

The Open-Ended Investigation: A Method for Regulation of New Medical Services

As medical care¹ becomes increasingly complex and expensive, evaluation of that care becomes more important, particularly with regard to newly developed forms of treatment. Although governmental mechanisms have been established for the screening of new drugs and medical devices, the evaluation of physicians' use of those therapies, an essential component of any comprehensive regulatory plan, has been neglected. The responsibility for monitoring new therapies has remained almost entirely under the auspices of the medical profession, but such self-regulation has not, on the whole, proven effective.

This Note proposes a method for the evaluation of physicians' use of innovative medical services. This method utilizes an open-ended investigation, conducted by a "sponsor" of the new therapy, in which only physicians meeting specified qualifications are given "investigator" status and are permitted to use the new therapy while it is being studied. Such an arrangement would serve to improve evaluation of the effectiveness of new therapies as they are actually applied by large numbers of physicians. At the same time, it would limit access to those therapies to the physicians demonstrating competence in their use.

I. The Need for External Evaluation of New Medical Services

Present methods for regulation of newly developed drugs and devices²

1. For the purposes of this Note, the terms "medical services" and "medical care" will include all therapeutic measures that a physician might use to care for a patient, including drugs, medical devices, and surgical procedures. The terms "medical treatments" and "therapies" will refer more narrowly to drugs or devices and to the physicians' competent use of them. For instance, penicillin is not a "therapy" unless it is competently administered to a patient who needs the treatment. The term "procedures" will refer to treatment methods involving the activity or work of a physician that does not depend on the use of a particular tool. For example, the implantation of a prosthetic joint would be a therapy while an appendectomy would be a procedure. Finally, the term "physician practices" will refer to the physician's behavior in delivering either procedures or therapies.

2. Federal regulation of drugs began with the Act of June 26, 1848, ch. 70, 9 Stat. 237, which provided for custom house inspection of imported medicines for "quality, purity, and fitness for medical purposes." The Food and Drug Act of 1906, Pub. L. No. 59-384, 34 Stat. 768, prohibited the manufacture of, or interstate commerce in, "adulterated" or "misbranded" drugs. The Food Drug and Cosmetic Act (FDCA) of 1938, Pub. L. No. 75-717, 52 Stat. 1040 (current version at 21 U.S.C. §§ 301-392 (1976)), expanded the terms "adulterated" and "misbranded" to include any drug whose labelling "is false or misleading in any particular." 21 U.S.C. § 352(a) (1976). The 1938 Act also provided for the premarket review of new drugs to ensure safety. The Drug Amendments of 1962, Pub. L. No. 87-781, 76 Stat. 780 (codified in scattered sections of 21 U.S.C. (1976)), required that a new drug be evaluated both for safety and for efficacy before approval for marketing was given. Under the 1962 amendments, medical devices were subject only to the "misbranding, adulteration, and labelling" provisions of 21 U.S.C. §§ 351-352. With creative interpretation, however, some medical devices were classed as "new drugs" and therefore were subject to premarket approval. *See United States v. An Article of Drug . . . Bacto-Unidisk*, 394 U.S. 784, 793-801 (1969) (reviewing history of device coverage under FDCA and upholding FDA's treatment of device as new drug). The necessity

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do little to monitor or control the actual application of those therapies by physicians once the therapies are released for general use.³ Instead, present regulatory efforts—ranging from individual self-evaluation to state licensing—focus primarily on evaluating the safety and efficacy of new drugs or devices as ideally utilized.⁴ Little effort is made to control or evaluate the actual use of therapies after they are released to the market.⁵ Newly developed surgical procedures⁶ are even less closely regulated.⁷

Two problems arise as a result of this failure to police the implementation of new medical therapies. First, the evaluation of the therapies them-

for premarket evaluation of increasingly complex medical devices resulted in the Medical Device Amendments of 1976, Pub. L. No. 94-295, 90 Stat. 539 (current version at 21 U.S.C. §§ 360c-360j (1976)).

On the history and operation of the Food and Drug Administration (FDA), see generally J. MASHAW & R. MERRILL, *THE AMERICAN PUBLIC LAW SYSTEM* 459-542 (1975), and Munch, *A Half Century of Drug Control*, 11 *FOOD DRUG COSM. L.J.* 305 (1956).

3. New medical treatments developed in the United States undergo extensive scrutiny before they may be released for general use. All new drugs must receive premarketing approval from the Food and Drug Administration, see *supra* note 2; a similar requirement was created for medical devices in 1976, see *id.*; Note, *infra* note 33, at 1110. The FDA bases its decision to approve or disapprove a drug or device on data supplied by the manufacturer, who bears the burden of proving that the new therapy is safe and effective for its proposed use. See Ames & McCracken, *Framing Regulatory Standards to Avoid Formal Adjudication: The FDA As a Case Study*, 64 *CALIF. L. REV.* 14 (1976) (description of FDA procedure as effective means of using summary judgment on manufacturers' data to reduce burden of administrative hearings). Once the FDA approves an application, however, the drug or device becomes available for use by all licensed physicians; there is little further external control over their use of the therapy. See *infra* note 5.

4. The FDA currently evaluates applications for premarket approval on the basis of information generated by small groups of investigators carefully selected by the manufacturer of the drug or device. In such circumstances, the manufacturer has an incentive to select unusually skilled, if not positively biased, physicians to conduct the studies. See R. MERRILL & P. HUTT, *FOOD AND DRUG LAW* 406 (1980) (noting suspicion that investigators carry pro-manufacturer bias).

5. An approved drug may be used by any licensed physician in any lawful manner; this policy reflects the traditional freedom of physicians to tailor a therapy to a particular patient. The FDA has expressed concern about the prescription of approved drugs for unapproved uses, see 37 *Fed. Reg.* 16,503-04 (1972), but it does not have clear authority to control such behavior. See Kessler, *Regulating the Prescribing of Human Drugs for Non-approved Uses Under the Food, Drug, and Cosmetic Act*, 15 *HARV. J. ON LEG.* 693, 693-760 (1978) (providing general overview and suggesting that FDA be given authority to disapprove particular uses). Attempts have been made to control unapproved uses by instituting mislabelling charges, with mixed results. Compare *United States v. An Article of Drug . . . Diso-tate*, [1976-1977 Transfer Binder] *FOOD DRUG COSM. L. REP. (CCH)* ¶ 38,086 (E.D. La. 1976) (prohibiting advertisement of approved drug for non-approved use) with *United States v. Evers*, 453 *F. Supp.* 1141 (D. Ala. 1978) (holding on similar facts that same physician could use the drug on his own patients without FDA approval). The FDA has generally been unsuccessful in attempting to control the distribution of approved drugs that are used for non-approved purposes. See *American Pharmaceutical Ass'n v. Weinberger*, 377 *F. Supp.* 824 (D.D.C. 1974) (FDA efforts to limit distribution of methadone to specific treatment centers blocked because controlled substances held within jurisdiction of Justice Department). The agency concedes that misuse is largely uncontrollable until it becomes so widespread that it endangers the public health. 37 *Fed. Reg.* 16,503-04 (1972).

6. It is not uncommon for physicians to perform unproven procedures that require both new medical devices and new medications simultaneously. For instance, a coronary by-pass procedure requires the use of a by-pass pump to aerate and circulate blood while complex combinations of medications are used to control the irritability of the heart. See Bethune, Collis, Hardy & Latimer, *Anaesthesia for Coronary Artery Surgery*, in *TOWARDS SAFER CARDIAC SURGERY* 267-77 (D.B. Longmove ed. 1981); see also M. MILLMAN, *THE UNKINDEST CUT* 215-49 (1978) (discussing coronary by-pass as overused and unproven procedure).

