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Patients as partners in innovation

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ABSTRACT

As the culture of medical practice has evolved, so has the relationship between the physician and patient. This is decidedly true with regards to the introduction of innovative therapies, especially in the surgical arena. A critical challenge is identifying and defining innovative therapy. Is the proposed treatment an incremental change, a research proposal, or more commonly someplace in between? This gray area creates a transition zone commonly referred to as innovative therapy. Given the complexities of the current landscape of innovation, innovation therapy committees may provide a mechanism to help to guide both physicians and patients through such difficult topics as the process of informed consent, managing conflicts of interest, and how to evaluate the outcomes of innovative therapies. As surgical innovation remains critical to the advancement of care, it must occur in a transparent partnership with patients, under the eye of guiding entities, aimed at ultimately improving outcomes and care.

Introduction

The culture of medical practice has undergone significant change in the past several decades. The relationship between physician and patient has moved from a patriarchal model towards one of equals. This has not occurred in a vacuum, but reflects many other societal changes that have resulted in a flattening of hierarchies and a move towards a more inclusive community. This change in the physician–patient relationship over time, from one of dependency towards a more equal partnership, has illuminated the challenges and obligations both physicians and patients face when they engage in a discussion of innovative therapy.

What is innovative therapy?

Advances in medical care, and particularly advances in surgical treatment, have always been dependent upon the incremental improvements in practice brought about by innovative therapy. By the very nature of their practice, surgeons innovate constantly and unexpectedly. This is exemplified by surgery on infants with congenital anomalies where the anatomy and circumstances can vary from patient to patient. In these situations, there may have been no plans to perform an innovative procedure, and the usual standard of care has been altered incrementally, in response to patient need. This situation represents thoughtful, attentive patient care with a slight departure from the accepted standard (i.e., the "practice of medicine"), for which the risks can be reasonably estimated and the likely clinical outcomes are known.

On the other end of the spectrum is "research" which is an activity designed to generate knowledge by testing a hypothesis and developing conclusions which may (eventually) be generalizable to other patients. Although there may be a potential for individual patient/subject benefit, that is not the primary intent of the intervention. Generally for the patients' protection, this kind of innovation is subject to oversight by Institutional review boards (IRBs), which codify the engagement of patients as research subjects. Between incremental innovation and research is a "gray area" or "transition zone" referred to as "Innovative Therapy." Innovative therapy describes a procedure that represents a degree of novelty that includes the possibility of unforeseen outcomes with the potential for generalizable knowledge to be collected, yet unlike clinical research, the direct intent of the innovation is to benefit the patient. While innovation is critical for the advancement of surgical care as a whole, the risks faced by the individual patient receiving innovative therapy can be substantial, especially early on in the acquisition of procedural experience. A physician's fiduciary responsibility to his/her patient, and the desire to maintain the trust required to support a true physician–patient partnership, requires that we strive to be transparent in

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differentiating innovative therapy from standard clinical care, and when appropriate, move innovative care to a more rigorous form of evaluation, where the primary intent is understanding treatment outcomes (effectiveness) and risk, rather than treating individual patients. Ultimately, it is the physician who needs to be honest with themselves and the patient in his/her efforts to maintain this transparency.

