Youth and family engagement in a pediatric rare disease research network

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Background and setting

- Engaging individuals with lived experience in research that is relevant to their health and well-being is increasingly recognized as important to improve research quality and to ensure results are meaningful.[1]
- In pediatric research, it is important to engage youth and also their family members or caregivers, as they play important roles in health and health care.[2]
- INFORM RARE: a Canadian research network established in 2020, co-designed by patients & families, health care providers, policy-makers, methodologists, ethicists.
  - Patient and family member partners are involved as co-investigators and advisors in co-designing our research and are actively involved in key decisions.
- INFORM RARE generates evidence to improve care, outcomes, and policy for children with rare diseases including phenylketonuria (PKU), spinal muscular atrophy (SMA), and mucopolysaccharidoses (MPS).

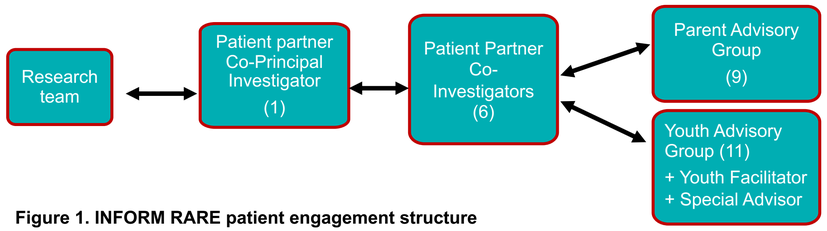

Figure 1. INFORM RARE patient engagement structure

Approach to Patient Engagement

- Co-developed a multi-layered patient engagement strategy that facilitates a range of levels of engagement[3]: lead/support, collaborate, and involve.
- A patient partner co-principal investigator (MS) has co-led our patient engagement approach from the grant idea stage.
- Six patient partner co-investigators (four parents of children with MPS, PKU, or SMA and two patient group leaders) have been involved since the Letter of Intent stage, and participate in all aspects of the network.
- As co-investigators, our patient partners are invited to join INFORM RARE's eight working groups, contributing to research across the network. This has required training and support for working group members (e.g., clinicians, policy-makers, methodologists) and research staff, as they may not be accustomed to including the perspectives of patients and caregivers.
- At the end of first year, we established two separate advisory committees of 11 youth (aged 12-18 years) and 9 parents with lived experience of PKU, SMA or MPS to contribute at key points. Advisors were selected through an application process to achieve a breadth of perspectives based on age, clinical condition, and geography.
- We are committed to nurturing a partnership with patients, families, and patient groups that is based on continuous, mutual learning.

Figure 2. Patient partner/advisor locations

Results

Patient partners (co-principal investigator and co-investigators):

- Contributed to the development of a successful funding application that established INFORM RARE, including selection of outcomes
- Co-developed a statement of patient engagement principles adapted from related frameworks[4-6] to meet the unique needs of a rare disease pediatric research network (Table 1).
- Joined working groups to co-design projects related to developing patient registries and interventions, and selecting clinical trial questions.

Advisory group members (youth and caregivers):

- Contributed to the content and layout of online surveys for MPS core outcome set development:, feedback on recruitment materials, ensuring that outcome categories and definitions are youth and parent friendly, and feedback on Delphi survey instructions
- Feedback from youth on a video game intervention

Lessons Learned

- **Take into consideration your environment:** the realities of pediatric rare disease research: clinician-researchers collaborating with their own patients/families; the small pool of potential partners; partnering with people who live with the uncertainties of their health journeys; and the demands of complex care on the youth and their families.
- **Acknowledge the unique strengths and experiences** families of children with rare diseases often have, such as in-depth knowledge of their child's health and the health care system, longstanding relationships with providers, commitments to advocacy.
- **Establish fluid levels of engagement** where patient partners can move in and out of the involve, collaborate and lead/support levels depending on the nature of the engagement activity are necessary.
- **Embrace the spirit of mutual learning** and let it guide you every step of the way. Be aware of transactional behaviours and remember that patient partners bring their lives into this. The ability to acknowledge errors and learn from them is essential.

Working together	• We provide patient engagement training to INFORM RARE researchers and students to ensure best practices that respect patient partner and advisor roles.
Inclusiveness	• We use accessible materials and language so that advisors can engage meaningfully with the research team and provide feedback. A young adult facilitator (CM) moderates youth advisory group meetings.
Support & learning	• We are committed to empowering our patient partners/advisors by offering training and adapting based on their needs as we move forward. We provide compensation to advisors in appreciation of their time.
Co-build	• We offer opportunities at various levels of engagement for our 28 parent and youth partners/advisors. Our strategy is flexible to move in and out of levels of engagement as needed (e.g., to co-design the MPS and PKU registries with patient groups).
Impact	• We communicate frequently with advisors (Figure 3) and let them know how we used their feedback (Figure 4). We regularly ask how we can improve our approach.

Table 1. Patient engagement principles & examples of how we apply them

Figure 3. Selections from end-of-year newsletters sent to parent advisors (left) and youth advisors (right, still from a video)

What we heard	What we did
Add a QR code	✓
White text in light blue textbox is hard to read – suggest making the text darker (black, dark blue).	✓
Make the "call to action" the first bullet point (it's ok if the ask is repeated).	The first point under the title now reads, "Calling all patients with mucopolysaccharidoses (MPS) and their caregivers: We need to know which outcomes are important to you."

Figure 4. Example of reporting back to patient partners/advisors on how we used their feedback

Next steps

- Co-design an evaluation strategy to more formally evaluate the experiences of patient partners, advisors, and our research team.
- We anticipate making changes to how we engage with our patient partners/advisors and understanding the impacts of patient engagement on INFORM RARE research.

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