Advancing the Science of Patient Input

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Workshop

Cyndi Grossman, PhD
Director, Science Patient Input

FasterCures
A CENTER OF THE MILKEN INSTITUTE

FasterCures Report 2015

ENRICHED UNDERSTANDING OF:

- Disease burden
- Patient journey
- Unmet medical need
- Patient preferences
- Natural history
- Subgroups
- Patient-centered outcomes and endpoints

KEY CONCEPTS TO ELICIT:

- Symptoms experienced
- Chief complaints (most significant or serious symptoms that cause individual to seek healthcare)
- Burden of managing or living with a condition
- Impacts on daily living and functioning
- Strengths and weaknesses of currently available therapeutic options
- Experience of progression, severity, and chronicity
- Views on unmet medical need
- Minimum expectations of benefits
- Maximum tolerable harms or risks
- Acceptable tradeoffs
- Attitudes toward uncertainty
- Decisions regarding care that patients might encounter

DATA SOURCES TO EXPLORE:

DIRECT
- Unstructured personal narratives
- Focus groups
- Patient advisory boards
- Structured interviews
- Ethnography studies
- Online communities
- Surveys
- Patient registries
- Patient-preference studies
- Clinical trials
- Wearables and mobile devices

INDIRECT
- Social media listening
- Electronic health records
- Adverse event reports
- Claims data
- Published literature
- Conference presentations

Science of Patient Input

Examples of good practices in Patient Engagement

- *Practice of patient engagement: CTTI, PCORI, NHC, DIAB, BEI—necessary and should have metrics or ways to measure success, but this is different from*

Examples of initiatives to drive SPI research methods, data collection, and use

- *Research needed to systematically collect and use input from patients to answer key patient-centered questions*

Initiatives driving good practices in PE

CTTI Patient Groups and Clinical Trials (PGCT) Project (2015)

- *Set of tools for patient groups to identify and assess their assets, external relationships, and internal structure. Also, series of recommendations and best practices for incorporating patient group engagement in clinical trials.*

DIA & Tufts Center for the Study of Drug Development Study (2016)

- *Study with insights on the metrics, benefits, barriers, and costs of patient-centric initiatives.*

National Institutes of Health (NIH) & National Center for Advancing Translational Science (NCATS) Toolkit (2017)

- *The toolkit shows patient groups how to establish registries, drive development, work with the NIH/FDA, and conduct post-market surveillance*

Initiatives aimed at regulatory & product development

National Health Council (NHC) & Genetic Alliance (GA): FDA Guidance White Paper (2016)

- *White paper, Integrating the Patient into the Drug Development Process: Developing FDA Guidance, that produced key findings, themes, and priorities from the discussion for the FDA's guidance document.*

Biotechnology Innovation Organization (BIO) & Parent Project Muscular Dystrophy (PPMD) Report (2016)

- *A report, Best Practices for the Development of Disease-Specific Patient Preference Studies, designed to inform development of patient preference studies and their use in drug development and regulatory processes.*

