For Tough Medical Decisions, Hard Choices Require Hard Facts—Not Conventional "Wisdom"

written by Robert McNutt, M.D. | December 7, 2017

What matters in medical decisions is what we *know*, not what we *think*.

In the late 1980's I cared for a pregnant woman with breast cancer. Breast cancer is the most common form of cancer in pregnancy, but uncommon in frequency, occurring in about 1 in 3,000 pregnant women. Providing and receiving treatment is certainly a complex emotional experience; at that time, uncertainty about how to treat was the norm. The woman had a mastectomy but did not take chemotherapy based on concern for her baby.

Three months after her delivery, now receiving chemotherapy for her aggressive breast cancer, the woman asked me to consider treating her newborn child with "mild" chemotherapy—a contrarian idea, given her reluctance to expose her child while in utero. Her reasoning, she said, after giving it "lots" of thought, was that it made sense to her; she had cancer at a young age and reasoned her child would, also. In her mind it was rational and reasonable to give her

infant treatment.

Fear and depression clearly fueled her concern. The woman would not live to see her child's second birthday and wanted to do what she could. But there was no evidence of benefit to the baby, making her request irrational. So, I did not comply. Indeed, what would you have thought of me if I had?

Proof of Treatment Benefit Is Essential Before Treating

In 1882, a surgeon reasoned that removing a woman's cancerous breast, nodes and muscles was the way to eradicate her breast cancer. There was no proof of benefit, but the idea spurred action. That idea and the radical surgery persisted as the treatment of choice, even after the publication of a randomized trial (NSAPB-04) in 1974 showing that more surgery was not better than less; about 5,000 radical mastectomies were still performed in 1983, a century after the first. What should we think of physicians who acted without proof of benefit and, even, after proof of no benefit?

In the 1990's more than 40,000 women received high-dose chemotherapy and bone marrow transplants if they had breast cancer. Some of those women died of treatment; I know so because I knew a few who suffered that fate. But conventional "wisdom" prevailed. Doing more and more to a woman with breast cancer was the default treatment philosophy, compelled by politics and legal threats for not performing the "best," and most—despite eventual publication of randomized trials showing that more and more was actually less. All this wrangling occurred without knowing if the treatment plan was better than others. What should we think of those physicians, lawyers and insurers who forced compliance and then complied with an idea rather than knowing what was best?

There is data on bilateral mastectomy for Ductal Carcinoma in Situ (DCIS), but no information, as comparative studies with unilateral mastectomy have not been done. I was consulted by several women who were considering complying with the bilateral procedure based on the idea that getting rid of everything might be good for them. Their physicians had proposed the procedure.

What should we think of these physicians for proposing an unproven procedure based on an idea? Acting without knowing, in my view, should be considered an abdication of professional responsibility. A professional obligation includes informing patients that there is *no evidence of benefit* for some treatment plans, and then not proposing the plan.

Medical Decisions Must Be Based on Sound Research Comparing Treatment Outcomes

The only way a decision can or should occur is if there is a balance between compared options. These compared options must be examined in randomized studies that give the best chance of determining the independent contribution of one intervention versus another. Ideally, the balance between treatment outcomes and complications will become clear: some treatment will be shown to contribute independently to better disease-related outcomes that outweigh any treatment-related complications. The differences in outcomes of disease and treatment compete for a patient's attention and ultimate choice.

Without such comparisons, no choice should be made. Ideas don't suffice; it doesn't matter whether the ideas are the physician's or the patient's.

How many hundreds of thousands of women underwent radical mastectomy before it took only 1,700 in a randomized trial to show that this treatment offered no benefit and increased harm? What about high dose chemotherapy and bone marrow transplant? Six randomized trials, reviewed in 2005, including a cumulative total of 850 women, showed high-dose treatment offered no significant benefit. Nonetheless, studies kept trying to prove the value until, finally, 14 randomized trials involving 5,600 women came to the same conclusion. Far more women underwent the procedure, not knowing what was best, than the actual number required to find out what truly isn't best. This should be a lesson to us all. Knowing is better than not knowing, and acting without knowing may be the worst a profession can do.

Physicians Should Not Allow Treatments of Unproven Benefit

I am writing this blog with a purpose: to make physicians and patients uncomfortable about acting on beliefs without evidence from comparative research trials. I believe patients are the best decision makers, but that will be demonstrably true only if they are informed and allowed to decide.

Physicians need to be on board, as well. They should not allow treatments of unproven benefit and, instead, they should demand study over action. There are too many examples of the failure of "good ideas" to subsequently help patients. What matters is what we *know*, not what we *think*. Informing patients that no evidence exists while promoting a dutiful process of scientific inquiry could be a powerful way to change medical care for the better.

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