7. See *infra* p. 567.

selves is flawed because it fails to take into account the effect that less-than-ideal performance can have on the results obtained. Studies that extrapolate from the results of a few selected investigators in order to predict the worth of a new therapy as used by the general physician population are thus based on unwarranted assumptions of physician homogeneity in skill and practice.⁸ Second, after the new drugs and devices are approved for marketing, no central control exists over physician access to those therapies, so that practitioners with inadequate training or experience may use them in an unsafe or ineffective manner.⁹ The dangers inherent in the release of a therapy to the general physician population is one of the factors that tends to make the FDA extremely risk averse in evaluating new forms of treatment, a posture that some claim has resulted in the denial to the public of important medical benefits.¹⁰

A. Individual and Professional Self-Evaluation

Individual self-evaluation cannot, in general, be relied upon for effective control of physician practices, especially where newly developed therapies are concerned.¹¹ In any clinical situation, the treatment that is chosen by a physician, the skill with which it is administered, and the results of the therapy are greatly influenced by the diagnostic and therapeutic skills, professional biases,¹² and training and experience of the individual physi-

8. See *supra* note 4. Physicians vary in the specialties for which they are trained, see Grad, *The Antitrust Laws and Professional Discipline in Medicine*, 1978 DUKE L.J. 443, 472 (describing specialty societies and their procedures), and in competence, S. LAW & S. POLAN, PAIN AND PROFIT: THE POLITICS OF MALPRACTICE 28-30 (1978) (noting examples of substandard practice by physicians).

9. Licensed physicians may use any approved drug for any legal purpose in their practices. See *supra* note 5. Thus, an ophthalmologist could prescribe contraceptives, or a psychiatrist could order insulin, although both would be working outside their areas of expertise.

10. See, e.g., *Tutoki v. Celebrezze*, 375 F.2d 105 (7th Cir. 1967) (plaintiff claimed FDA engaged in illegal acts to prevent distribution of Krebiozen, a cancer drug); Hutt, *Laetrile Decision Ignores Constitutional Question*, Legal Times of Washington, July 2, 1979, reprinted in R. MERRILL & P. HUTT, *supra* note 4, at 434 (recounting denial of access to purported cancer drug); Kelly, *Bridging America's Drug Gap*, N.Y. Times, Sept. 13, 1981, § 6 (Magazine), at 100 (criticizing FDA for being too cautious); Peltzman, *An Evaluation of Consumer Protection Legislation: The 1962 Drug Amendments*, 81 J. POL. ECON. 1049 (1973) (concluding that benefits lost by delaying use of effective new drugs greatly exceed costs avoided by preventing use of harmful or ineffective drugs).

11. That physicians need help in judging the worth of a treatment was recognized in the Congressional considerations on the Medical Device Amendments of 1976. According to the Senate Report on that act, "[m]any devices are so intricate that skilled health professionals are unable to ascertain whether they are defective" and there is a "need for regulation [of devices] to assure . . . that health professionals can have more confidence in the performance of devices." S. REP. NO. 33, 94th Cong., 1st Sess. 5, 6, reprinted in 1976 U.S. CODE CONG. & AD. NEWS 1070, 1075.

12. Professional bias in medicine occurs at three levels: (1) bias by the medical profession against practitioners who are not medical doctors, (2) biases among specialties within doctors' ranks, and (3) biases within specialties between advocates and opponents of specific therapies.

Licensed professions generally attempt to limit competition by claiming exclusive competence to perform certain tasks. See J. LIEBERMAN, *THE TYRANNY OF THE EXPERTS* 135-58 (1970). The medical profession, through the American Medical Association (AMA), certainly fits the pattern by claiming exclusive authority to define what is legitimate health care. See J. BURROW, *AMA: VOICE OF*

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cian, and also by the highly personal nature of the physician-patient relationship.¹³ To evaluate his own performance in the use of a new medical service, an individual physician must know (1) the general effectiveness of the device or therapy as used competently, (2) the special circumstances that might increase or decrease the safety or efficacy of the treatment for his patient, and (3) the effect of any external factors that might influence his patient's course. Current drug and device evaluation procedures furnish data on only the first point.

Evaluation of physician practices by the medical profession as a whole is equally unreliable. None of the major professional medical organizations¹⁴ currently makes any systematic effort to evaluate on a continuing basis the quality of the patient care provided by its members.¹⁵ Moreover, were such evaluations undertaken, professional organizations might well

AMERICAN MEDICINE 108-131 (1963) (describing AMA crusade against "quackery," as AMA defined it); Comment, *Restrictions on Unorthodox Health Care in California: A Legal and Economic Analysis*, 24 U.C.L.A. L. REV. 647, 649, 657 (noting that unorthodox health care is essentially prohibited by any but licensed M.D.'s, who are also pressured to conform to orthodox therapies).

Specialties within the medical profession hold varying therapeutic concepts. For example, pediatricians and otolaryngologists differ on the value of tonsillectomy and adenoidectomy, see *Hearings before the Subcommittee on Oversight and Investigations of the House Committee on Interstate and Foreign Commerce*, 95th Cong., 1st Sess., (1977) (statement of Charles D. Bluestone, M.D., Professor of Otolaryngology, University of Pittsburgh School of Medicine); and internists, radiologists, and surgeons disagree on the proper therapy for breast cancer, each specialty touting its own wares, see Anglem, *Management of Breast Cancer: Radical Mastectomy*, 230 J. A.M.A. 99 (1974) (supporting radical mastectomy); Bonadonna, *et al.*, *Combination Chemotherapy as an Adjuvant Treatment in Operable Breast Cancer*, 294 NEW ENG. J. MED. 405 (1976) (supporting medication as adjuvant therapy for post-operative treatment of breast cancer); Proznitz *et al.*, *Radiation Therapy as Initial Treatment for Early Stage Cancer of the Breast Without Mastectomy*, 39 CANCER 917-23 (1977) (supporting radiation as primary therapy). See also Crile, *Management of Breast Cancer: Limited Mastectomy* 230 J. A.M.A. 95 (1974) (supporting limited mastectomy); Crile, *Management of Breast Cancer: Limited Mastectomy in Rebuttal to Dr. Anglem*, 230 J. A.M.A. 106 (1974) (disputing Anglem article); Mosher, *Letter*, 294 NEW ENG. J. MED. 1344 (1976) (disputing claims of Bonadonna article).

13. The personal nature of a physician-patient relationship may, for example, create a placebo effect that makes evaluation of a medical therapy in that particular context difficult. Any treatment can have positive therapeutic effects on a patient who believes that the therapy will help; the effect is enhanced if the therapist also believes in the treatment. Indeed, it has been argued that until recently most therapies in use had little or no real medical value, and that the positive results obtained from them were the result primarily of faith on the part of physician and patient. See Bok, *The Ethics of Giving Placebos*, SCI. AM., Nov. 1974, at 17-23 (until recently, psychotherapeutic effect of placebos based on belief of both healer and sufferer accounted for positive results of most therapy); Cousins, *The Mysterious Placebo—How Mind Helps Medicine Work*, SAT. REV., Oct. 1, 1977, at 9-16 (examples of placebo action); Leslie, *Ethics and Practice of Placebo Therapy*, 16 AM. J. MED. 854-62 (1954) (definition and use of placebo therapy).

14. Certification by a specialty board is valuable to the reputation of a physician, but such certification is purely voluntary and not a requirement for the practice of a specialty. Grad, *supra* note 8, at 473-75.

