

Embedding the Patient Voice in Endpoint Design

A Structured and Quantitative Approach



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Trials often fail to reflect the full treatment value

Pivotal decisions are often based on a single primary endpoint and the consideration of secondary endpoints



This approach can overlook meaningful treatment effects that matter to patients

A robust statistical solution :
The Net Treatment Benefit (NTB)



Comprehensively estimates
treatment effects from multiple
prioritized outcomes

Translates patient preferences
into actionable choices



50+ peer-reviewed publications
1 methodological handbook



Key principles of the Net Treatment Benefit framework

- **Multiple outcomes** are selected for analysis together (efficacy, safety, tolerability, ...)
- **Priority order** of outcomes is key
- We can **capture the preferences** of patients and/or clinicians to understand the most relevant outcomes and priority order

But how to select and prioritize outcomes across all stakeholders?

One2Treat Voice[®] software engages physicians and patients to select outcomes across multiple stakeholder groups



Aggregated results from 30 responses in One2Treat Voice[®]



Article in review

A phase III trial evaluating pCR in patients with HR+, HER2-positive breast cancer treated with neoadjuvant docetaxel, carboplatin, trastuzumab, and pertuzumab (TCHP) +/- estrogen deprivation: NRG Oncology/NSABP B-52

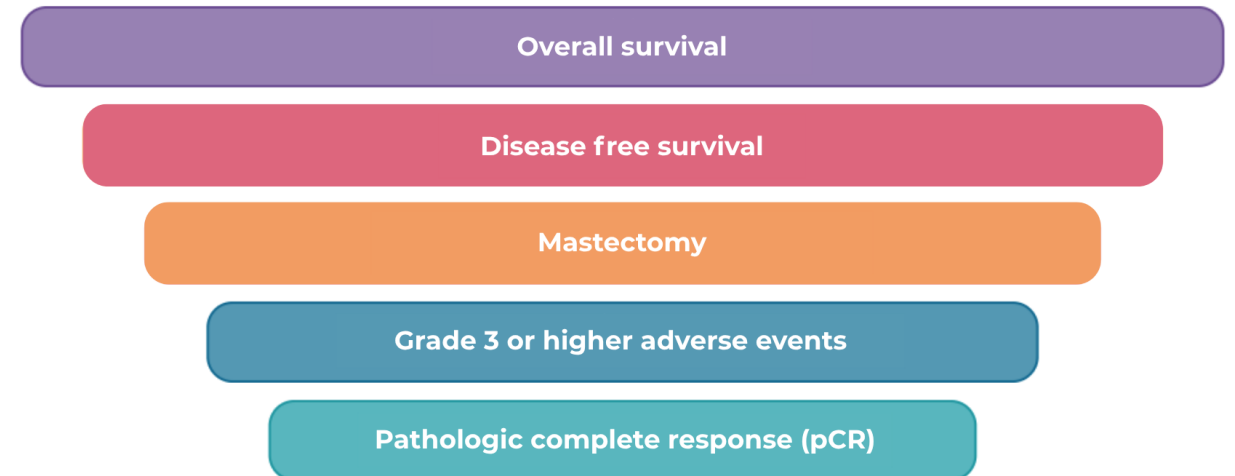
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Background: NRG Oncology/NSABP B-52 is a phase III, multicenter, randomized neoadjuvant therapy trial designed to determine whether the addition of estrogen deprivation to neoadjuvant therapy consisting of docetaxel, carboplatin, trastuzumab, and pertuzumab (TCHP+ED) yields a greater rate of pCR (breast and nodes) than TCHP alone.

Methods: A total of 315 patients (pts) were randomly assigned between January 15, 2014 and March 17, 2016 to receive neoadjuvant therapy consisting of TCHP with or without estrogen deprivation therapy. Pts with locally advanced, hormone receptor-positive, HER2+ invasive breast cancer with no evidence of metastatic disease were eligible. Premenopausal women randomized to estrogen deprivation therapy received ovarian function suppression with goserelin (LHRH agonist) or equivalent plus an aromatase inhibitor (AI). Postmenopausal women received an AI.

The determination of pCR was the primary endpoint of the trial. Secondary endpoints included overall survival (OS), disease-free survival (DFS), mastectomy rates, and grade 3 or higher adverse events. The rates of pCR (breast and nodes) were analyzed using Co

A Generalized Pairwise Comparison Test was performed to estimate the Net Treatment Effect (NTE) for each endpoint deemed most relevant. The NTE was estimated in a sequence of endpoints, breast-conserving





Incorporate multiple outcomes into a patient-centric endpoint to:

- Engage stakeholders and **align patients and sponsors**
- Capture multidimensional **patient preferences** instead of relying on a single endpoint
- **De-risk** development by detecting meaningful treatment effects
 - **Better clinical trial endpoints**
 - **Better treatments for patients**

Thank you

Come and try the app for yourself at our booth!



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