Patient Preference in the Total Product Life Cycle (TPLC)

Patient preference data can be pre-market and post-market data. Discuss where in the TPLC patient preference information can be included to impact/ improve medical devices for the public. Topics to discuss include clinical trial design, current collaborations, and novel external approaches.

For the online webcast: *Please submit your questions to the panel via the chat box. The online hosts will be collecting the questions during the session to be brought to the panel moderator during the panel discussion.*

Moderator: ***Kelly Slone***
Project Lead for Patient-Centeredness
Medical Device Innovation Consortium (MDIC)

Panel: ***Andrea Furia-Helms, M.P.H.***
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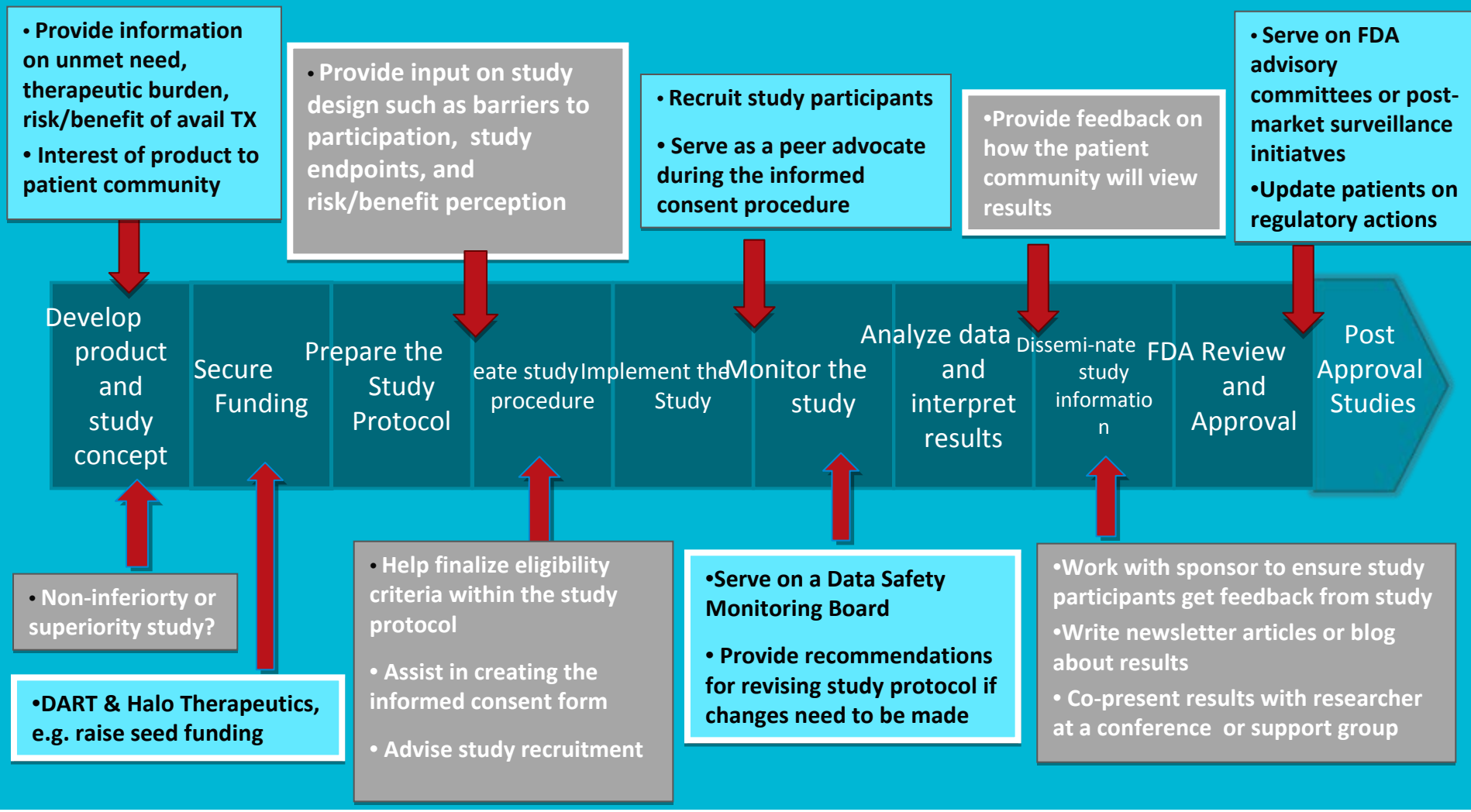
Patient Engagement Across the Continuum of the Life Cycle of a Product and its Associated Studies