15. The evaluative efforts of licensing boards and specialty societies typically focus on initial screening of applicants for membership. Periodic recertification or continuing medical education requirements are sometimes imposed, but, like the initial certification procedure, they involve no evaluation of the physician's actual practice with patients. Grad, *supra* note 8, at 474 ("Only two [specialty] boards, Family Practice and Urology, mention incompetence as a ground for revocation [of board certification].")

tend to focus on detecting deviations from the standards and practices of that group, rather than on evaluating individual physicians against objective or absolute standards.¹⁶

B. *External Forms of Regulation*

Because of the limitations of individual or professional self-regulation, several forms of external evaluation of the medical profession have evolved. The most important of these is state licensing of physicians.¹⁷ Licensing creates an entry-level check on eligibility for the practice of medicine, but provides for little or no continuing evaluation of the quality of practice of the licensed physician.¹⁸ Moreover, licensing requirements are typically administered by medical boards controlled by physicians.¹⁹ The professional disciplinary efforts of such licensing boards usually emphasize statutory and "ethical" requirements rather than the evaluation of medical competence.²⁰ Revocation of a medical license on any ground is uncommon, but revocation of a medical license for incompetence in the practice of medicine is particularly rare.²¹

Other means of external evaluation of physician practices include formal procedures such as Professional Standards Review Organization

16. Any professional organization must determine qualifications for membership, thus establishing norms of conduct. Medical specialty societies, for instance, control the training programs prerequisite for entrance to that specialty. Moreover, the specialties are controlled by those members most likely committed to the biases of the specialty. Deviation from the specialty norms after certification may leave one open to malpractice claims. See *infra* note 81. Early deviation will preclude board certification. Cf. M. FRIEDMAN, *CAPITALISM AND FREEDOM* 157 (1962) (orthodoxy of profession limits experimentation of individual); J. LIEBERMAN, *supra* note 12, at 135-58 (groups try to organize as licensed professions for self-interest of "profession"); Comment, *supra* note 12, at 657 (even holders of unrestricted medical licenses are limited in their choice of remedies to customary practices of their specialties because of tort law and specialty pressure).

17. No one may practice medicine in any state without a medical license. Grad, *supra* note 8, at 454. See also Forgotson, Roemer & Newman, *Licensure of Physicians*, 1967 WASH. U.L.Q. 249 (detailed survey of medical licensing laws); Moore, *The Purpose of Licensing*, 4 J. L. & ECON. 93 (1961) (discussing consumer-based rationale for licensure).

18. S. LAW & S. POLAN, *supra* note 8, at 36; cf. Grad, *supra* note 8, at 457 (most states require no showing of competence for license renewal).

19. Grad, *supra* note 8, at 455. Although the licensing function is technically performed by the state, it is in fact largely controlled by members of the medical profession and is not, therefore, an "external" form of evaluation. Licensing in most states is based on scholastic requirements and written tests, and not on any evaluation of the actual practice skills of the physician. *Id.* Moreover, licensing tests usually focus on the general knowledge of the applicants, not on their knowledge and skill in the specialty to be practiced; the examination for licensure typically comes at the end of medical school, not after specialty training. S. LAW & S. POLAN, *supra* note 8, at 36.

20. See Grad, *supra* note 8, at 459-68 (arguing that professional self-discipline under licensing statutes does more to protect doctors than to evaluate them).

21. From 1973 to 1975, only 134 medical licenses were revoked in the United States; from 1970 to 1975, only eight physicians in the entire nation were disciplined for incompetence. S. LAW & S. POLAN, *supra* note 8, at 31-34. The most common reason for license revocation is drug abuse; mental incompetence is the second most common reason. *Id.* at 32. "Malpractice, even gross malpractice, whether resulting from failing skill and intelligence, habitual failure to pay attention, overwork, or any other cause, is not a common reason for disciplining physicians." *Id.* at 33.

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(PSRO) evaluations²² and hospital staff evaluations,²³ and less regular means such as tort law²⁴ and informal peer evaluations.²⁵ These too have serious limitations.²⁶ PSRO evaluations may be directed more at controlling overutilization of medical services than at ensuring competence in the services delivered.²⁷ Hospital staff evaluations, even when formal, tend to be superficial, biased, or erratic,²⁸ and when informal, surprisingly tolerant of incompetence.²⁹ Tort law sanctions are too indirectly linked to specific patterns of substandard care to deter such behavior effectively, although they may lead to a generally risk-averse attitude in the profession.³⁰ These weaknesses in the external evaluation of physicians'

22. Professional Standards Review Organizations (PSROs) were established in 1972 to determine that payments made under Social Security for inpatient health care were for services that were medically necessary and that met recognized standards of health care. See G. MCLACHLAN, *A QUESTION OF QUALITY* 228-33 (1976) (describing PSRO procedures).

23. Most hospitals seek accreditation from the Joint Commission on the Accreditation of Hospitals (JCAH), although such accreditation is wholly voluntary. As a means of evaluating quality of care, the JCAH has devised the Performance Evaluation Procedure, under which hospitals select diagnoses for evaluation of physician care within that institution. These evaluations are voluntary and are not reported to any public agency. G. MCLACHLAN, *supra* note 22, at 233. The JCAH also scrutinizes hospitals' staffing practices. The physician's qualifications to join a hospital staff and his practice in the hospital are subject to formal review. In practice, however, it is quite difficult to remove a physician from a hospital staff. See A. HOLDER, *MEDICAL MALPRACTICE LAW* 354-61 (2d ed. 1978) (discussing grounds for suspension of hospital privileges); S. LAW & S. POLAN, *supra* note 8, at 51-68 (hospitals and their staff physicians do not have tools or interest to adequately police surgical staff); Grad, *supra* note 8, at 468-72 (physician self-discipline in hospitals in practice rarely leads to removal of incompetent physicians). Evaluation of physician practices is accomplished to some extent through review of applications for hospital staff privileges—that is, for the opportunity to practice medicine and surgery on private patients in the hospital. Such reviews are not always done thoroughly, see S. LAW & S. POLAN, *supra* note 8, at 62-68, although hospitals may increase the rigor of their reviews as the threat of liability for failure to screen physicians becomes more widespread, see Cray, *Due Process Considerations in Hospital Staff Privileges Cases*, 7 HASTINGS CONST. L.Q. 217, 217 (1979) (hospital may be held liable for negligent admission of incompetent physician to staff).

24. See *infra* note 81 (discussing tort law standard of care).

25. Once a physician is admitted to a hospital staff, his hospital records and practices are available for evaluation by fellow members of the staff on a limited basis, but such review is not done routinely in most hospitals. See S. LAW & S. POLAN, *supra* note 8, at 51-77 (discussing lack of adequate peer control of physician competence in hospitals).

26. Institutional Review Boards (IRBs) are another means of external control over physicians' experimentation within hospitals. Any clinical investigation on human subjects that is regulated by the FDA must be reviewed and approved by an IRB. 45 C.F.R. § 46.102 (1980). The IRB is intended to help protect patients and to check the abuse of subjects by investigators. In practice, however, the IRB may be dominated by physicians' interests because members of the IRB and the investigators are likely to be colleagues. Moreover, the IRB requirement is only triggered when a physician announces that he is doing research, not when he simply decides to use a new, unregulated therapy in his practice. See R. LEVINE, *ETHICS AND REGULATION OF CLINICAL RESEARCH* 226-43 (1981) (discussing credibility and effectiveness of IRB's).

27. Although the mandated reviews include a requirement of quality assurance, the program has focused in practice on controlling costs and levels of utilization. S. LAW & S. POLAN, *supra* note 8, at 37; Smits, *The PSRO in Perspective*, 305 NEW ENG. J. MED. 253 (1981).

