

Youth and family engagement in clinical trials about phenylketonuria, spinal muscular atrophy, and mucopolysaccharidoses

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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).

Results

- Eleven youth and nine parents with lived experience of PKU, SMA and MPS were selected from across Canada (Figure 2) to join the advisory groups.
- Advisors have provided their perspectives, guidance, and feedback at key points during the research process via virtual meetings and email.
- Advisors' contributions so far: 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, Delphi survey instructions, feedback from youth on a video game intervention.
- We follow patient engagement principles co-developed with our patient partners and adapted from related frameworks⁴⁻⁶ (Table 1).

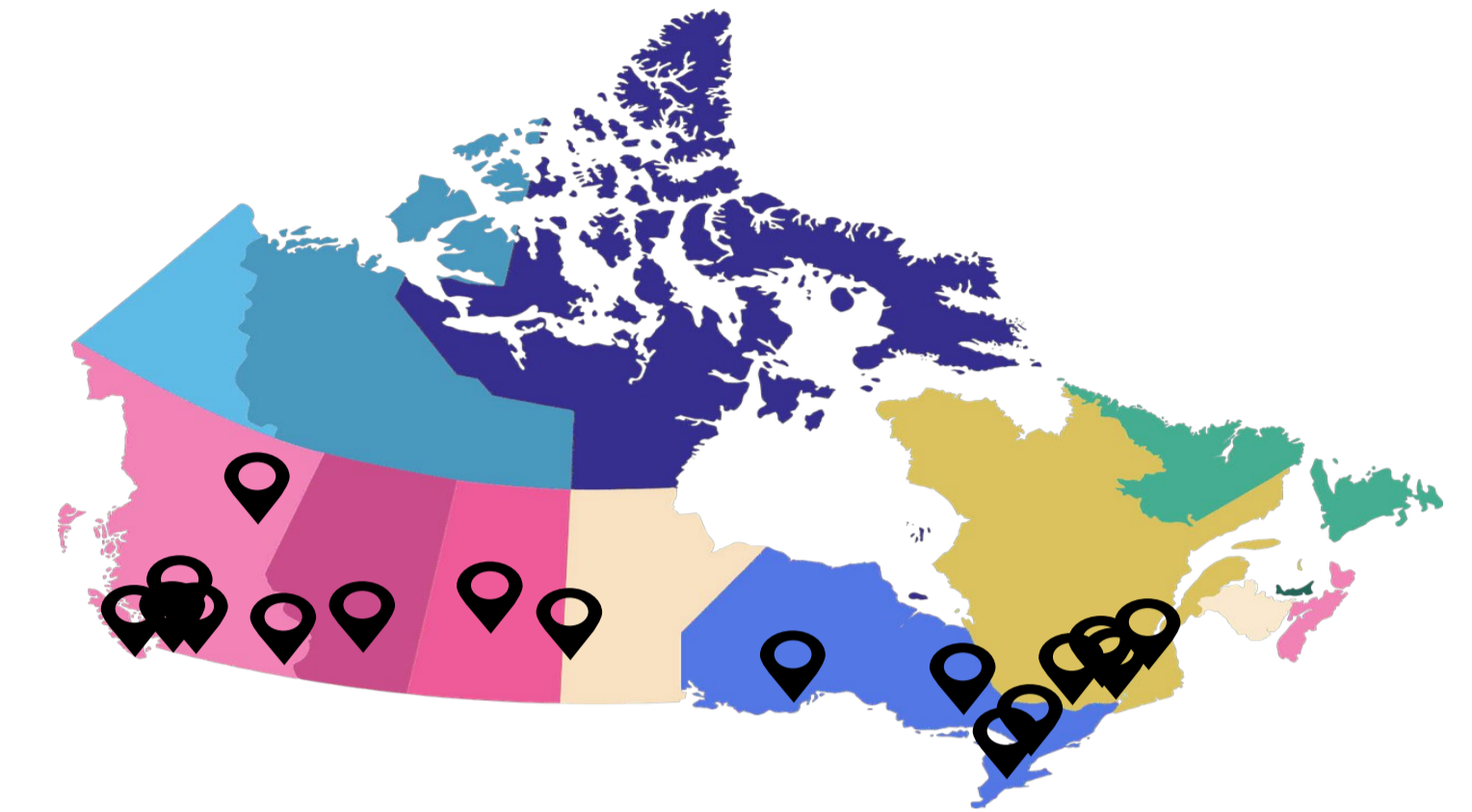


Figure 2. Parent and Youth Advisory Group member locations

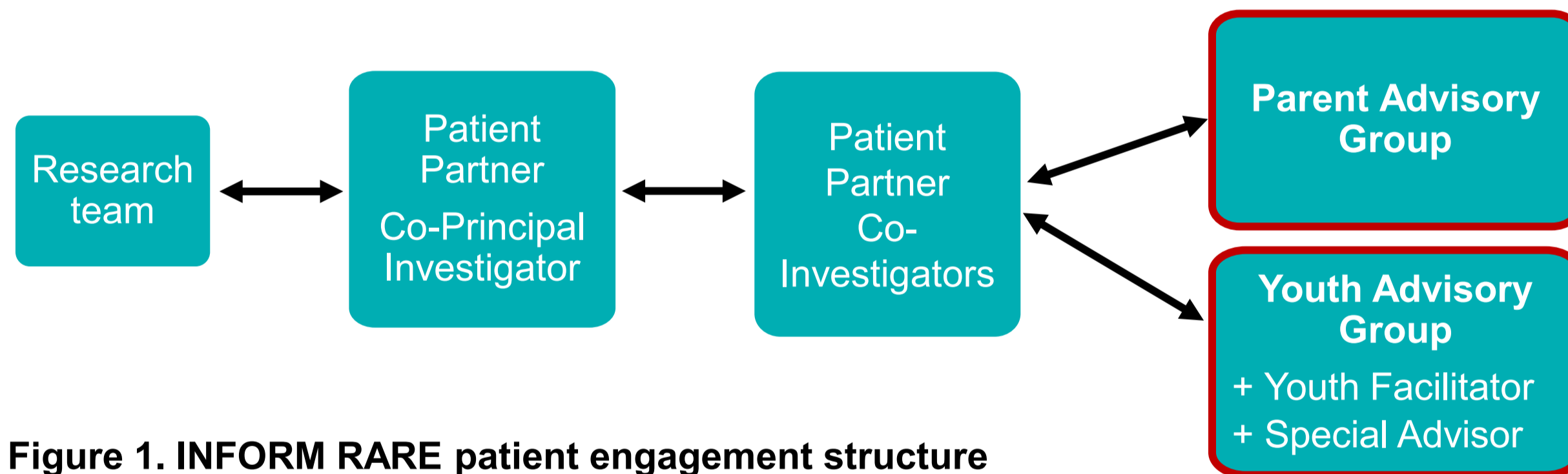


Figure 1. INFORM RARE patient engagement structure

Objectives

- This poster focuses on INFORM RARE's creation and work with two separate **advisory groups** of youth and parents/caregivers who have lived experience with PKU, SMA and MPS.
- These advisors work with our team, which also includes a patient partner co-principal investigator (MS); and 6 patient partner co-investigators (Figure 1). This structure allows varying levels of engagement³: lead/support, collaborate, and involve.
 - Advisors are mostly at the "involve" stage but there is flexibility depending on the specific task or stage.

Methods

- We invited youth (aged 12-18 years) and family members (parents, caregivers) through patient organizations and social media to apply to join the advisory groups.
- We selected advisors to achieve diversity based on age, clinical condition, and geographical location.