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Patient Preference in the Total Product Life Cycle

David H. Hickam, MD MPH
Director, Program on the Assessment of Prevention, Diagnosis and Treatment Options
Patient-Centered Outcomes Research Institute (PCORI)

Patient Preference Initiative Workshop
Silver Spring, MD
September 19, 2013

What is Patient Centered Outcomes Research?

Patient-Centered Outcomes Research (PCOR) helps people and their caregivers communicate and make informed health care decisions.

PCOR is a type of comparative effectiveness research.

PCOR has the following characteristics:

•Actively engages patients and key stakeholders throughout the research process.

•Compares important clinical management options.

•Evaluates the outcomes that are most important to patients.

•Addresses implementation of the research findings in clinical care environments.

Challenges in Using Patient Preferences for CER

- Outcomes that are important to patients often require direct reporting (Patient Reported Outcomes or PROs).
- Validated instruments for PROs are often lacking.
  - Studies may require a preliminary phase to develop instruments.
- Avoid selective use of outcome measures.
  - Pre-specify all outcomes in protocol.
- Synthesis of multiple outcome measures can be a challenge.

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Patient Preference Initiative

Venture Capital Perspective on FDA's Patient Preference Initiative

Jack W. Lasersohn

Partner, The Vertical Group

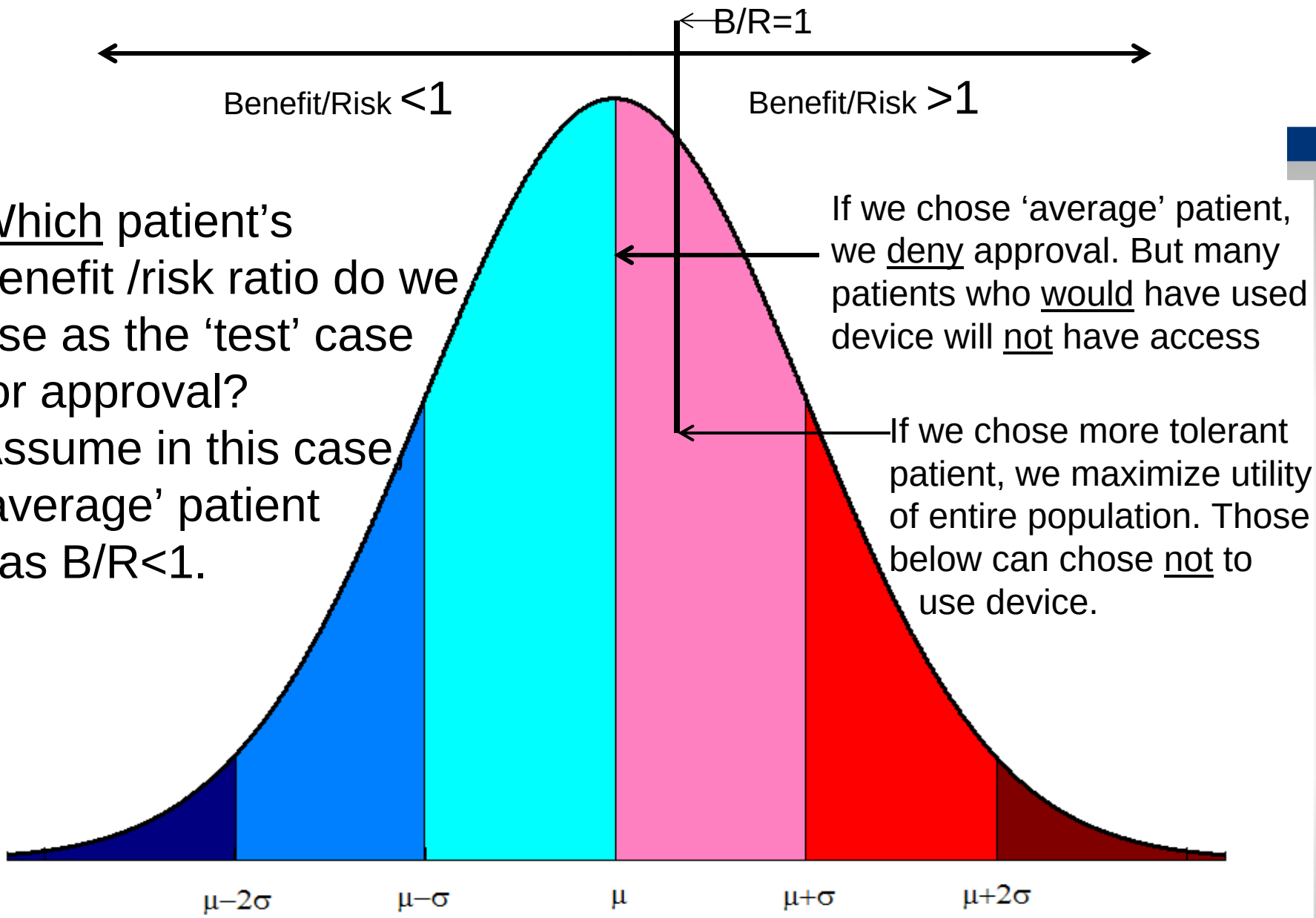
September 2013

Why does a Patient-Centered analysis matter to innovators?

- 'First' generation technology often has benefit/risk ratio that most patients will value at less than 1, because benefits are small and risks large.

- But some patients may have a benefit/risk greater than 1. For example, patients who could not tolerate open surgery valued first generation PTCA, percutaneous valves, AAA stent grafts and carotid stents.

- Innovators target these patients as their 'beachhead market' and need to know that FDA will agree.



Which patient's benefit /risk ratio do we use as the 'test' case for approval? Assume in this case 'average' patient has B/R<1.