Initiatives aimed at data systems and capacity building for SPI

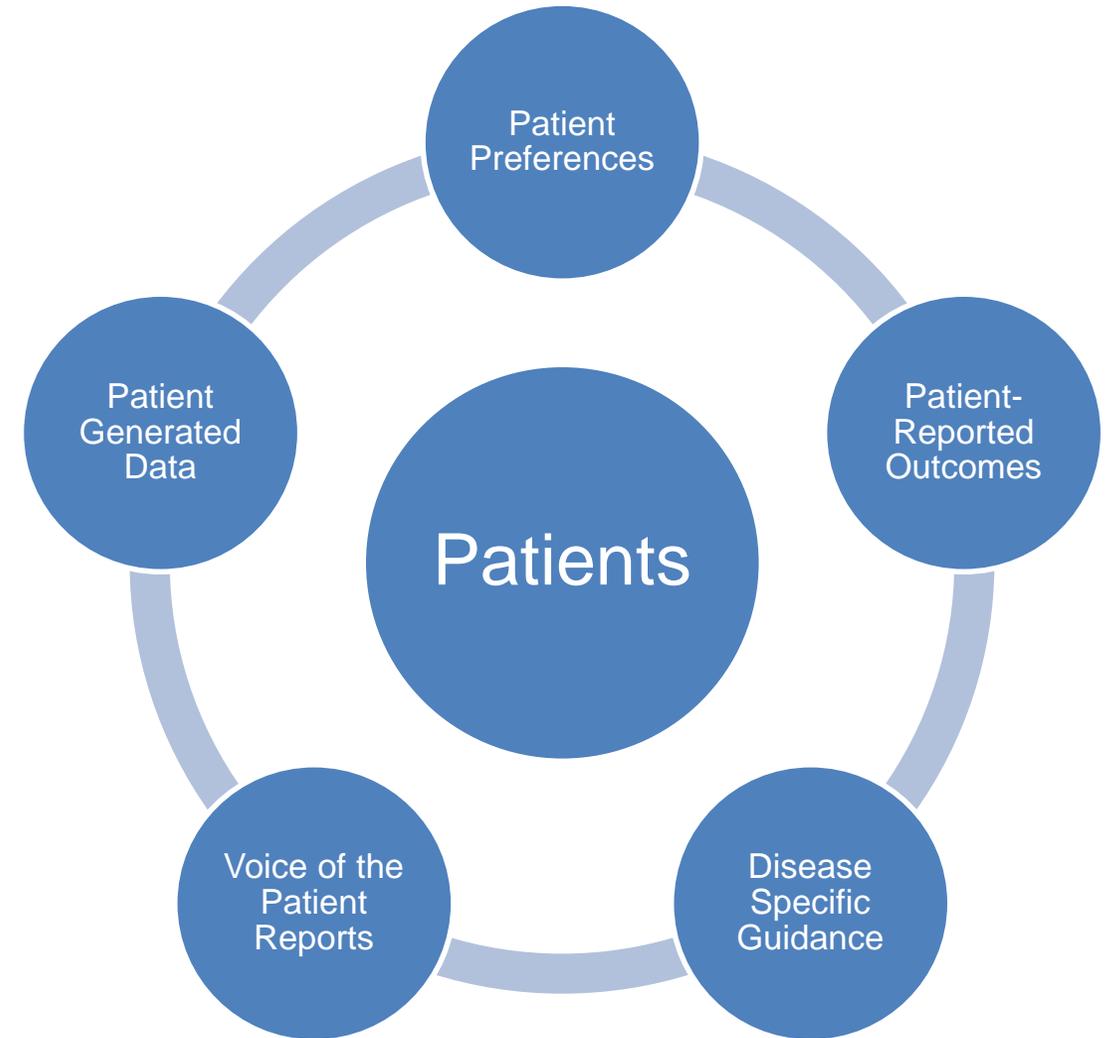Genetic Alliance: Platform for Engaging Everyone Responsibly (PEER) (2016)

