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**The Dividing Line Between the Role of the FDA and the Practice of  
Medicine: A Historical Review and Current Analysis**

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**Introduction:**

Over the years, the Food and Drug Administration (FDA) has consistently asserted that it does not regulate the practice of medicine (the Practice of Medicine Exception) . This prohibition has never been specifically set forth in the statutory scheme which guides the FDA's action. However, the Practice of Medicine Exception has been inferred from the Congressional intent expressed in the legislative history. Although the FDA purports to maintain this exception, the reality is that agency action certainly affects medical practice.

The boundaries of the FDA's providence and the Practice of Medicine Exception is not always clear. This paper traces the historical dividing line between the role of the FDA in protecting the public from unsafe drugs and medical devices and the discretion of licensed health care practitioners to engage in the practice of medicine. This paper then highlights the potentially unreconcilable differences between the oft lengthy FDA approval process for drugs and devices and the more immediate nature of the doctor/patient relationship. It then sets forth the FDA's inconsistent treatment of the unapproved uses of drugs and devices, which is justified by the non-interference with medical practice. Finally, this paper sets forth recommendations for reconciling the FDA's position on the Practice of Medicine Exception.

**Overview of FDCA**

The basic purpose of the Federal, Food, Drug and Cosmetic Act of 1938 (FDCA or the Act) is for the protection of the public

health and safety.<sup>1</sup> Congress clearly intended that the FDA's jurisdiction be as broad as its literal language indicates<sup>2</sup> and the FDCA should be liberally construed. The FDA's mandate should be analyzed in terms of consumer protection, not dialectics.<sup>3</sup> The Act vests broad substantive rulemaking authority in the Secretary of Health and Human Services.<sup>4</sup> Regulations promulgated pursuant to the FDCA will be sustained so long as they are reasonably related to the purposes of the enabling legislation.<sup>5</sup> The Acts' potentially broad coverage should be effectuated, even if it would result in far-reaching inroads upon customary control by local authorities of traditionally local activities.<sup>6</sup>

Although Congress has given broad regulatory powers to the

1 *U.S. v. Diapulse Corp. of America*, 457 F.2d 25, 27 (2nd Cir. 1972); *Pharmaceutical Mfrs. Ass'n v. Food and Drug Administration*, 484 F.Supp. 1179, 1182 (1980) . Legislation that involves issues of safety, must be liberally construed to effectuate congressional intent. *Whirlpool Corp. v. Marshall* 445 U.S. 1, 13 (1980)

2 *U.S. v. Bacto-Unidisk*, 394 U.S. 784, 798 (1969), *reh'g den.* 395 U.S. 954.

*U.S. v. Urbuteit*, 335 U.S. 355, 358 (1948) . Regard for these purposes should infuse construction of the legislation if it is to be treated as a working instrument of government and not merely as a collection of English words. *U.S. v. Dotterweich*, 320 U.S. 277, 280 (1943) *reh'g den.* 320 U.S. 815.

21 U.S.C.A. § 371(a) (1995)

*Mourning v. Family Publications Services, Inc.*, 411 U.S. 356, 369 (1960) .

When agency rulemaking serves the purposes of the statute, courts should refuse to adopt a narrow construction of the enabling legislation which would undercut the agency's authority to promulgate such rules. *Pharmaceutical Mifra. Ass'n v. Food and Drug Administration*, 484 F.Supp 1179, 1183 (1980)

6 *U.S. v. Sullivan*, 332 U.S. 689, 693 (1948)

agency, the FDA cannot alter its jurisdiction by agency action<sup>7</sup> and cannot promulgate regulations which extend or amend the Act itself.<sup>8</sup> Also, the expansive discretion given to the FDA pursuant to the Act cannot be exercised in an unbridled fashion.<sup>9</sup>

#### **FDCA History Relating to the Practice of Medicine**

The Federal Food and Drug Act of 1906 was the first national statute to provide for the regulation of the American food and drug supply. Congress gave FDA the power to remove misbranded or adulterated foods and drugs from the market.<sup>10</sup> This initial act

did not deal with the issue of regulating the practice of

11

medicine.

Between 1906 and **1938,12** there was no comprehensive attempt to

*US. v. Pro-AG, Inc.*, 796 F.Supp 1219 (1991), *aff'd* 968 F.2d 681 (1992)

8 *U.S. v. Antikamnia Chemical Co.*, 231 U.S. 654 (1914).

The FDA's procedures must satisfy the rudiments of fair play. *Rutherford v. U.S.*, 429 F.Supp. 506 (1977) (citing *Weinberger v. Hymson*, 412 U.S. 609, 624 (1973)).

<sup>10</sup> See generally, Hutt, *Regulation of the Practice of Medicine under the Pure Food and Drug Laws*, 33 Q. Bull. Ass'n of Food & Drug Off., No.1, at 7 (1969).

*Id.*

<sup>12</sup> The initial bill was introduced in 1933. After three years of extensive debate and several versions of the bill, President Franklin D. Roosevelt, urged Congress to pass legislation during its 73rd Session. President's Message, House Report, 74th Congress, 2d Sess., vol. 3, pg 1-2 (1936). In spite of the President's request, the legislation was not enacted for two more years.