28. See *supra* note 23.

29. See S. LAW & S. POLAN, *supra* note 8, at 28-50 (documenting physician tolerance of peer incompetence).

30. See A. HOLDER, *supra* note 23, at 407 (physicians may practice defensive medicine and decline to perform a service because of fear of malpractice exposure).

use of common therapies are likely to be compounded where new therapies are concerned, because then there is no standard by which to measure either the therapy or the physician.

Although external evaluation of innovative physician practices has not proven effective in the past, it can be made effective by modifying and applying more generally a regulatory mechanism that is already used, in a different context, by the federal Food and Drug Administration (FDA). This mechanism is the "Investigational Device Exemption," or IDE, which was enacted as part of the Medical Device Amendments of 1976 (MDA).³¹ Using this kind of procedure, a regulatory agency such as the FDA can control the access of physicians to new forms of therapy, and at the same time better evaluate the therapies themselves.

II. The IDE Procedure in Practice

In the Medical Device Amendments,³² Congress extended the regulatory authority of the FDA to reach new medical devices as well as food and drugs. In enacting procedures for the regulation of devices, however, Congress provided for an exemption, in certain circumstances, from the usual premarket approval requirement.³³ This procedure, the Investigational Device Exemption, allows the FDA to withhold judgment on an application for approval of a device while evaluation and development continue in extended clinical investigations. The IDE was designed to encourage the cooperation of agency and industry in the "discovery and development" of new medical devices.³⁴

31. 21 U.S.C. § 360j(g) (1976).

32. 21 U.S.C. §§ 360c-360j (1976).

33. Ordinarily, the manufacturers of a new device would have to furnish the FDA with sufficient proof of its safety and efficacy *before* marketing the product. In determining the extent to which premarket approval would be required, the Amendments created three categories of devices that are subject to differing levels of regulation. 21 U.S.C. § 360c(a)(1). Class I includes devices, such as tongue blades and artificial eyes, believed to be adequately controlled by good manufacturing practices. Such devices are not subject to performance standards or premarket approval. 21 U.S.C. § 360c(a)(1)(A). Class II includes devices such as tampons and toothbrushes which demand more regulation than those in class I and are subjected to performance standards to ensure that the mass-produced products measure up to the specifications of the tested prototype. 21 U.S.C. § 360c(a)(1)(B). Class III devices such as heart valves and intrauterine devices must, in addition to meeting the requirements imposed on devices in class II, receive FDA approval before they are released for marketing. All new devices are placed in class III unless the manufacturer can convince the FDA that a lesser level of control is adequate. 21 U.S.C. § 360c(a)(1)(C). See Note, *Medical Devices Amendments to the Food, Drug, and Cosmetic Act Gives the FDA the Power to Regulate the Manufacture and Use of Medical Devices through Recommendations by Expert Panels*, 50 TEMP. L.Q. 1105 (1977) (discussing operation of classification procedure and examples of each class).

On the history of and perceived need for regulation of medical devices, see H.R. REP. NO. 853, 94th Cong., 1st Sess. 5-12 (1976); S. REP. NO. 33, 94th Cong., 1st Sess. 2-10 (1975), reprinted in 1976 U.S. CODE CONG. & AD. NEWS 1070.

34. The regulation of medical devices differs from the regulation of drugs because of the continuing nature of device development. A drug is typically a fixed compound that can be tested over a period of time; in contrast, a device is often modified during testing as more information and experi-

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A. *The IOL Study*

An important and instructive application of the IDE procedure is the FDA's study of the intraocular lens implant (IOL),³⁵ a surgically implanted prosthetic lens. The experience gained in this study demonstrates that a Federal agency can effectively monitor the delivery of new medical services, including the practices of individual doctors, without undue interference in the practices of physicians.³⁶

Although the intraocular lens implant was controversial, it had been widely used before the Medical Device Amendments were enacted.³⁷ Congress therefore specifically directed that the IOL be made reasonably available for use by all physicians who qualified under the new regulatory structure.³⁸ In carrying out that direction, the FDA imposed a requirement of premarket approval for the IOL,³⁹ but published guidelines inviting manufacturers of the lens to apply for an investigational device exemption for their product.⁴⁰

ence are gained. In enacting the Medical Device Amendments, Congress wished to encourage such development while granting the FDA adequate authority to regulate that process. See S. REP. NO. 33, *supra* note 33, at 2; 21 U.S.C. § 360(g)(1) ("It is the purpose of this subsection to encourage . . . the discovery and development of useful devices . . . [and] to maintain optimum freedom for scientific investigators in their pursuit of that purpose.")

35. The intraocular lens implant (IOL) is a small plastic lens that is implanted in the eye after cataract surgery in order to correct the refractive error created by the removal of a cataractous lens. The IOL has theoretical advantages over the conventional refraction with spectacles or contact lenses.

36. It should be emphasized that the discussion below makes no judgment on the scientific merit of the IOL study, or on the merits of the IOL itself. The focus here will be instead on the methodology of the study and its potential value as a model for monitoring physician practices. On the merits of the IOL study itself, see J. AMCHIN & R. LEFLAR, *THE HAZARDS OF INTRA-OCULAR LENSES 29-40* (1979) (criticizing study for lack of scientific validity and danger to patients).

37. The IOL had been in use since 1949 and had been used widely in this country in the early fifties. After a report critical of the device was issued in 1953 by the Panel for Cataract Surgery, an unofficial group of ophthalmologists, the IOL was rarely used in the United States. The IOL continued to be used in Europe, however, and after supposed improvements in the lenses and implantation techniques, it was reintroduced to the United States in 1967. The market was ripe and despite the previously disastrous experiences with the lenses, the devices again became popular here. By the time the Medical Device Amendments were being considered, the lenses were in wide use in the private practices of ophthalmologists, with a wide variety of complications. See 42 Fed. Reg. 58,874-876 (1977).

38. The conference committee on the Medical Device Amendments established specific provisions applying to any device declared to be a "new drug" after March 31, 1976, requiring that if such devices were relegated to investigational use, they should be made reasonably available to all physicians who met appropriate qualifications as prescribed by the FDA. That provision applied, by design, solely to the IOL and had much to do with the ultimate design of the IOL study. H. R. CONF. REP. NO. 1090, 94th Cong., 2d Sess. 63, *reprinted in* 1976 U.S. CODE CONG. & AD. NEWS 1070, 1115, 1116.

39. Under the MDA, any device that had been treated as a new drug by the FDA would be placed in Class III and therefore be subject to premarket approval. See *supra* note 33. The FDA had previously declared the IOL to be a new drug, 41 Fed. Reg. 14,570 (1976), and therefore premarket approval was required for its use, 41 Fed. Reg. 38,802 (1976). See 42 Fed. Reg. 58,876 (1977) (reviewing history of classification of IOL); *supra* note 38 (conference committee crafted statute for IOL knowing it had been declared new drug).

40. Investigational Exemptions for Intraocular Lenses, 21 C.F.R. § 813 (1981). See [1978] 2 MED. DEVICES REP. (CCH) ¶ 9000.1 (discussing in detail FDA reasoning in framing the exemptions

In the normal IDE process, the sponsor of a new device must specify in his application the identities of the physicians who will participate as investigators, and the rules of the study under which the physicians have agreed to work.⁴¹ That application, including the sponsor's proposal regarding the investigators, must be approved by the FDA before the manufacturer receives an exemption for his device.⁴²

In the case of the IOL study, because of Congress's mandate that the lens be made widely available, a somewhat different procedure was used. The FDA published, as agency regulations, minimum qualifications for investigators who were to be given access to use of the IOL.⁴³ The sponsors of the IOL then had the obligation to ensure that their investigators met those minimum training and experience requirements, and to monitor their investigators' compliance with the approved investigational protocol.⁴⁴ The duties of the investigators included the submission of records concerning their use of the IOL, with a description of results and complications,⁴⁵ for scrutiny by the sponsor and the agency.