- *PEER, an adaptable platform, allows patients to easily, quickly, and safely share specified health information with fellow patients and researchers.*
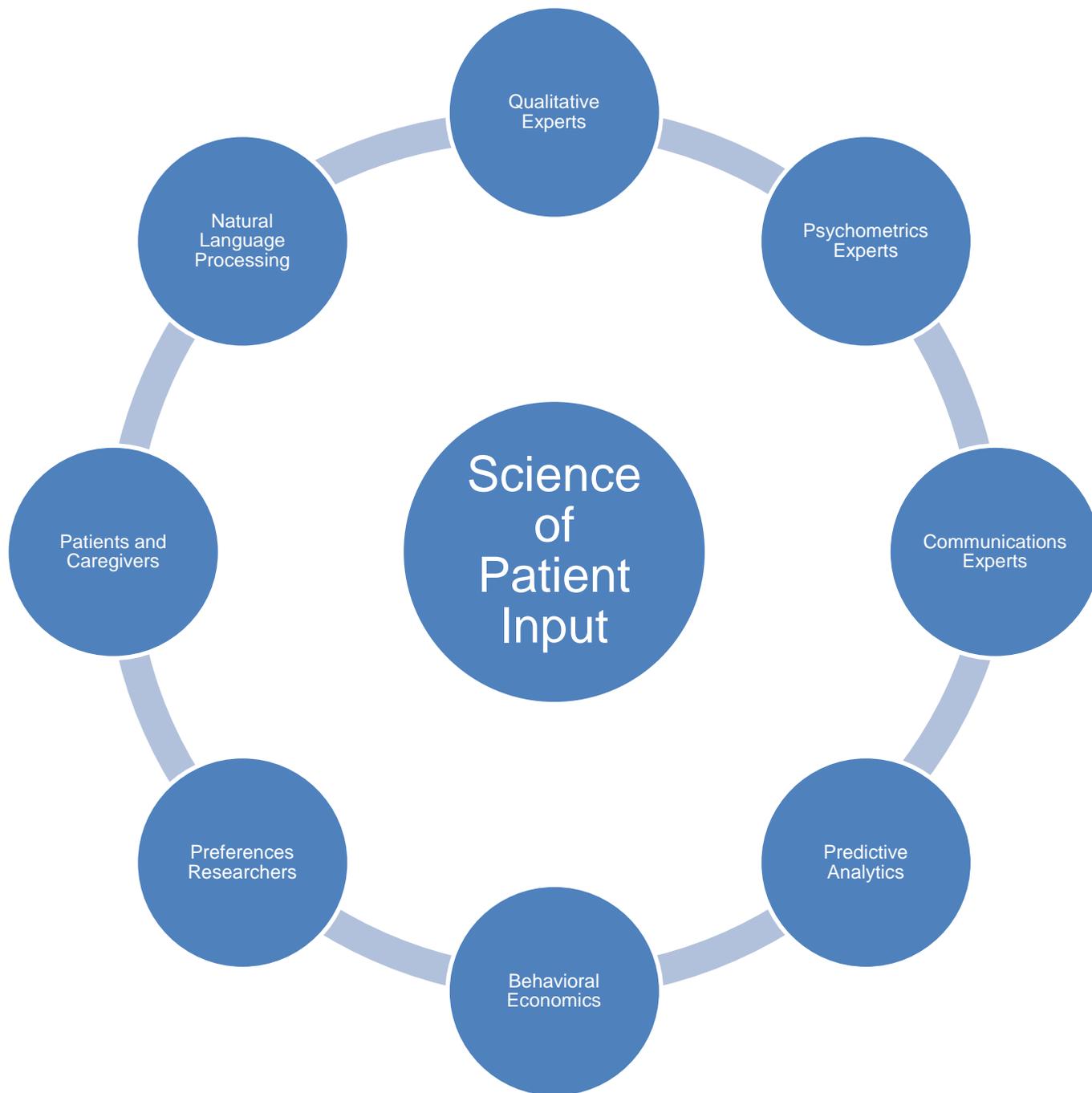
International Academy of Health Preference Research (IAHPR): Health Preference Study and Technology Registry (HPSTR) (2017)

- *Built an open-access, web-based database that hosts information on health preference studies and technologies.*

What we are hearing from the SPI field:

- Need to include patient input earlier on in the product lifecycle

- May be differing needs for data rigor, validity, representativeness depending on *where* in the product lifecycle and *what* you are trying to understand or determine.

- Regulatory decision-making is important, but shouldn't be the only consideration

- Building a science of patient input: what are the knowledge gaps, what capacity is needed to fill the gaps (workforce/systems)?

Patient Preferences

Patient-Reported Outcomes

Patients

Patient Generated Data

Voice of the Patient Reports

Disease Specific Guidance

Coming together is a beginning. Keeping together is progress. Working together is success. *--Henry Ford*