During the rancor of the debates, Senator Copeland, the sponsor of the FDCA, stated: I thought I had all the troubles one could have in this life: but in all my experience I have never had so many worries and so much trouble as I have had in connection with this bill. 78 Cong. Rec. 2728 (Feb. 19, 1934).

reform the regulation of food and drugs within the United States. The 1938 Act<sup>3</sup> significantly expanded the FDA's reach by adding the responsibility of cosmetic and medical device regulation. The FDCA also provided the agency with the mandate of assuring the safety of a product prior to it being marketed.<sup>4</sup>

Although there was no prohibition within the language of the statute, the legislative history of the FDCA contains many statements that the regulation of the practice of medicine was outside the purview of the statutory scheme.<sup>5</sup> During the hearings the medical profession expressed concern that the FDCA would interfere with the prerogatives of the doctor.<sup>6</sup> In response, Senator Copeland stated that this bill makes certain that the medical practitioner shall not be interfered with in his practice. <sup>v</sup>

Amendments to the statutory language prior to passing the FDCA also indicate an intent on the part of Congress to not interfere with medical practice. Initially section 321(b) of the 1938 Act, defining the term drug, contained language stating that it was

<sup>v</sup> Congress enacted the Federal Food, Drug and Cosmetic Act of 1938 and repealed its forerunner, the Federal Food and Drugs Act of June 30 1906. 21 U.S.C.A. § 301 et seq.

*Id.*

<sup>15</sup> Senator Copeland, a strong proponent of the legislation, was a physician and was mindful of the potential impact such legislation could have on the medical profession. See, Hutt, *Regulation of the Practice of Medicine under the Pure Food and Drug Laws*, 33 Q. Bull. Ass'n of Food & Drug Off. No.1, at 15 (1969)

<sup>16</sup> 78 Cong. Rec. at 2728.

<sup>17</sup> *Id.*

not intended for the regulation of the legalized practice of the healing art.<sup>18</sup> Committee reports indicate that the bill was not intended as a medical practices act and [would] not interfere with the practice of the healing art by chiropractors and others in the States where they are licensed by law to engage in such practice.<sup>19</sup> Although, this proviso was omitted in the final version of the bill, the Committee on Interstate and Foreign Commerce which submitted the report to Congress explicitly explained the deletion in order to avoid possible misunderstanding as to the intention of the committee.<sup>20</sup> The committee stated that the phrase was ultimately omitted because the words were unnecessary as Et] he bill does not undertake to regulate the practice of the healing art.<sup>21</sup>

However, despite several comments that the FDCA would not regulate the practice of medicine, Congress enacted labeling requirements that applied to prescription drugs over objections from representatives of the medical profession.<sup>22</sup>

H. Rep No. 2755, 74th Cong., 2nd Sess. 5 (1936).

*Chaney v. Heckler*, 718 F.2d 1174, 1180 fn.13 (1983), *rev'd* 470 U.S. 821 (1985) (quoting S.Rep. No. 361, 74th Cong., 1st Sess. 3 (1935); S.Rep. No 646, 74th Cong., 1st Sess. 1 (1935)).

<sup>20</sup> *Id.*

<sup>21</sup> *Id.*

<sup>22</sup> *Pharmaceutical Mfrs Ass'n*, 484 F.Supp. at 1184 (citing Hearings on S. 5 Before a Subcom. of the Senate Commerce Coin., 74th Cong., 1st Sess. 148 (1935); Hearings on H.R. 6906 Before a Subcom. of the House Coin. on Interstate and Foreign commerce, 74th cong., 1st Sess. at 316, 322 (1935))

In 1951, the Durham Humphrey Amendment<sup>23</sup> was the first major amendment to the 1938 Act. The amendments exempted prescription drugs from previous FDA labeling provisions and clarified the distinction between over-the-counter drugs and prescription medications.<sup>24</sup> The adequate directions for use and warnings against misuse requirements were deemed unnecessary if the prescription label contained specified information for use and any necessary cautionary statements.<sup>26</sup>

There are only sparse references in the House or Senate hearings and reports that refer to the prescription drug exemption as it relates to the practice of medicine. One comment, however, indicated that the warning requirement of section 352(f) (2) was unnecessary when a prescription medication was prescribed because the practitioner had the responsibility to see that the drug would be properly used.<sup>27</sup>

<sup>23</sup> 65 Stat. 648 (1951), codified in 21 U.S.C.A § 353(b)

<sup>24</sup> 21 U.S.C.A. § 353 *et seq.* The 1938 Acts was ambiguous, thus permitting a manufacturer to determine whether a drug would be sold as by prescription or over-the-counter. This led to situations where the same drug could be classified as an over-the-counter preparation by one manufacturer and classified as a prescription drug by another. The Durham-Humphrey Amendment sought to remedy this dilemma by vesting the FDA as the sole determinant of prescription drug status. Temin, *The Origin of Compulsory Drug Prescriptions*, **22** J. of Law & Econ. 91 (1979).

<sup>25</sup> 21 U.S.C.A. § 352(f).

<sup>26</sup> 21 U.S.C.A. § 353 (b) (2) For a general discussion see,

*Pharmaceutical Mfrs. Ass'n*, 484 F.Supp at 1185.

<sup>27</sup> *Pharmaceutical Mfrs. Ass'n*, 484 F.Supp at 1185 (citing remarks from the Federal Security Administrator Oscar Ewing, Hearings on H.R. 3298 Before the House Committee on Interstate and Foreign Commerce, 82nd Cong., 1st Sess. 20-21 (1951)).