In the IOL study, which is still in progress, the sponsors have divided the investigators into "core" and "adjunct" groups. The "core" group of investigators consists of a few surgeons who make relatively heavy use of the IOL and whose combined data is used to evaluate the fundamental efficacy of the device. The great majority of "investigators" have been placed in the "adjunct" group, whose data is used to evaluate the safety of the device as it is applied in wide practice.⁴⁶

Normal IDE procedures prohibit the marketing of devices while they

for IOL).

41. 21 C.F.R. § 812.20(b) (1981).

42. 21 C.F.R. § 812.30 (1981).

43. The regulations provided that

[t]he sponsor shall select as investigators only individuals who, because of their training or experience, qualify as suitable experts to investigate the safety and effectiveness of the lens. At a minimum, investigators shall have completed successfully a residency in ophthalmology or its documented equivalent and be licensed to practice medicine in the State or country in which the investigational study is to take place. Sponsors shall adopt appropriate additional criteria for investigators (bearing in mind the investigational plan, the report of prior investigations of the lens, and what is known about the lens).

21 C.F.R. § 813.43(a) (1981).

44. 21 C.F.R. §§ 813.43(b), 813.45(a), (c), 813.46(a), (b), (c), (e) (1981). One commentator has described the sponsor's role as follows: "the device manufacturer is to play the role of FDA vis-a-vis its investigators, assuring through on-site visits and a review of required record keeping, that the clinical investigator is doing precisely what he obligated himself (or herself) to do when he signed on at the onset." Bozeman, *The Clinical Investigation of Medical Devices—A Preliminary Guide for Manufacturers*, 34 FOOD DRUG COSM. L.J. 289, 298 (1979).

45. FDA regulations require that the investigator agree to ensure that patients treated under the study give their informed consent, 21 C.F.R. §§ 813.120, 130 (1981), and to maintain records on the results obtained, which are to be reported to the sponsor. 21 C.F.R. §§ 813.150, 153, 155 (1981).

46. J. AMCHIN & R. LEFLAR, *supra* note 36, at 10; Coburn Optical Industries, Investigational Protocol, Confidential Report 3 (May 1, 1980) (unpublished report on file with *Yale Law Journal*).

are under the special exemption.⁴⁷ The IOL study, however, allows the distribution of intraocular lenses to all physicians who are qualified as investigators.⁴⁸ Because of the popularity of the IOL among ophthalmologists, this controlled marketing approach has resulted in widespread use of the device, which is now employed in a significant fraction of all ophthalmic surgery.⁴⁹ At the same time, because of the requirement that the device be marketed only for use by qualified investigators on patients who are included in a study,⁵⁰ all use of IOLs takes place under investigational protocols in which the clinical records of the surgeons are regularly and voluntarily presented for scrutiny by outside parties—the sponsors and the FDA.

B. *Consequences of the IOL Study*

The regulatory implications of the IOL study are substantial. The IDE procedure, as applied in that study, has enabled a federal agency to monitor continuously the implementation of a proposed therapy over an extended period of time,⁵¹ without direct interference into either the practices of the physicians involved or the marketing process of the manufacturers. Physicians participating in the study have willingly opened their practices to detailed scrutiny by sponsors and, indirectly, by the FDA, in return for the privilege of using the IOL in their practices. As a result, the IDE process has allowed manufacturers to make the IOL available to a large fraction of the potential market, without requiring a time-consuming, conclusive premarket showing of the device's safety or efficacy. At the same time, the agency has avoided general release of an unproven therapy to physicians not capable of using it competently, be-

47. 21 C.F.R. § 812.7 (1981) prohibits marketing of a device under the IDE; § 812.7(b) prohibits making a profit on the sale of investigational devices, and § 812.7(d) prohibits any representation by the manufacturer to physicians regarding the device's safety or effectiveness.

48. Distribution of the IOL is limited to sponsors who have been granted an exemption by the FDA, and all patients who receive a lens must be treated as subjects in an investigational study. In addition, marketing methods are restricted to some extent; for example, 21 C.F.R. § 813.50 (1981) prohibits publication of any promotional literature claiming safety and efficacy for the IOL, but does allow "full exchange of scientific information." The exchange of information exception has been relied upon to justify placement of multicolor advertisements in the professional literature of ophthalmologists, albeit with enclosed statements such as "CAUTION: Investigational Device Limited By Federal Law To Investigational Use. See package insert for full prescribing information." Advertisements, *OPHTHALMOLOGY TIMES*, November 1981, at 63, 64, 66, 71, 73, 78, 81, 84, 93.

49. Approximately 100,000 IOL's are being implanted each year under the IDE. J. AMCHIN & R. LEFLAR, *supra* note 36, at Introduction.

50. 21 C.F.R. § 813.50(b) (1981).

51. After November 28, 1977, IOLs could be used only if they had been approved for general marketing or permitted under an investigational exemption. 42 Fed. Reg. 58,889 (1977), *corrected* 43 Fed. Reg. 6216 (1978), *reprinted in* [1978] 2 *MED. DEVICES REP.* (CCH) ¶ 9000.1.

cause the manufacturers, as sponsors, are obliged to monitor the qualifications and practices of the physicians acting as their investigators.⁵²

III. Evaluating Physician Use of New Medical Services

The intraocular lens study focused on the evaluation of a new medical device, in the traditional manner of FDA regulation. The experience gained under that study, however, suggests that an open-ended, broadly based investigation, if applied to other new medical services, could provide an effective tool for monitoring the practices of physicians using those new therapies as well as for evaluating the therapies themselves.

A. *The Mechanics of an Open-Ended Investigation*

Under such an investigational approach, as under current FDA procedures, the sponsor of a new medical service⁵³ would apply to the agency for premarket approval of his therapy. In most cases, the agency would not make an immediate decision, but rather would outline areas of concern regarding the treatment; the sponsor would then propose an investigational protocol to answer those concerns. A primary consideration in any such protocol would be the competence of the general physician population to employ the proposed therapy safely and effectively.⁵⁴

After agreement had been reached on a protocol, the sponsor would engage large numbers of physicians to act as "investigators." If approved by the agency, these physicians would be allowed to use the therapy in their practices.⁵⁵ They would be obliged to submit details of their clinical experience with the service to the sponsor, to be forwarded to the agency for scrutiny. For any given group of investigators, the combined data would speak to the safety and efficacy of the treatment as used by that

52. An investigational exemption may be withdrawn for any of a number of procedural shortcomings, or if the agency determines that the risks of the investigational treatment outweigh its benefits. See 21 C.F.R. § 813.35 (1981). Loss of the IDE would require that the sponsor either submit the device for approval or cease distribution. It is unlikely that a sponsor that had lost its IDE for a device could then gain approval of a premarketing application.

53. The term "medical service" as used here clearly would include drugs and medical devices, but could also reach other "services" such as surgical procedures. See *infra* p. 567 & note 76.

54. The proposed procedure is analogous to the regulation of Class III devices under the Medical Device Amendments. See *supra* note 33. Such devices must first be shown to be safe and effective, and once approved are subject to performance standards to ensure that the devices in production measure up to the prototypes tested. Here the service is also evaluated as a prototype for safety and efficacy, and the concurrent evaluation of the performance of physicians delivering the service takes the place of a performance standard.

