

# **Ethical Aspects of Rare Diseases Research: Best Practices for Expert Patient Engagement**

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*Abstract:* As rare diseases continue to pose challenges to patients, caregivers and researchers, they have been increasingly recognised as a public health and social issue, as well as a crucial research topic. The growing focus on the priorities and needs of the rare disease community is accompanied with growing efforts to bring the patients' voice in the multidisciplinary field of rare disease research. The paper discusses ethical aspects of rare disease research, giving special consideration to the concept of "expert patients" as real partners in research. Best practices and self-regulatory guidelines for improving patients' engagement with rare disease research are presented. On the other hand, the paper focuses also on critical aspects and ethical concerns about the institutional roles of expert patients, especially in ethics committees, highlighting the need for a critical reflection on ethical principles and values which should orient their activities.

*Keywords:* Patients' engagement, Rare diseases, Research ethics, Advocacy.

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