Thus, the Durham-Humphrey Amendment did not appear to increase any regulatory control over the practice of medicine. In fact, the exemption of prescription drugs from the requirements of section 352(f) gave physicians primary responsibility to inform the patient as to the adequate directions for use any necessary warnings regarding misuse.<sup>28</sup> However the court in *Pharmaceutical Mfrs. Ass'n*, stated that the amendment did not strip the Commissioner of the regulatory authority he had possessed for thirteen years over prescription drug labeling.<sup>29</sup>

The Drug Amendments Act of 1962,<sup>30</sup> significantly expanded the FDA's responsibilities regarding new drug regulations and for the first time gave FDA power to scrutinize and evaluate drugs for effectiveness as well as safety.<sup>31</sup> However, the Drug Amendments contain some specific exclusions to the FDA's jurisdiction that continue provide support for the Practice of Medicine Exception. For example, section 374 gives the FDA extensive powers to inspect facilities, vehicles, materials, containers, and records to determine whether prescription drugs or restricted devices have been adulterated or misbranded.<sup>32</sup> However, Congress specifically

28 *Id.* at 1185.

29 *Id.* at 1186 (the court concluded that when the Commissioner determines that the possible side effects of a drug when used as customarily prescribed are sufficiently serious as to be material to the patient's decision on use of the drug, he or she may require disclosure of those side effects on the labeling)

30 Pub.L. No. 87-781, 76 Stat. 781 (1962)

*Weinberger v. Hynson*, 412 U.S. 609, 630 (1973)

32 21 U.S.C.A. § 374(a) (1).

exempted licensed practitioners who administer, prepare or manufacture drugs or devices solely for use in the course of their professional practice.<sup>33</sup> Additionally, the amendments require that producers of drugs or devices must register with the Secretary of Health and Human Services.<sup>34</sup> Again, licensed practitioners who prescribe or administer drugs or devices and who manufacture, prepare, propagate, compound, or process drugs or devices solely for use in the course of their professional practice are also exempted from this requirement.<sup>35</sup>

In 1972, the FDA issued a Notice of Proposed Rule Making regarding, in part, the issue of prescriptions written for uses unapproved by the FDA.<sup>36</sup> The proposed rule indicated that a prescription new drug is not permitted to be shipped in interstate commerce if the intended use is not contained in the approved uses set forth in the labeling.<sup>37</sup> An unapproved use even includes actions such as a different dosage or a different patient population or a different regimen.<sup>38</sup> However, the rule states that once a new drug has been introduced into interstate commerce,

21	U.S.C.A.	§	374	(a)	(2)	(B)
21	U.S.C.A.	§	360(a)	(1)	&	(b)
21	U.S.C.A.	§	360(g)	(2).		

**36** *Legal Status of Approved Labeling for Prescription Drugs; Prescribing for Uses Unapproved by the Food and Drug Administration*, 37 Fed.Reg. 16503 (1972)

*Id.* at 16504. The labeling of a prescription new drug contains all information regarding the conditions for which there is substantial evidence that the drug is safe and effective. *Id.*

38 *Id.*

a physician may prescribe the drug for an unapproved use if the prescription is written as part of the practice of medicine.

Pursuant to the proposed rule, if the FDA determines that an unapproved use of a new drug may pose a danger to the patients receiving the medication, the agency may confine distribution of the drug to specified channels or restrict the physicians who are able to prescribe the drug. The FDA would have the power under the proposed rule to permit only physicians with specified qualifications to prescribe, dispense or administer certain medications.<sup>41</sup> This action would impact some physician groups, in the context of their doctor/patient relations, in limiting their ability to prescribe the medication of their choosing. Only specialists or particularly qualified physicians would be permitted to treat patients with certain designated medications. This action, more than any other would interject the FDA in to the doctor's office.

The FDA has stated that this rule expresses its established policy.<sup>42</sup> However, this author could not locate any instance of enforcement action involving the FDA's limiting the prescription of a specified medication to a particular medical specialty. This is perhaps in recognition of the impact this rule could have on the doctor-patient relationship.

*Id.*

*Id.*

41 *Id.* at 16504-05.

42 See *generally*, *Food and Drug Law* at 621, note 1.

Interestingly, for almost twenty years, the FDA did not take final action on this proposed regulation. In August of 1991 the FDA issued a Notice of Intent to withdraw certain proposed rules for which a final rule or notice of withdrawal had not been issued, including the rule regarding the prescription of drugs for uses unapproved by the FDA.<sup>43</sup> Later that same year, the FDA decided not to withdraw this proposed rule but also not proceed to a final rule. The FDA stated that the agency has established an Unlabeled Use Task Force to examine the promotion and use of prescription drugs for indications not included in their approved labeling. ... Therefore, the agency will defer consideration on the withdrawal of this proposed rule until after the task force has completed its review.<sup>45</sup>

Although this paper primarily focuses on the practice of medicine as it relates to prescription medications, it also discusses medical device issues where they are pertinent. Therefore a brief background of the FDCA treatment of medical devices is included. The 1976 Medical Device Amendments Act fundamentally restructured the FDA's approach to the regulation of medical devices. The amendments require premarket notification

56 Fed.Reg. 42668-01.

56 Fed.Reg. 67440 (1991)

*Id.* at 67442. This author could not locate any further published action on this issue.