55. In the consideration of an application for an IDE, the agency may prescribe any requirements reasonably necessary to protect the public health and safety. 21 U.S.C. § 360j(g)(2)(B)(iii) (1976). Such requirements could be initiated by the agency, or could be suggested by the sponsor as part of its proposed protocol.

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group; aberrant data submitted by individual investigators would speak to the competence of the individual investigator.

Such monitoring has not been done in the IOL study because of the focus on the device rather than on its use by physicians. If the FDA were to require, however, that the IOL study evaluate lens implantation as a “therapy” rather than as a “device”—that it consider delivery as well as design—the behavior of surgeons would become as important in the evaluation of the therapy as the quality of the manufactured product. The sponsors would then have an adequate incentive to monitor more closely the participating doctors’ practices.

Such an investigation would usually begin, as under present regulations, with small groups of selected investigators. If the results under such controlled circumstances were favorable, the sponsor could request an extension of the investigator pool.⁵⁶ The results obtained by the expanded pool would initially be measured against the baseline generated by the initial group of selected investigators; as the procedure evolved and more experience was gained with the expanded pool, the standard for comparison would be adjusted. The sponsor’s desire to expand the use of his proposed therapy, in conjunction with the agency’s desire to increase the number of controlled investigators, would result in use of the therapy by larger numbers of physicians even while the therapy was still under investigation. Because the investigational phase would be open-ended, and because patient demand for the therapy would grow with time and increasing use, most physicians with any potential need for the therapy would tend to join the study. Ultimately, therefore, the study could be expected to include almost all physicians who might ever desire to use the therapy.⁵⁷

Should the set of approved investigators come to approximate the whole set of physicians, general approval of the device might follow. Alternatively, if the initial results of the study demonstrate that the therapy is

56. It should be noted that the proposed method builds on the safeguards of present procedures. Before the open-ended investigation began, the proposed therapy would have to clear all of the preclinical and early clinical trials. The extended study would then serve to test the new therapy as it would be employed by the larger physician population.

Sponsors could petition for approval of a new therapy at any stage but would have the burden of convincing the agency that the therapy would be safe and effective in general use. The agency, working under the method proposed here, and viewing the therapy as including the practices of providing physicians, would require documentation that the therapy would be competently employed by the physicians likely to use the treatment. The sponsor would not risk disapproval of a premature application if the open-ended investigation allowed expanding use and marketing of the therapy without the need for approval.

57. The IOL study demonstrates that broad participation can be expected as the therapy becomes an increasingly common part of the physicians’ armamentarium. Physicians who were aware of their own incompetence might be deterred from participation in a study because it would entail surveillance of their practices. During the course of the study, such self-selection could only benefit the patients, and in many cases the investigation phase would probably last as long as the therapy was being used.

effective in the hands of some groups of physicians but ineffective or dangerous when used by others, the agency could extend the investigational exemption indefinitely in order to allow use of the treatment only by the group of investigators shown competent in its use.⁵⁸ In the latter case, granting a physician "investigator" status would amount to a form of licensing to use the new therapy based on the physician's qualifications and continuing performance.

The proposed method of evaluation differs in two ways from the existing IDE procedure. First, it focuses on the practices of physicians in the use of new therapies and procedures, as well as on the therapies themselves. Second, it envisions the possible acceptance of an investigational phase as the ultimate status quo, as a means of providing continuing evaluation of complex and changing medical services.⁵⁹

B. *The Dynamics of the Proposed Model*

The "sponsor" of the new therapy plays a crucial role in the regulatory scheme proposed here. As in current IDE studies, the sponsor would typically be a manufacturer who stands to profit from the wide use of a new drug or device.⁶⁰ The sponsor would therefore attempt, within the framework of the investigation, to secure the widest market possible for his product while gathering data that would support eventual premarket approval. During the study, the sponsor would wish to avoid the generation of data that call into question the safety or efficacy of the therapy. It would therefore be in the sponsor's interest to monitor the performance of investigating physicians to ensure that they performed the therapy in a competent manner.

58. Such restricted approval was foreshadowed in the Senate Report on the Medical Device Amendments of 1976:

[T]he skill of the user of the medical device has a direct and significant bearing on the safety and effectiveness of that device. . . . The Committee intends that if a device is safe only in the hands of eminently qualified specialists, that that device will be restricted to use by those specialists.

S. REP. NO. 33, *supra* note 33, at 13.

Under the Medical Device Amendments, the FDA is already given authority to restrict the use of medical devices. Such restrictions, however, may not limit the use of a device to persons with specific training or to use in certain facilities unless the Secretary finds that such restrictions are required for the safe and effective use of the device. In addition, such restrictions may not exclude a person solely because he is not board eligible for a particular medical specialty. See 21 U.S.C. § 360j(e)(1) (1976). The method proposed here would allow the Secretary to determine whether or not there is a need for such restrictions and what groups or hospitals should benefit from them—that is, who should have the use of the restricted therapy.

59. A sponsor unhappy working under the regulations of the continuing investigation would, of course, have the option of applying for premarket approval at any time; however, the incentives for the sponsor are such that such applications would not be common.

60. Physicians or hospitals might be sponsors in some contexts, such as in the evaluation of surgical procedures. See *infra* p. 567.

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Such a scheme would not tolerate manipulation of data by sponsors; the sponsor would be required to report any poor results received⁶¹ even though the chances for eventual approval of the therapy might be reduced. The sponsor would be permitted, however, either to exclude investigators who consistently reported poor results from further use of the therapy,⁶² or to help those investigators produce better results.⁶³ The exclusion of subsets of physicians would constitute an admission by the sponsor that the therapy was not suitable for general release, and could lead to a revision of the qualifications for investigator status if certain identifiable subgroups were consistently found to obtain substandard results. Such groupings might be based on a variety of objective variables such as the physician's subspecialty, surgical volume, time in practice, or access to specialized hospitals or other support facilities.⁶⁴

The sponsor's monitoring efforts might also lead to the identification of individual physician practices that enhance the results of the therapy. The sponsor could incorporate these practices into his protocol or attempt to remedy the deficient practices of other investigators by educating them in the use of preferred techniques. Such improved techniques incorporated into the protocol would become a condition of any eventual approval of the therapy.

The agency, while not directly supervising the use of the new therapy,

61. Current regulations provide for reporting of adverse effects during clinical testing. *Cf.* 21 C.F.R. § 812.46(b) (1981) (sponsor must immediately evaluate any unanticipated adverse effect and terminate any part of investigation that presents unreasonable risk); 21 C.F.R. § 813.46(c) (1981) (sponsor must suspend study if serious adverse reaction is lens-related); 21 C.F.R. § 813.153(b)(2) (1981) (requiring report of adverse effects of therapy to FDA).

62. In contrast, the regulations for the IOL study, *see supra* pp. 557-59, make no direct reference to removal of an investigator by the agency for incompetence. They provide only for disqualification because of procedural deficiencies, or for behavior adversely affecting the safety of subjects. *See* 21 C.F.R. § 813.119 (1981). Under current procedures, then, an investigator who was procedurally conscientious and technically safe, but not effective in treating patients, could not be disqualified by the agency. The sponsor, however, may terminate an investigator for any reason, but must notify the agency of its grounds. 21 C.F.R. § 813.119(j) (1981). If the reason involved consistently poor results, the exception might be saved, but eventual approval jeopardized by the sponsor's implied admission that the therapy was not safe or effective in general use. The disqualification of an investigator for producing poor results would make the study suspect, because it would allow the sponsor to select investigators likely to produce unusually good results. Under the proposed procedure, however, excluded investigators would never gain access to the therapy, because exclusion of investigators would demonstrate that the therapy could not be released for general use.

