Using Patient Experience Data to Evaluate Medical Interventions

Generating, understanding and using patient experience data within and alongside clinical trials.

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CHAPTER 7

Generating patient experience data (PED) in complex populations and programs; examples of pediatrics, central nervous system (CNS), cancer and rare disease

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Key takeaways

- Pediatric populations
  - » Children should be able to engage in qualitative and quantitative patient experience data (PED) research from age 8.
  - » Between 5 and 8, validity of self-report is questionable and should be based on very simple, concrete, current, and sufficiently remarkable events.
  - » Asking concrete momentary assessments is recommended for young children and new digital technologies could help with this.
  - » When children are unable to self-report, observable facts should be evaluated by external persons, and proxy evaluation avoided.
- Populations with central nervous system (CNS) disorders
  - » Patients with CNS disorders experience a wide array of physical, cognitive and/or sensory limitations that can make it difficult for them to reflect and report on their experiences. Further, measurement of the patient experience in CNS disorders is often done through
- Cancer programs
  - » PED is increasingly important in the development of anti-cancer drugs, and in cancer care. However, generation of robust and informative PED is complicated by the increasing use of accelerated drug development programs and basket and super umbrella trials, high attrition rates, and a substantial amount of missing PED due to disease progression, treatment-related toxicity and death.
  - » Yet PED collection is possible and informative with appropriate planning.
- Rare disease programs
  - » Rare diseases are often complex, multisystemic and chronic. Most people with a rare disease will not be cured in their lifetimes, and thus identifying ways to improve quality of life (QoL) is important.

Advice for researchers interested in collecting PED in complex populations and programs

- Pediatric populations
  - » The ability to collect valid PED with children will vary across age groups and developmental stages. Precautions and interactive approaches should be used to make children and adolescents comfortable and improve their engagement in PED research.
  - » The use of innovative, participatory and friendly digital health tools adapted to the pediatric population may improve compliance and collection of robust data.
- Populations with CNS disorders
  - » Researchers must evaluate whether patients with CNS disorders have sufficient capacity to provide accurate and reliable reports of their experiences.
- Cancer programs
  - » PED in cancer often comes from patient-reported outcome (PRO) instruments. Careful planning is essential for data analysis and interpretation. If accelerated approval is likely to be sought, standalone studies may be needed to support PRO interpretation, including the generation of psychometric evidence.
  - » Other forms of PED can be useful to supplement PRO data, including preference research.
- Rare disease programs
  - » PRO instruments should be used to collect information on QoL, where possible, from rare

"You guys give up? Or are you thirsty for more?"

— *Kevin McCallister in Home Alone (1990 film)*

Prior chapters in this book have outlined the approaches to generating reliable, valid, meaningful and interpretable patient experience data (PED) from qualitative and quantitative exploration. These approaches are, in most cases, tried and tested across drug development programs from various institutions and intervention developers/sponsors. However, there are some populations in which the collection of PED is a bit more difficult. This includes collection of PED from people with symptoms that make reporting experiences difficult. This includes people with speech issues, physical limitations impacting on inability to complete surveys or clinical outcome assessments (COAs), or cognitive impairment. Two specific populations in which the collection of PED needs special consideration are children and those with neurological or psychiatric indications (summarized as "central nervous system (CNS) disorders"). This chapter will discuss the collection of PED in children and among people with CNS disorders.

In addition, prior chapters in this book have focused on collecting PED in a traditional model of drug development; that is a structured progression from Phase I, through Phase II and Phase III for registration. In some diseases, including oncology and rare disease, this may not be possible or necessary. This chapter will therefore also discuss the impact of novel development pathways on

the generation and use of PED in the context of oncology, and provide a short discussion on rare diseases for whom generation of PED have some additional nuances that are worth highlighting.

Generating PED in pediatric populations

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Chapter 3 of this book discuss the role of qualitative insights as the cornerstone of PED, explaining how discussions with patients are essential to understand perspectives of disease, preferences and priorities for treatment, and experiences of research studies. Subsequent chapters (**Chapters 4–6**) speak to the role of PED derived from structured outcome measures and surveys completed by the patient (patient-reported outcome (PRO) instruments; patient preference research) or delivered through technology worn by the patient.

These all inherently assume that reliable and valid PED can be generated from the patient population. This may not always be the case when trying to generate PED from children.

Children are defined – for the purpose of pediatric research – as those between the ages of 0 and 17. This is quite a large age range, with significant differences in motor, cognitive, linguistic, social and emotional development in infants, young children, adolescents, and young adults.[1,2] Acknowledging this broad age range, it is important to explore whether children can reliably report their experiences, and how to generate PED about those who cannot.