**46** Unlike the 1962 changes in the law governing drugs, the Medical Device Amendments were the culmination of fifteen years of study and debate, not only within congress and the agency, but also among representatives of clinical medicine, biomedical engineering,

for devices substantially equivalent to those already on the market and premarket approval for new medical devices.<sup>47</sup> The Safe Medical Devices Act of 1990, further strengthened and defined the FDA's role in the medical device arena.<sup>48</sup>

**Scope of the Practice of Medicine Exception:**

As noted in the previous section, there are several references throughout the enactment and amendments to the FDCA that indicate the role of the agency is not to regulate the practice of medicine or otherwise interfere with the practice of the healing arts.<sup>49</sup> However, the scope of this general terminology and thus the Practice of Medicine Exception, has never been clearly defined.

As the right to privacy developed in the courts, it was initially argued that the doctor-patient relationship should be protected as a privacy interest. The Supreme Court in declaring the right of privacy<sup>50</sup> to be a fundamental right, carved out two types of privacy interests. One type of privacy right is the interest in non-disclosure of personal matters;<sup>51</sup> the other type of

device manufacturers, and consumer groups. Hutt & Merrill, *Food and Drug Law* 13 (2nd ed. 1991).

*Food and Drug Administration Performance and Accountability Act of 2996*, Senate Report 104-284, 104th Cong., 2nd Sess., preamble at 5 (1996) (hereinafter referred to as the Kassebaum Bill)

48 Codified at 21 U.S.C.A. § 531 et seq.

See *supra*, fn. 15-21 and accompanying text.

50 The right of privacy is founded in the Fourteenth Amendment's concept of personal liberty and restriction upon state action. *Roe v. Wade*, 410 U.S. 113, 152, 155 (1973)

51 *Whalen v. Roe*, 429 U.S. 589, 599 (1977)

privacy interest involves the interest in independence in making certain kinds of important decisions.<sup>52</sup> In *Paul v. Davis*,<sup>53</sup> the Supreme Court characterized the latter privacy interest as matters relating to marriage, procreation, contraception, family relationships, and child rearing and education.<sup>54</sup>

In California, defendants convicted of prescribing and selling laetrile, an unapproved drug for the treatment in cancer, argued that there was a constitutionally protected privacy interest in a right to in medical treatment.<sup>55</sup> The defendants argued that the state statute prohibiting the sale, delivery, prescription or administration of any drug or device that has not been approved by the designated federal agency, was unconstitutional based on a compelling state interest standard.<sup>56</sup> The *Privitera* court declined to follow the defendants' position and held that the types of important decisions deemed fundamental by the Supreme Court did not include medical treatment.<sup>57</sup>

52 *Id.* at 599-600.

242 U.S. 693 (1976)

*Id.* at 713.

*People v. Privitera*, 23 Cal.3d 697, 701 (1979) *cert. den.* 44 U.S. 949 (defendants were convicted of the felony of conspiracy to sell or prescribe an unapproved drug, in violation of state law)

56 *Id.* at 701.

*Id.* at 702; *see also*, *People v. Younghanz*, 156 Cal.App.3:

811, 816 (1984) (the right to seek a cure for one's illness is not a fundamental right)

In *Rutherford v. U.S.*,<sup>58</sup> the trial court held that terminally ill cancer patients had a fundamental right to receive medical treatment whether or not it was approved by the FDA. The court stated that [bly denying the right to use a nontoxic substance in connection with one's own personal health-care, FDA has offended the constitutional right of privacy.<sup>59</sup> The trial court granted petitioners' request to enjoin the federal government from interfering with the shipment and sale of Laetrile.<sup>60</sup> The Supreme Court reversed the decision on other grounds and remanded the case for consideration of the constitutional issues.<sup>61</sup> On remand, the court of appeals held that patients do not have a fundamental interest in their selection of a particular treatment and that the availability of specific medication is within the area of governmental interest in protecting public health.<sup>62</sup> The Supreme Court denied certiorari, so it seems clear that patients do not have a constitutional right to receive the medical treatment of their choosing and doctors do not have the unfettered ability to provide medical care that is within the jurisdiction of the FDA and

58 ~ F.Supp (1975), *aff'd and remanded*, 542 F.2d 1137  
(1976), *on remanded to*, 424 F.Supp 105 (1977), *on remanded to*, 438 F.Supp 1287, *remanded by*, 582 F.2d 1234 (1978), *reversed*, 442 U.S. 544 (1979) , *on remand to*, 616 F.2d 455 (1980) , *cert. denied*, 449 U.S. 937.  
*Rutherford*, 438 F.Supp. 1287, 1310 (1977)  
*Rutherford*, 339 F.Supp. 1208.  
61 442 U.S. 544.  
62 *Rutherford*, 616 F.2d 455, 457 (The premarketing requirement of the [FDA] ... is an exercise of Congressional authority to limit the patient's choice of medication)

that the FDA has not approved.

Because the right to receive medical treatment is not a fundamental right, the appropriate standard of review of any legislation would be the rational basis test rather than the compelling state interest test. It should be noted that any regulation promulgated by the FDA is subject to even less scrutiny. A regulation, properly enacted is only subject to attack on constitutional grounds.<sup>63</sup> The challenged regulation will be deemed valid unless it is plainly inconsistent with the provisions of the Act under which it was promulgated.