Institutional oversight: The innovative therapy committee

Innovative surgical therapy and clinical research involving novel surgical techniques or devices bring forward issues that are not routinely considered in evaluating the safety of medical or pharmaceutical research. These include not only the safety of the procedure, but the inherent variability associated with the skill, insight and experience of the surgeon, and the team that will be performing the procedure. The oversight process must consider not only the procedure or device, but also "human factors" such as foundational expertise, specialized training, demonstrated skill and experience, and should be required whether the proposed innovation is completely novel, or has already been shown to be safe and effective by pioneering surgeon innovators elsewhere, but is new to the institution in question. Institutional review boards may have limited knowledge of these specific variables and cannot readily make informed decisions on their safety and use. Additionally, many IRBs may be overwhelmed with providing oversight for formal clinical research protocols, which are subject to significant regulatory requirements. The surgeon or team requesting privileges to perform an innovative procedure should summarize the existing clinical or experimental evidence, and offer some justification of the anticipated safety and effectiveness of the proposed procedure. Additionally, the review should describe what preparatory work has been done via courses, simulators, and by animal or cadaver labs to try and maximize the safety of the new approach. Innovative therapy committees should be composed of physicians with foundational understanding of the proposed treatment and alternatives (if any), and other stakeholders including social workers, patient advocates, clinical ethicists, and legal representatives when appropriate. The Innovative Therapy committee should also review inclusion and exclusion criteria, the consenting process and should review the consent form for clarity and transparency. All real or perceived conflicts of interest by any member of the surgical team must be declared and discussed. These can be significant when a new device is being utilized. Finally, the committee (or a group designated by the committee) should be prepared to evaluate patient outcomes at appropriate intervals, and help make a determination as to whether the innovative procedure should be trialed in more patients, whether unanticipated outcomes suggest the need to impose a procedural moratorium, or if further evaluation is more appropriate in the context of a clinical trial. They may also be tasked with reaching out to other institutions to confirm their experience in the therapy being proposed. The committee may also comment on how best to disseminate information and education on the innovative procedure regardless of the outcome.

The process of informed consent

The participation of patients and their families in shared medical decision-making regarding clinical care is embodied in the informed consent process. At its best, informed consent discussions allow the physician to share information about the disease process and the various therapeutic options while the patient provides information on their understanding of their illness, and its impact on their life. Productive informed consent discussions depend on physician transparency in sharing the extent of our understanding of the disease and its prognosis, the risks and benefits of various treatment options, and the generally expected outcomes of the recommended options. The process of informed consent provides an opportunity for the physician to consider where along the spectrum from standard clinical care, through to clinical research, the proposed intervention resides. As interventions move towards the research end of the spectrum, there needs to be increasing clarity about what we do not know regarding risk and outcome. This is vitally important to minimize the risk of therapeutic misconception, where physicians, patients and families may be confused regarding the therapeutic intent of a research procedure. Clinical research has very clear guidelines, dictated by federal regulation, regarding the requirements for informed consent, mandating the inclusion of information that the procedure represents research, with a description of the foreseeable risks, any potential benefits, and any alternative procedures or courses of treatment. This guidance may be helpful in considering informed consent discussions surrounding innovative procedures that are not part of a formal clinical trial and should generally be crafted with the assistance of an innovative therapy committee.

The preparation for this discussion should provide the physician the opportunity to reflect on his/her intent in proposing the innovative intervention—is he/she proposing this innovative therapy as the best option available to treat or ameliorate the patient's disease, to have an opportunity to learn or refine new technical skills, or in the hopes of learning more about a new therapeutic option. These are very different indications for introducing an innovative procedure and should be clearly identified in the course of an informed consent discussion as they may influence the patient's willingness to consent to the procedure. When the balance of surgeon intent "tips" in favor of acquiring new skills or creating an evidence base for the continued utilization of the procedure, it should be considered "research," and be subject to IRB oversight.

More commonly, challenges arise when there is insufficient experience with the procedure to confidently state what benefits the patient will gain, and how those are balanced against potential risks. With less data to share and consider, there is the potential for both the physician and the family to rely instead on their perceptions of the risks and benefits of the proposed intervention. The phenomenon of "Optimism bias" refers to the unwarranted belief in the efficacy of new therapies, and may exist in both the surgeon-innovator and his or her prospective patient. For the surgeon who has invested intellectually (and sometimes financially) in the development of a novel treatment, the desire to demonstrate benefit by the creation of clinical evidence may lead to advocacy, or even excessive, frequently subconscious encouragement for the novel treatment over its conventional alternative. In contrast, a surgeon with equivalent knowledge and expertise, but without personal investment in the procedure is more likely to honestly question the existence of clinical equipoise, and may present the innovative procedure being considered in a more appropriate, but perhaps less favorable light. For this reason, some have suggested that an objective third party, such as a surgeon-colleague not involved in performing the innovative procedure, should be involved in the primary patient discussion rather than the operating surgeon. Alternatively, an innovative therapy committee could meet with the family collectively to discuss the procedure and its alternatives in the presence of the operating surgeon.

Patients can also fall victim to optimism bias if they present for surgical consultation having made up their mind that the innovative procedure is better than the traditional alternative. One