63. Congress recognized the possibility of such training when it enacted the Medical Device Amendments of 1976. *See* S. REP. NO. 33, 94th Cong., 1st Sess. 17, *reprinted in* 1976 U.S. CODE CONG. & AD. NEWS 1070, 1086 ("The committee recognizes that a device is only as good as the expert who uses it and therefore authorizes the Secretary to conduct programs for the education and training of individuals with respect to proper installation and use of devices.") Such educational efforts could, of course, distort the results of the investigation. To remedy this, the agency could require the sponsor to report any training it provides, and make such training a condition of agency approval.

64. The open-ended investigation is an attempt to evaluate the norms or biases of subgroups both as to the worth of their tools and the competence of their application of those tools. Once such norms for subgroups are evaluated, the traditional methods such as peer review, *see supra* note 16 and tort law, *see infra* note 81, are relatively efficient means for controlling individual behavior.

would be able to maintain indirect control because of its power to terminate an open-ended study for either substantive or procedural reasons.⁶⁵ By creating the proper incentives for the sponsors, the agency could leverage its resources to control indirectly and to evaluate the practices of a great number of physicians.

A unique virtue of the regulatory scheme proposed here is that it would impose burdens only upon those physicians who voluntarily choose to become "investigators"—those who, wishing to use a new form of treatment, agree to work under a research protocol that requires regular reporting and monitoring.⁶⁶

C. *Benefits of the Open-Ended Investigation*

The primary aims of the open-ended investigational approach are to monitor and evaluate the performance of individual physicians in their use of new therapies, and to ensure that physicians' practices are taken into account in the evaluation of the therapies themselves. Other benefits include greater flexibility in regulation, better evaluation of the long-term effects of new services, and better patient protection.

1. *Improved Regulation of New Medical Therapies*

The proposed investigational model would improve the evaluation of new therapies because it permits decisions based on the use of these therapies by a broad population of physicians, rather than on the results generated by small, selected sets of investigators.⁶⁷ Moreover, the relatively widespread use permitted during the investigation would generate better data on variations in patient response to the therapy.

During the course of a study, the proposed investigational mechanism would limit access to new therapies to those physicians who were most qualified to use them, as determined by the regulating agency.⁶⁸ If a study

65. The agency could terminate a study either because the data being produced indicated that the therapy was unduly dangerous as used in the investigation, or because the sponsor or his investigators did not comply with the requirements of the investigational protocol. *See supra* note 52.

66. *See supra* note 45 (describing responsibilities of investigators); *supra* note 44 (discussing responsibility of sponsors to monitor activities of investigators).

67. *See supra* p. 552 & note 8. The need for long-term evaluation of some therapies has already been recognized by the FDA in its regulations. In the case of drugs that are of proven value, but that require long-term use, the agency may mandate some long-term monitoring after approval of the drug. 37 Fed. Reg. 201 (1972). The method proposed here would reverse the presumption implicit in that approach and view long-term studies as the norm. Under the proposed approach approval would come after, rather than before, the extended evaluation.

68. The exclusion (or remedial training) of physicians who achieve substandard results in the study might raise concerns regarding the scientific validity of the data generated. But in studies that are continued indefinitely without an application for approval of the therapy being studied, the extent to which the data are generally valid would be of limited concern because the only physicians with access to the therapy would be those participating in the study itself. In other studies, to the extent

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is brought to a close, and permanent approval of a therapy is sought, the agency could limit its approval to use by those groups that had shown themselves most competent in the use of the therapy during the extended study.

2. *Increased Flexibility in Regulation*

Because it would reduce the risks involved in the marketing of a new medical therapy, an open-ended investigational approach would allow the FDA greater flexibility in its regulation of medical services. Such an approach may provide an alternative to the highly risk-averse approach presently favored by the agency.⁶⁹ In contrast to the current system under which the FDA's regulatory role largely ends with the approval of a drug or device,⁷⁰ the proposed method would permit the agency to release a controversial therapy for limited marketing under carefully specified conditions. With such flexibility, the FDA could allow limited clinical use of popular, but disputed, therapies⁷¹ while maintaining control over their application and obtaining information on their medical value.⁷²

3. *Evaluation of Long Term Effects*

An incidental benefit of the continuing study of physicians' use of a new therapy will be improved monitoring of long-term effects, a form of evaluation not usually performed under current law.⁷³ Continuing patient evaluations may uncover late-appearing, unexpected side effects of a new treatment that would otherwise be difficult to discover. The discovery of unexpected adverse effects would, of course, lead to tighter control of the

that exclusion is based on identifiable groups, and subsequent approval bars use of the therapy by those groups, the study data will represent accurately the competence of the population of all physicians who will have access to the therapy.

In the case of therapies presented for approval, the exclusion or education of individual investigators would raise concerns about scientific validity that would have to be taken into account. These concerns could be met, however, by statistically adjusting the data presented in support of a therapy to reflect the expected frequency of individual incompetence upon release of the therapy. In many cases, the set of investigators will approach the total population of physicians with an interest in the treatment.

69. See *supra* p. 552 & note 10 (citing criticisms of risk-averse nature of FDA).

70. See *supra* p. 551.

71. See *supra* note 10 (examples of popular and unproven therapies).

72. Under the proposed method, the therapy could be proposed by any interested party, including patient or physician groups. Because the investigational method ensures that patients are informed and that their treatment is carefully monitored, see *infra* p. 566, the agency could allow the use of any therapy not obviously dangerous. Such an approach by the FDA would blunt criticism of its risk-averse nature, see *supra* note 10, while monitoring results in the hope that some cult therapies might be helpful, see M. FRIEDMAN, *supra* note 16, at 157 (serendipitous discovery by aberrant physicians); at the very least, the freedom under this method would allow some patients the benefits of placebo therapy.

73. See *supra* note 67 (FDA has expressed only limited interest in evaluation of long-term effects of drugs).

therapy. Unforeseen positive results, on the other hand, would lead to expanded use of the treatment.⁷⁴

4. *Information for Consumers*

The open-ended investigational method will also provide better information for health care consumers. The investigational protocol would require that patient consent be given for the use of the new treatment, based on full disclosure of the circumstances of the study. Such disclosure would include, at a minimum, notice to the patient that the therapy was under investigation, that alternative forms of therapy were available, that the competence of the physician to perform the therapy was being monitored, and that the patient's medical records relating to the new therapy would be open for agency evaluation.⁷⁵ As the sponsor accumulated data demonstrating the relative skills of his investigators, and as the agency obtained information regarding the relative merits of therapies marketed by various sponsors, that information would also become part of the required disclosure.

IV. Further Areas of Application

The discussion above has focused on the application of the open-ended investigational model to the kinds of therapies that already receive FDA evaluation—new drugs and medical devices. The same regulatory approach could be applied to other aspects of medical practice, ranging from new surgical procedures to the everyday practices of physicians.

A. *Evaluation of New Surgical Procedures*

The regulatory approach described above could clearly be applied to the evaluation of newly developed surgical procedures, which are often dangerous, expensive, and controversial, and which are not currently evalu-

74. Many drugs initially approved by the FDA for one use have subsequently been found valuable in quite unexpected applications. For example, methotrexate, a treatment for uterine choriocarcinoma, was found to be of use in treating severe psoriasis. Xylocaine, which was initially approved as a local anaesthetic, was found in practice to be useful in the treatment of cardiac arrhythmias. See *New Drugs for Nonapproved Purposes: Hearing Before a Subcomm. of the House Comm. on Government Operation*, 92d Cong., 1st Sess. 19 (1971) (statement of James Grant, Deputy Commissioner, Food and Drug Administration).