HOW RELEVANT IS PED IN PEDIATRIC INTERVENTION DEVELOPMENT?

Some interventions are developed specifically for use in children, while others are developed for use in adults and used off-label in children.[3,4] However, children are not "little adults," and interventions for children need

specific considerations to ensure that they are meeting the priorities, needs and preferences of children and their caregivers. This information can be obtained through PED. For the collection of PED, researchers generally recommend direct reports from patients,[5] but this is not always easy in pediatric research and thus, children are not always asked to share their own experiences and insight to inform the development of interventions.[6,7] There are some initiatives, including the International Children's Advisory Network (iCAN), that are seeking to change this through generating a network of young persons' advisory groups (YPAGs) for intervention development. Research supports such initiatives, showing the potential for improved success in the trial process (including recruitment and retention) and subsequent health outcomes when involving children in design.[8,9] Researchers and families alike require training and education to be able to involve the pediatric patient in PED research in a sensitive manner.[10]

WHAT MAKES PED DIFFICULT TO GENERATE IN PEDIATRIC POPULATIONS?

The generation of robust PED relies on good scientific research methods (as described in other chapters of this book) and a patient population willing and able to reflect on and share their insights, experiences, priorities, attitudes, knowledge and health-related behaviors. Some children – especially young children – may not be able to understand questions that researchers tend to ask to elicit PED, nor articulate their thoughts and feelings in a way that is easily interpretable to researchers. That does not, however, mean that robust PED cannot be collected in pediatric intervention development. Rather, care must be taken to maximize quality.[9,11,12]

HOW DO I COLLECT ROBUST PED IN PEDIATRIC RESEARCH?

It is generally agreed that children's understanding of health-related concepts increases with age,[2,13] and while developmental evolution may not be the same in all

children,[14] age is a reasonable way in which to initially classify pediatric populations for PED research. The age at which children can provide reliable information in a qualitative interview (as described in **Chapter 3**) and the age at which a child can reliably respond to a PRO instrument or preference survey (see **Chapters 4 and 5**) are two different issues.[1]

Qualitative PED research

While it is impossible to collect health information directly from infants,[2] children as young as 4 or 5 may be capable of providing some specific information on concrete aspects of their health status (about events that are sufficiently remarkable).[1] But, it is not until age 8 that children are generally able to explain simple concepts like "pain" in their own words[1,2]; and even then only about their current or very recent experience.[1,15] At this stage (i.e., from age 8), it would therefore be reasonable to engage children in simple concept elicitation research; that is qualitative research to explore the patient experience of managing illness, disease or a condition (see **Chapter 3** for details). By the age of 12, most children can understand and answer questions about abstract concepts or hypothetical events[1] and thus can be expected to answer the same types of questions in qualitative research as adults. However, social and emotional aspects may be considered with caution in adolescents (age 12–17), as they may not be willing to share.[2] This is particularly true when the discussion could cause embarrassment or shame, such as with sexual functioning.[16]

Special attention should be paid to standards for good practice in conducting interviews with older children (age 8–11) and adolescents (age 12–17). For example, the interviewer should learn the discussion guide well to adapt and improvise,[17] use warm-up exercises to make children feel comfortable,[18] use age-appropriate language,[1,14,16,19–21] and ask questions in a neutral manner with simple wording, avoiding clinical terms.[16] For adolescents (age 12–17), there are additional

considerations. For example, it is important to avoid "child-like" terms during the interview.[16] It may also be beneficial to have an interviewer of the same sex to encourage open and honest discussion, particularly about sensitive topics. The interviewer should emphasize at the beginning and during the interview that the adolescent is free to not answer any questions they are not comfortable with.

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As children have limited attention span[14,19,22] and can easily become bored, it is also important to use interactive techniques to engage children in qualitative interviews.[14,17,19,23] The use of drawings, photographs, illustrations, props, and toys could be beneficial for children age 8–11, especially if they are shy.[14,18,21] Asking them first to draw a picture describing their condition or its impact, and then explain it, encourages children to share and help starting the discussion.[1,14,15,18,21]

Similarly, photo-elicitation interviews have been shown to be particularly beneficial when working with children with poor written or verbal literacy.[17,21,24–26]

Social media can also be used to engage adolescents, provide a starting point for conversation, and facilitate an understanding of other people's experiences.[27]

NHS (National Health Service) Digital's Widening Digital Participation Programme further recommends considering digital technologies as an enabler for improving health services with adolescents.[27] Even with these techniques, the length of interviews should be shorter for adolescents than it is for adults, and even shorter still for children age 8–11.[1,23] Several breaks should also be made during an interview.[1]