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

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 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 27 parent and youth partners/advisors.
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.

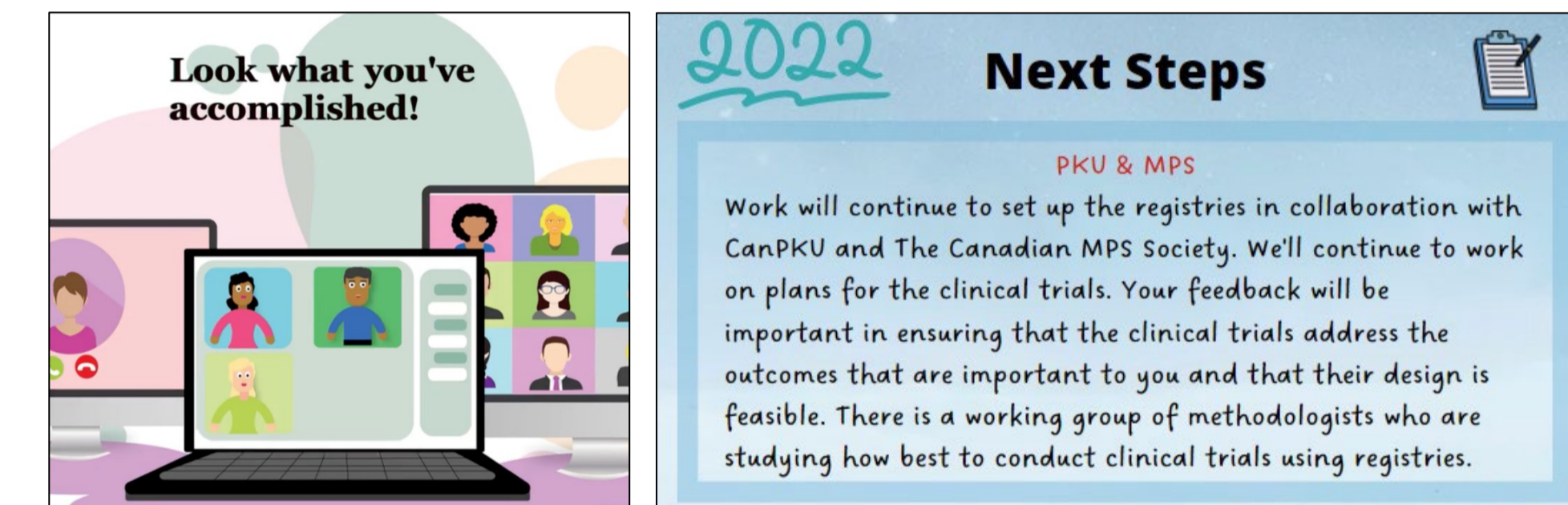


Figure 3. Selections from end-of-year newsletters sent to youth advisors (left, still from a video) and parent advisors (right)

Thank you so much for taking time to provide such thoughtful, valuable feedback. We considered everything you said and incorporated changes when possible. With your help, we have designed an appealing recruitment poster and a survey that is easier for patients and families to fill out!

Recruitment poster	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).	✓
	Font colours work well for colour blind people.	Thanks for letting us know!
	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."
	Reconsider positioning of the introductory section and the boxes.	We experimented with changing the format but couldn't get it just right. We bolded the "call to action" (first sentence of introductory section) to make it stand out.

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

Reflections and Next Steps

- Although advisors were given the option to contribute to activities that were related solely to their condition, many were willing to be engaged in all opportunities. This has added to team cohesion and allowed us to incorporate diverse patient and family experiences.
- A "one size fits all" approach does not work. Meetings, requests for feedback, interactions with the research team, and training must be tailored to youth and parent engagement.
- Next steps are to 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 advisors and understanding the impacts of patient engagement on INFORM RARE research.

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