

INFORM RARE Patient Engagement Guiding Principles

Our goal: To co-create INFORM RARE’s Patient Engagement (PE) Guiding Principles based on well-established broad principles while keeping in mind our unique needs.

Our process: We reviewed PE guiding principles from the UK’s INVOLVE, SPOR, and Ontario’s Patient Engagement Framework (formerly Health Quality Ontario) and co-designed our own principles in collaboration with our patient partners and researchers.

Preamble: As a patient-oriented pediatric research network, a core value of INFORM RARE is that patient partnership is essential to ensure that our research focuses on the priorities of rare disease patients and their families, both in the questions we pursue and the approaches we use. Parents, representatives of patient groups, and youth patients and family member partners are involved as co-investigators and advisors in co-designing INFORM RARE research. They are actively involved in selecting and refining research questions and priorities, planning and conduct of clinical trials, and dissemination of results.

The term « patient partners » is intended to include patients, family members, caregivers, and organizations that are representative of INFORM RARE’s population of interest. This includes patient partner co-investigators and our parent and youth advisors.

It is important that patient partners are not confused with patient participants in research (individuals actually enrolled in the study). Patient partners are involved in the planning, conduct, and sharing of research.

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These are the guiding principles to which all INFORM RARE investigators and collaborators will adhere in pursuing the goal of integrating patient engagement into our research:

1. WORKING TOGETHER: Work together in a way that values all voices, fosters mutual learning and respect, and that builds and sustains ongoing collaboration. Acknowledge the power imbalance that occurs when patients collaborate with healthcare professionals and implement mitigation strategies.

2. INCLUSIVENESS: Offer patient engagement opportunities that are accessible and integrate a diversity of patient perspectives by being mindful that this is deeply personal for patients and families. Proactively provide opportunities and the flexibility to accommodate diverse needs and preferences for contributing to INFORM RARE.

3. SUPPORT & LEARNING: Adequate support, training, and flexibility are provided to patient partners to ensure that they can contribute fully to discussions and decisions. This implies creating safe environments that promote honest interactions, cultural competence, use of plain language, and education. Support also implies financial compensation for their involvement and reasonable requests for time commitment.

4. CO-BUILDING: Patients and families, health care providers, policy makers, methodologists, and research ethicists are empowered from the beginning to set priorities for research, evaluate new and existing therapies, and make recommendations to improve care for children with rare genetic diseases.

5. IMPACT: Identify and share the differences that patient engagement makes to INFORM RARE research, including impacts on the experiences of all team members and the research processes. Evaluate patient engagement impact to address gaps and strive to learn from each other.



We consulted the following guidelines to co-design guiding principles tailored to our network's need:

UK Standards for Public Involvement. <https://sites.google.com/nih.ac.uk/pi-standards/standards>.
Published November 2019. Accessed August 30, 2021.

Strategy for Patient-Oriented Research – Patient Engagement Framework: Guiding Principles.
Canadian Institutes of Health Research. <https://cihr-irsc.gc.ca/e/48413.html#a7>. Updated
March 27, 2019. Accessed August 30, 2021.

PCORI Engagement Rubric. PCORI (Patient-Centered Outcomes Research Institute) website.
<https://www.pcori.org/sites/default/files/Engagement-Rubric.pdf>. Published February 4,
2014. Updated October 12, 2015. Accessed August 30, 2021.