In 1985, the Commissioner of the FDA took the position that the legal basis of the Practice of Medicine Exception was based on the premise that individual states had the responsibility to license and regulate medical practice<sup>65</sup> and therefore, the FDA could not regulate in this arena. The *Chaney* court dismissed this premise and stated: There is scant legislative history on the subject, but the few sentences that can be found are more fairly

<sup>63</sup> *Byrd v. U.S.*, 154 F.2d 62, 63 (5th Cir. 1946).

*Toilet Goods Ass'n v. Gardner*, 278 F.Supp. 786, 788 (1968), *aff'd in part, rev, in part*, 419 F.2d 21 (1969)

<sup>65</sup> If the FDA were correct that physicians' use of drugs is not within FDA's jurisdiction simply because physicians are licensed by the states, then it would necessarily follow that FDA could have no authority to regulate drugs that state-licensed physicians administer to prison inmates in experimental clinical investigations and no authority to regulate drugs that state-licensed veterinarians use to put animals to their death. But FDA has in fact regulated drugs used in prison clinical investigations

and in veterinary practices .... In both situations FDA

has rejected arguments that it does not have authority to regulate unapproved uses of approved drugs. *Chaney*, 718 F.2d at 1180 (reversed on other grounds)



read as reflecting that the states do regulate the practice of medicine and that a physician cannot be eligible for the practice-of medicine exemption if he has not been so licensed. The practice-of-medicine exemption itself, however, cannot be attributed to the states' licensing of their physicians. The better explanation for the practice-of-medicine exemption is that Congress did not want to interfere with physicians' treatment of their patients.~ The basis of the Practice of Medicine Exception is not appropriately derived from deference to state action but rather a manifestation of Congressional intent that the FDA should not interject itself into the realm of the doctor/patient relationship.

The FDA has made it clear that although it will not oversee the practice of medicine, it will properly control what drugs are available to physicians.<sup>67</sup> The Court in *U.S. v. Rutherford*,<sup>68</sup> held that the distribution of Laetrile was prohibited for any use until it was approved by the FDA. Although the Court did not explicitly address the practice of medicine exception, its holding necessarily places limitations on a physician's unfettered practice of medicine by interpreting the Act as intending a pre-market approval process applicable to all drugs, whether sold over-the-counter or by

*Id.* at 1180.

67 ~ Fed.Reg. at 16504.

68 442 U.S. 544 (1979)

prescription.<sup>69</sup> In the FDCA 70 Congress has required the FDA Commissioner to assure that all 'new drugs' are safe and effective for the use under the conditions prescribed, recommended, or suggested in the labeling.<sup>71</sup> In order for the FDA to allow a new drug into interstate commerce, there must be substantial evidence, upon which experts in the field could conclude, that the drug is safe and effective.<sup>72</sup>

Defining the boundaries of the Practice of Medicine Exception is not a facile task. Therefore, the extent and manner to which the FDA can impinge on medical practice is not certain. What is

**69** *U.S. v. Algon Chemical Inc.*, 879 F.2d 1154, 1161 (3rd Cir. 1989); *see also U.S. v. Evers*, 643 F.2d. 1043, 1048 (5th Cir. 1981) (Of course, while the Act was not intended to regulate the practice of medicine, it was obviously intended to control the availability of drugs for prescribing by physicians)

**70** 21 U.S.C.A. § 331 *et. seq.*

See generally, 21 U.S.C.A. § 355. [W]hen the FDA grants approval [of a drug] it is not merely of a drug substance, but additionally represents approval of a safe process of manufacture, This process involves consideration of such issues as where the raw materials come from, which inactive ingredients are going to be added and in what measure, how the drug will be packaged and how shelf-life will be maintained. *Algon Chemical Inc.*, 879 F.2d at 1161.

**72** The new drug provision of the Act requires the filing of a new drug application (NDA) which must include full reports of investigations showing the safety and effectiveness of the drug; a list of articles used as components of the drug; a description of the composition of the drug; a description of the methods, facilities and controls used to produce the drug; samples of the drug; and the proposed labeling. 21 U.S.C.A § 355(b) (1). The applicant must then wait up to one hundred and eighty days or longer, to have the application approved or denied. 21 U.S.C.A §355 (c). It is unlawful to introduce any new drug into interstate commerce prior to the FDA's approval of the NDA which establishes the drug as safe and effective for its intended use. 21 U.S.C.A §355(a)

clear is that the lengthy FDA approval process for most drugs and devices, does not dovetail with the patients immediate needs in a clinical setting. Currently, there are two pending bills that seek, in part, to essentially clarify the FDA's Practice of Medicine Exception.

**Proposed Access to Medical Treatment Act**

In *Retkwa v. Orentreich*, the defendants argued that because Congress did not intend that the FDCA interfere with the practice of medicine, the FDA could not prohibit the use by a physician of any product whether or not it was approved or adulterated.<sup>73</sup> The court refused such statutory construction and stated there was no authority for the proposition that the Act was inapplicable to physicians using a drug or device which was never approved for any purpose.<sup>74</sup> However, the proposed Access to Medical Treatment Act (ANTA) would permit just such a scenario.

The ANTA highlights the potentially irreconcilable differences between protection of the public from unsafe or ineffective medical treatments and the freedom of a doctor to determine the course of treatment for her patients. Although the ANTA does not specifically address the FDA's Practice of Medicine Exception, in essence, the bill is an attempt to codify this exception and significantly expand its breath.

