



Tackling Representativeness: A Roadmap and Rubric

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National Organization for Rare Disorders

Tackling Representativeness

- What is representativeness in the context of patient engagement?
- Who and how many individuals from the patient community are needed to take part in engagement activities?
- What are the benchmark characteristics desired to engage the right individuals to capture the range of input needed?
- **Representativeness versus statistical sampling?**

Tackling Representativeness

- Multi-stakeholder Roundtable (May 8, 2017)
- Roadmap and Rubric (September 2017)
 - Intended to guide decision-making on representativeness for a given patient-engagement activity.



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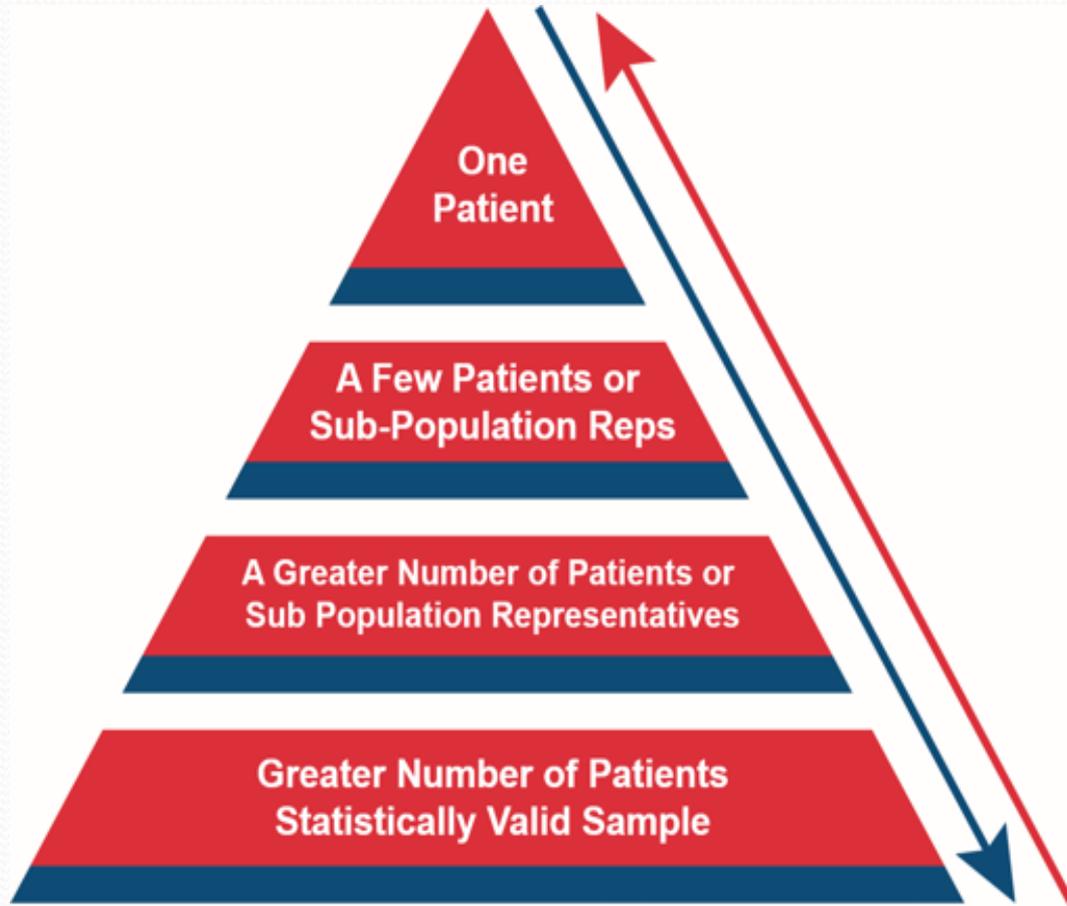


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Consensus Definition

“Representativeness” means a sufficient number of and types of people are included in the engagement activity to ensure that those engaged can speak for the target population. It refers to “who” and “how many” individuals to include in an interaction to, as closely as possible, engage with individuals that represent the broader, targeted patient population.

Representativeness Pyramid



Guiding Principles

1. *Define* objective(s) for each engagement effort
2. *Understand* the full population and subpopulations, and challenges to reaching them
3. *Specify* minimum target(s) for representativeness for the engagement activity
4. *Plan* to achieve the minimum target(s) defined.
5. *Evaluate* to assess progress on achieving target(s) or if they need to be adjusted based on new information.
6. *Document* how patient representativeness was defined, targeted, achieved, and assessed.

Rubric Example

Understand – Understand as much as possible about the full population and subpopulations, and challenges to reaching them

Guiding Questions to Consider	Good Process	Poor Process
<ul style="list-style-type: none">• What sources of information about this population are available?• Are the sources outdated or inaccurate?• Is the target patient population for the engagement activity the full population or a subpopulation?	<ul style="list-style-type: none">• Patients are engaged in helping describe the full patient population, subgroups, and anticipated challenges and burdens.• Issues with feasibility or lack of resources to get to the target are considered	<ul style="list-style-type: none">• Sources of information on the full population were not tapped or were ignored.• Patients were not consulted when trying to understand the full and target populations

Conclusion

- A single target for patient representativeness cannot fit every patient-engagement situation.
- Context, including the objective of the engagement, must influence how patient representativeness is defined for any engagement activity.
- Variability of patient interactions requires that stakeholders address representativeness as a process with a minimum target, rather than a fixed standard

Resources

- National Health Council. Tackling Representativeness: A Roadmap and Rubric. Washington, DC: National Health Council; 2017. Available at <http://www.nationalhealthcouncil.org/sites/default/files/Representativeness%20in%20Patient%20Engagement.pdf>



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