75. Such requirements would be similar to those in force under the Medical Device Amendments, which require that informed consent be obtained from each patient before an investigational device is used, except where disclosure is medically infeasible and so documented. 21 U.S.C. § 360j(g)(3)(D) (1976). Under this requirement, the physician must disclose the experimental nature of the therapy, the availability of any alternative procedures, and all foreseeable risks and potential benefits of the therapy; he must also provide a statement describing the extent to which confidentiality of the medical records will be respected. 21 C.F.R. § 50.25 (1981). Under the method proposed here, it would be necessary to disclose in addition that the skills of the investigating physician were under study.

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ated by any external agency.⁷⁶ At present, the safety and efficiency of new procedures are evaluated solely by the individual physician, and the decision on whether to use such procedures remains solely in the discretion of each doctor. Even for surgical procedures widely considered to be of value their performance by particular physicians is not routinely evaluated.⁷⁷

Extending the model to cover physician procedures would require the designation of "sponsors"—that is, parties with enough at stake in the recognition of the safety and effectiveness of a procedure to ensure their enthusiastic advocacy of the operation.⁷⁸ One way to obtain such sponsors might be to make payments by the Federal government to physicians and hospitals, such as Medicare and Medicaid,⁷⁹ contingent on proof of the safety and efficacy of the care provided when new and experimental procedures are used. Hospitals and other providers of medical services could thus be induced to serve as sponsors of the surgical procedures taking place in their institutions, in order to obtain reimbursement for the costs of those operations. In a manner similar to the process described above for the regulation of drugs and devices, the evaluating agency would grant the provider the right to conduct new procedures on an "investigational" basis, only if the provider reports results regularly and limits use of the procedure to physicians who meet predetermined qualifications and who will work within the investigational protocol.

B. *Evaluation of General Medical Services*

The extension of the investigational approach to the evaluation of physician procedures would bring practically all physician use of new medical

76. Before the Food, Drug and Cosmetic Act of 1938 (FDCA), *see supra* note 2, physicians evaluated the drugs, devices, and procedures they used based on the experiences of the profession, without external interference. The FDCA requires external evaluation only of drugs and devices, but there is good reason for external evaluation of surgical procedures as well. The current evaluation of new surgical procedures is far from scientific. *See* M. MILLMAN, *supra* note 6, at 231-42 (demonstrating need for external evaluation of surgical procedures).

New procedures are reported in journals, discussed at meetings, and practiced on patients, but all on a purely voluntary and uncontrolled basis. *See* C. BOSK, FORGIVE AND REMEMBER 61 (1979) (noting physician uncertainty about the worth of surgical procedures); White, *Commentary*, 59 AM. J. PUB. HEALTH 66-68 (1969) ("I know of no evidence to suggest that more than 10 to 20 per cent of all that doctors and nurses do for or to patients are based on objective evidence that their pills, potions, or elixirs, their manipulations or maneuvers, operations, advice or counsel are likely to be more beneficial and useful than they are harmful or useless.")

77. *See* M. MILLMAN, *supra* note 6, at 231-32 (coronary by-pass surgery may be performed by any surgeon who wishes to try the procedure; no requirement exists for demonstration of skill or reporting of results).

78. *See supra* pp. 562-64 (discussing role of sponsor).

79. "Over \$22 billion a year, about one-third of the national health care dollars, are spent in hospitals. Blue Cross provides about half of hospital revenues, administering over \$11 billion in 1970. Public funds comprised over half of Blue Cross payments to hospitals—\$4.9 billion under Medicare, \$1.2 billion under Medicaid, and \$545 million under other federally financed programs." S. LAW, BLUE CROSS: WHAT WENT WRONG? 1-2 (2d ed. 1976).

services under federal supervision.⁸⁰ Although limitations on agency resources would require most services to be approved or disapproved for general use after some period of study, the most complex or controversial services might remain under surveillance indefinitely. Thus, over time, with continuing innovations in medical services, such regulations would reach a considerable portion of the general practice of medicine.

It is not anticipated, however, that the proposed investigational model would be applied to physicians' performance of medical services that are already generally accepted as safe and effective. First, no central agency is likely to have the resources required for such widespread evaluation. In addition, once the merits of a procedure are generally known, normal medical training will provide sufficient familiarity with its use, and central evaluation becomes less essential.⁸¹ The existing means of evaluation and regulation, though imperfect, are likely to be at their most effective in dealing with the use of standard and familiar therapies.⁸²

C. *Application to Innovative Practices of Individual Physicians*

Presumably, the regulatory method proposed here would not be applied to the individual physician who uses an innovative therapy tailored to the needs of a specific patient.⁸³ Physicians have traditionally been free to use any therapy that has not been specifically prohibited in the treatment of an individual patient, if the patient gave informed consent. Such freedom

80. As with medical devices, there would be services that would not come under the proposed model. Some form of classification would identify those practices that were significantly different from old procedures and that might be sufficiently harmful or ineffective to invoke the model. Thus, under a grading similar to that in the Medical Devices Amendments, *supra* note 33, a new procedure for patient bathing might be a Class I procedure, subject only to due care in performance, while a new manner of pedicure might be a Class II procedure requiring proven skill in performance. Most surgical procedures would be Class III procedures subject to "premarket clearance," with exemption under the model.

81. Once a standard of care has been defined for a form of treatment, tort remedies apply to deviations from that customary and usual standard. If the patient benefits from the deviation, there would be no damages. If the patient is harmed, the physician may be liable. See W. PROSSER, TORTS 165 (4th ed. 1971) (defining malpractice as neglecting to use customary degree of skill for that profession). Specialists are now expected to adhere to national standards of care for their specialty. See A. HOLDER, *supra* note 23, at 61. A physician may deviate from the norm if he is supported by a "respectable minority" of physicians, but whether that minority is professional and "respectable" or unreasonable and negligent may be a close question. See *Hood v. Phillips*, 537 S.W.2d 291 (Tex. Civ. App. 1976), *aff'd*, 554 S.W.2d 160 (Tex. 1977) (defendant surgeon was one of only six surgeons in the world who regularly performed carotid body surgery for emphysema—but he performed approximately 2,000 such operations).

The method proposed by this Note would furnish external evidence on the reasonableness of the standard therapy and identify groups likely to perform that therapy competently. Individual deviations could be handled by tort remedies.

82. See *supra* pp. 554-55 (discussing existing mechanisms for regulation of medical practices).

83. A similar policy is reflected in the Medical Device Amendments, which specifically exempt custom devices from premarket approval or performance standard requirements. See 21 U.S.C. § 360i(b) (1976); H. R. CONF. REP. NO. 1090, *supra* note 38, at 61.

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has been deemed necessary to enable the physician to mold a therapy to fit each patient's unique and changing needs. At this level, the individual physician should have primary loyalty to his patient, and not to a scientific investigation.⁸⁴

A successful innovation, of course, might lead to repetition; if a therapy attains widespread use in this fashion, the exemption for tailor-made procedures would no longer apply. If demand for the therapy were sufficient, a sponsor would presumably appear, and the therapy would then be evaluated in the usual fashion.

84. See *supra* note 76 (noting conflict between caring for patient and scientific investigation); Jonas, *Philosophical Reflections on Experimenting with Human Subjects*, 98 *DAEDALUS* 219, 238 (1969) (fundamental right of sick person to have physician obligated to him rather than to investigation).