*Retkwa v. Orentreich*, 579 N.Y.S.2d 577, 580 (1991)

*Id.*; see also, *Algon Chem, Inc.*, 879 F.2d 1154 (ct concluded that the practice of medicine exemption did not protect physician's preparation of an unapproved illegal drug)

On May 19, 1994, Senator Daschle<sup>75</sup> introduced the original version of the ANTA.<sup>76</sup> The purpose of the legislation is to allow patients an increased freedom of choice of available medical treatments.<sup>17</sup> Senator Daschle stated: The ANTA represents a significant departure from current medical practice. It is grounded in the belief that our current health care delivery system actually discourages rather than encourages the development of alternative therapies that could effectively treat illnesses that often do not respond well, if at all, to conventional medicine. And it seeks to open up the system to such treatments under controlled conditions. **78**

The intent of the proposed legislation is two-fold. Firstly, it seeks to permit increased access to alternative treatments not currently approved by the FDA for use. Secondly, in allowing increased opportunities for use of alternative treatments, additional effective treatments and cures may be found.<sup>79</sup> The effect of the ANTA is that treatments considered alternative, or

Senators Harkin, Pell, Grassley, Haffield, and Deconcini  
also sponsored the initial bill. 140 Cong. Rec. S6123-02, S6130  
(May 19, 1994)

**76** *Id.*

The term medical treatment is defined in the act as any food, drug or device, or procedure that is used and intended as a cure, mitigation, treatment, or prevention of disease. ANTA § 2 (10) . The terms food, drug and device have the same meaning given such terms in the FDCA. *Id.* § (3), (4) & (5)

<sup>78</sup> *Id.* The Medical Treatment Act seeks to guarantee all Americans the freedom to choose not only their doctor but also the form of treatment they want to pursue. 140 Cong. Rec. at E1657.

*Id.*

out of the mainstream of conventional medical acceptance, would be more available to the general public.

The bill is an attempt to circumvent the FDA approval process for treatments desired by a patient and that a licensed health care practitioner is willing to administer. The concern with the current FDA process is that its stringent requirements are time consuming and extremely costly, preventing many potentially effective treatments from receiving FDA approval.<sup>81</sup> Some argue that only large conglomerates have the financial backing to survive the time and expense of the FDA approval process. It is generally estimated that it can take up to fifteen years for the approval of a new drug and the process may cost up to five hundred million dollars.<sup>82</sup>

The heavy demands and requirements of the FDA approval process, and the time and expense involved in meeting them, serve

Notwithstanding any other provision of the Federal Food, Drug, and Cosmetic Act, a person may (1) introduce or deliver into interstate commerce a food, drug, device, or any other equipment; produce a food, drug, device, or any other equipment, solely for use in accordance with this Act if there have been no advertising claims by the manufacturer, distributor, or seller. ANTA § 6.

<sup>81</sup> Senator Daschle stated: It will be asked why this legislation is necessary. If a particular alternative treatment is so effective, then why can't it simply go through the standard FDA approval process? The answer is that the time and expense currently required to gain FDA approval of a treatment makes it very difficult for all but large pharmaceutical companies to undertake such an arduous and costly endeavor. 141 Cong. Rec. S9998-02, S99999 (July 14 1995)

<sup>82</sup> 104-28 Food and Drug Administration Performance and Accountability Act of 1995, 5. 1477, 104th Cong. 2nd Sess., p.6 (1996) (citing DiMasik Trends in Drug Development Cost, Times, and Risks, 29 Drug Information Journal 375, 382 (April-June 1995)); *see also*, 141 Cong. Rec. E1424-01, E1425 (July 12, 1995)

to limit access to the potentially innovative contributions of individual practitioners, scientists, smaller companies, and others who do not have the financial resources to traverse the painstakingly detailed path to certification.<sup>83</sup> Additionally, many alternative treatments involve natural products and are thus not able to be patented. Often without a patent on a medical treatment, an individual or company may not be able to recoup the significant financial investment necessary for FDA approval. Thus, the FDA approval requirements may prevent market access to relatively inexpensive, alternative treatments .-

The current bill provides that a patient has a right to receive any medical treatment from a health care practitioner<sup>86</sup>

**83** a remark by Representative Defazio - cite needed Dr. Najarian (recently acquitted of violating provisions of the FDCA) developed an antirejection serum for use in organ transplants. He indicated that the laboratory that originally slated to manufacture the drug was too small to handle the enormous cost of getting FDA approval for marketing, and dropped the project after about a year. Star-Tribune, Minneapolis-St. Paul (February 26, 1996).

141 Cong. Rec. at E1424.

**85** Amended from the original version and reintroduced on July 14, 1995. 141 Cong. Rec. S9998-02 (July 14, 1995); see *also* 1995 Cong U.S. 5 1035, 104th Cong, 1st Sess (July 14, 1995)

A similar bill was introduced on July 12, 1995, with wide bipartisan support, in the House of Representatives by Representative Defazio of Oregon (53 Co-sponsors: 28 Democrats and 23 Republicans). 142 Cong. Rec. H4115-01; see *also*, 1995 Cong. U.S. HR 2019, 104th cong., 1st Sess. (July 12, 1995).

The bill did not pass either the House or the Senate during the second session of the 104th Congress. However, the legislation will likely be re-introduced in the 105th Congress. The Tan Sheet 4(32):13-15, The News This Week (Aug. 5, 1996).