If we chose 'average' patient, we deny approval. But many patients who would have used device will not have access

If we chose more tolerant patient, we maximize utility of entire population. Those below can chose not to use device.

Number of patients with B/R ratio for a specified therapy

Patient-centered analysis: Which patient should be the proxy?

- The patient ‘proxy’ for approval for any device should be any set of rational patients in the indicated population with a benefit risk ratio greater than 1 (for the outcomes associated with the device).
- The proxy is not the ‘average patient ‘ or even ‘most’ patients.

- Patients with lower benefit /risk ratios can chose not to use the device. This maximizes ‘utility’ for the entire population.

Patient Considerations in Development

Jonathan Morris, Ph.D.
VP Quality
Medtronic Endovascular Therapies

September 19, 2013

How do we decide what focus on from a development perspective?

First priority is to provide safe and effective therapies to patients.

New therapies must address one or more of the universal healthcare needs as a first step:

- Improve Clinical Outcomes
- Expand Access

Direct impact on patient health and well-being

- Optimize cost and efficiency

Indirect impact on patient satisfaction via reduction in overall health care costs

How do we make this a reality?
Through the Product Development Process

Stakeholder Inputs
Customers
Patients
Regulators
Administrators
Internal

- Prioritization
- Affinitization

Requirements
Specifications
Design

Check-in with stakeholders

Build & Test

Recheck-in with stakeholders

Clinical Trial

Regulatory Approvals

Medtronic

Panel Questions

- Where do you start in integrating patient preferences?
- How do you integrate patient preferences into clinical trial design?
- How could patient preference data be collected and used for post-market and compliance issues?
- What disease areas or device types are best suited for the patient preference approach?
- What additional safe guards should FDA consider when including patient preference information into its regulatory decision-making?
- What do you propose as the first steps for the patient preference approach?

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