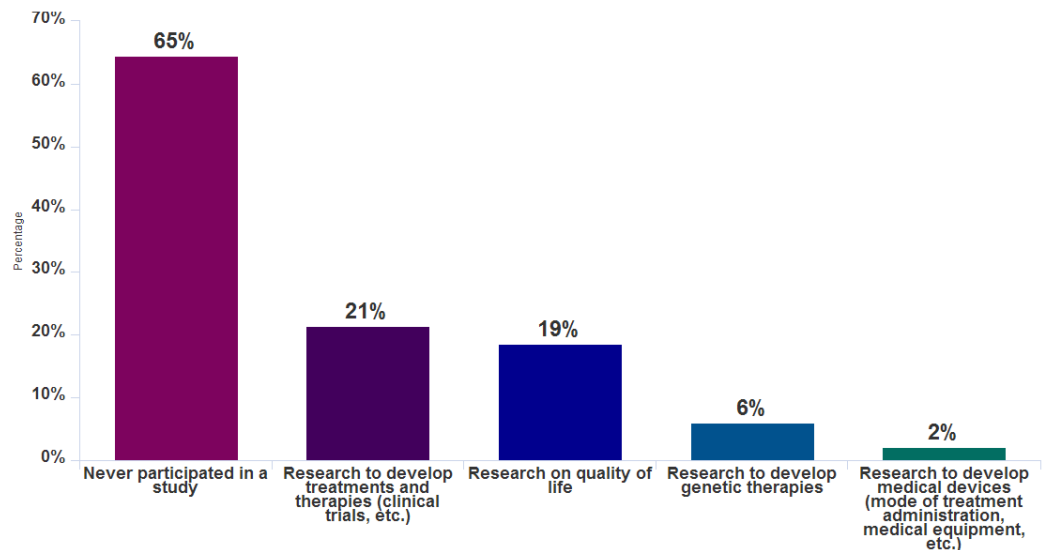
- Defining unmet medical needs is difficult – the paediatric context adds a layer of complexity
- Assessing the burden of a condition – PROMs – **weight of the outcome / dynamic component** as might be **age-related**
- **Holistic approach** – unmet parents' needs – QoL of the parents as a surrogate endpoint
- The need may be unmet only because of lack of access to a product
- Criteria to be taken into account:
  - **Rarity of the condition**
  - **No treatment at all**
  - **Product-related: potential to address the needs - curative treatment, disease-modifiers, stopping disease progression, targeting prominent symptoms of the condition, impact on QoL**
  - **Expression of the needs – finding commonalities –transversal endpoints**

# How we get feedback from the Rare Diseases Community

- Feedback from our members : individuals, patient organisations, the National Alliances for Rare Diseases, the European Rare Disease Federations, the 24 European Patient Advocacy Groups (one EPAG per European Reference Network)
- Feedback from the RD Community as a whole thanks to our **survey Programme Rare-Barometer Voices** - 40% of Rare Barometer Voices participants are parents of children living with a rare disease (n=2960)



What type of research did you participate in?  
(paediatric population)  
(n=447)



# Potential questions



**Do you or have you ever taken or experienced a treatment for your/the person you care for rare disease?** (multiple answers possible)

- Yes, a treatment to prevent the disease
- Yes, a treatment that completely cures the disease
- Yes, a treatment that relieves one or several symptoms directly linked to the disease
- Yes, a treatment that slows down or stops the disease
- No, because to my knowledge there is no cure for my disease
- No, there is a cure but I don't have access to the treatment

**Could you report all the negatives aspects/obstacles/difficulties in relation to your treatments?** [open question]