**86** Health Care Practitioner is defined as physician or another person who is legally authorized to provide health professional services in the State in which the services are provided. ANTA § 2((6).

even if such treatment is not approved, certified or licensed by the Secretary of Health and Human Services.<sup>87</sup> The treatment is permitted if there is no reasonable basis to conclude that the medical treatment itself, when used as directed, poses an unreasonable and significant risk of danger to the patient.<sup>88</sup> The term danger in the bill means any negative reaction that causes serious harm; occurred as a result of a method of medical treatment; would not otherwise have occurred; and is more serious than reactions experienced with routinely used medical treatments for the same medical condition or conditions.<sup>89</sup>

The ANTA includes what the sponsors state are carefully circumscribed conditions in order to protect the public yet allow greater access to treatment. The bill requires that a licensed health care practitioner personally examine the patient<sup>91</sup> and give the patient a written warning that the treatment has not been approved by the Federal Government. The health care

**87** *Id.* § 3(a).

**88** *Id.* § 3(b) (1)

**89** *Id.* § 2(2).

141 Cong. Rec. at S9999.

ANTA § 3 (a) (1). This provision was added to the amended bill to strengthen consumer protection. 141 Cong. Rec. S9998-02, S10002 (July 14, 1995)

The required written warning states: WARNING: this

food, drug, or device has not been declared to be safe and

effective by the Federal Government and any individual who uses

such food, drug, or device, does so at his or her own risk. ANTA

§ 3(b) (2) (B). This requirement was also added to the amended bill.

141 Cong. Rec. at S10002.

practitioner is prohibited from making any advertising claims regarding the efficacy of the proposed treatment<sup>93</sup> although she is not prohibited from making claims, in person, to her patient or from reporting her results to the medical community as long as she has no financial interest in the treatment.<sup>95</sup> Finally, the patient must give an informed consent prior to receiving the treatment.

ANTA § 3 (b) (4) . The bill strictly regulates the circumstances under which claims regarding the efficacy of a treatment can be made. It is designed to prohibit all claims by individuals for whom the underlying intent of promoting the treatment might be linked to personal financial gain. What this means is that there can be no marketing of any treatment administered under this bill. As such, I see very little incentive for anyone to try to use this bill as a bypass to the process of obtaining FDA approval. . . . In short, If an individual or a company wants to earn a profit off of their product, they would be wise to go through the standard FDA approval process rather than utilizing this legislation. Comments of Senator Daschle, 141 Cong. Rec. S9998-02, 510000.

ANTA § 3 (c) (1)

Subsection (b) (4) shall not apply to an accurate and truthful reporting by a health care practitioner of the results of the practitioner's administration of a medical treatment in recognized journals, at seminars, conventions, or similar meetings, or to others, so long as the reporting practitioner has not direct or indirect financial interest in the reporting of the material and has received not financial benefits of any kind from the manufacturer, distributor, or other seller for such reporting. *Id.* § 3(c) (2)

The ANTA, section 3(b) (3) provides that such individual has been informed in writing of the nature of the medical treatment, including -

- (A) the contents and methods of such treatment;
- (B) the anticipated benefits of such treatment; (C) any reasonably foreseeable side effects that may result from such treatment;
- (D) the results of past applications of such treatment by the health care practitioner and others; and
- (E) any other information necessary to fully meet the requirements for informed consent of human subjects prescribed by regulations issued by the Food and Drug Administration.

The patient must also desire the treatment and sign a statement



If the practitioner determines that the treatment itself was a danger to the patient, the practitioner is required to report the adverse reaction to the Secretary of Health and Human Services. The Secretary then has the ability to assess the potential danger and disseminate information regarding the nature of the danger.<sup>98</sup> The practitioner also is mandated to report beneficial effects of a life-saving treatment if they significantly exceed the positive effects that are expected from a conventional medical treatment for the same condition.<sup>99</sup>

The sponsors of the Senate and House bills assert that the ANTA is not intended to undercut the FDA's authority or its approval process, but rather the legislation seeks to compliment it.<sup>100</sup> The sponsors state the FDA would continue to protect the general public from unsafe drugs and medical devices,<sup>101</sup> while this legislation would allow individuals greater choice in their own medical care.

Curtailment of authority

Although there is sparse public information regarding the FDA's position on this bill, it is clear that the FDA is concerned this effect. *Id.* § 3(b) (6).

*Id.* § 4(a).

**98** *Id.* § 4(b).

*Id.* § 5.

<sup>99</sup> 141 Cong. Rec. at S9999 (discussing Senate version of the bill) ; 140 Cong. Rec. at E1657 (discussing House version of the bill)

*Id.*

about the effect such legislation would have on its existing authority to protect the public from unsafe drugs and devices. The FDA's Executive Assistant to the Commissioner commented that the agency's authority to remove products from the market would be compromised.<sup>102</sup> The FDA stated that pursuant to bill, the only authority the FDA would have when it learned of a dangerous treatment would be to publicize the fact. This deprives the FDA of any authority to remove products from the market even after there were reports of harm or deaths, or even when the product is clearly fraudulent.<sup>103</sup> Senator Harkin denied that the bill language supported the FDA's position.<sup>104</sup> He indicated there is nothing in this bill that would prohibit FDA from removing a clearly fraudulent or . . . dangerous product from the market. This is in the law.<sup>105</sup>

What Constitutes Danger

The FDA's mandate is to determine both safety and efficacy prior to allowing a drug or device on the market. In contrast, the ANTA permits only non-dangerous treatment options to be utilized by

102 The Gray Sheet, Industry & Washington Memos 22(32) (Aug.

5, 1996) . The FDAer likened the wakening of the agency's authority to the years before passage of the 1938 Food, Drug and Cosmetic Act, 'when FDA could do little more than warn the public and had no authority to assure the submission of reliable information or correct harmful situations. The Tan Sheet 4(32), The News This Week (Aug 5, 1996)

103 The Gray Sheet, Industry & Washington Memos 22(32) (Aug. 5, 1996)

*Id.*

8 *Health News Daily* No 155, p.3 (July 31, 1996)

health care practitioners, but it does not require a treatment to be effective. In fact, it is expected that ineffective treatment options will be placed into the stream of commerce and market forces will be sufficient to cull them out.<sup>16</sup> Thus, this bill would remove the FDA from being the initial forum where safety and effectiveness of a new drug or device is determined.<sup>17</sup>

It is also unclear what would actually qualify as an unreasonable and significant risk of danger. Representative Pallone stated that if a treatment is found to be harmful, it must be reported ... and it cannot be used again.<sup>18</sup> This comment makes it seem that if one patient is injured by the alternative treatment, the treatment must be discontinued. However, the language of the ANTA does not seem to support the Representative's assertion. The bill requires reporting adverse events only when they are worse than reactions that are caused by traditional therapies used for the same condition. However, potentially, serious harm can flow from the most common of treatments: One could die from an allergic response to aspirin. Since serious harm is a rare but actual risk of virtually any medical treatment, arguably only an extremely serious reaction, such as death, need be

<sup>16</sup> The Tan Sheet, *The News This Week*, 3(31) (July 1995).

<sup>17</sup> The court in *U.S. v. General Nutrition*, 638 F.Supp 556 (1986), stated that in cases where there is not recognized safety and efficacy of a new drug (or device), the initial determination of such issues should be the FDA. *Id.* at 561. 108 140 Cong. Rec. E1657-03, E1657; *see also*, 141 Cong. Rec. at S9999.

<sup>18</sup> ANTA § 2(2)(D).

reported. Therefore, it is unclear what types of negative reactions a practitioner, using a nonapproved drug or medical device, would be required to report. 110

Additionally a negative reaction must only be reported if it resulted from the alternative treatment itself.' Most health care practitioners are not researchers, and without a controlled study it may be virtually impossible to determine the cause of an adverse reaction. Many of these unapproved medical practices would also be administered in conjunction with traditional therapies, making it even more difficult for the practitioner to assess the source of the negative reaction. Because of the ambiguity, many practitioners may not report a serious adverse reaction to an alternative treatment.

Additionally, even if a practitioner reported a dangerous outcome to an unapproved treatment, the FDA would likely have difficulty reviewing any reported instances of harm. The report would not be derived from a controlled clinical study. Because of the likelihood of the patient receiving other treatments in addition to the unapproved treatment, it may be impossible for the FDA assess the nature of the danger of an alternative treatment.

It is also unclear if the definition of danger would include a safe but ineffective treatment that caused harm simply by

110 The FDA commented that under the bill, the way danger is defined, a negative reaction may not be reported even though it's a very serious one. 58(32) The Pink Sheet, In Brief (Aug. 5 1996)

' ANTA § 2(2) (B).

preventing a patient from seeking other helpful therapy. The language of the ANTA seems to focus on the nature of the treatment and not that another treatment was foregone that could have been effective. Delay in the institution of effective therapy caused by the use of an ineffective medical treatment can cause a disease to progress beyond the ability to treat.<sup>2</sup> However, individuals suffering from terminal diseases for which there does not exist a conventional effective treatment should arguably have the right to seek alternative treatments that may show a potential for cure. The issue of danger seems to become less important when the patient is terminal and without other viable options.<sup>3</sup>

HamPer Important Data Collection

The FDA has stated that this bill might hamper the current drug development process and reduce or eliminate controlled trials which allow the systematic and scientific collection of data.<sup>4</sup> Representative Waxman also expressed concern with allowing patient access to unapproved treatments without the concomitant requirement of data collection to assess safety and efficacy. What we need to do is support research, to make drugs available, if possible, while they are experimented upon to decide whether they are efficacious.

[W]e don't want people taken advantage of and exploited by

112 *Durovic v. Richardson*, 479 F.2d 242, 250 (7th Cir. 1973)

113 *But see contra*, (doesn't the KB have statements by physicians saying that cannot tell until dead?)

~ *See generally*, 58(32) The Pink Sheet, In Brief (Aug. 5 1996)

snake oil salesmen.<sup>5</sup> It is only by the use of carefully controlled and monitored studies of experimental treatments that data may be obtained to understand the benefits a patient may receive, or the risks that may result, from a treatment.

The potential lack of data collection also relates to the issue of informed consent. The bill states that a patient has not given informed consent until she has been apprised, in writing, of the risks and benefits of the proposed treatment, including the results of past applications of such treatment by the health care practitioner and others.<sup>6</sup> If controlled studies and data collection are not required, the patient may have access to merely antidotal evidence. The mechanism by which a health care practitioner should gather information from others is uncertain and even if obtained, the information would likely be antidotal as well. This may have the effect of having a treatment gain wide spread use without the accompanied gathering of data and knowledge regarding the safety and efficacy of the treatment.<sup>7</sup>

#### Advertising

The FDA views the ANTA's labeling and advertising restrictions to be inadequate to assure patients will not be

~ The Pink Sheet, Trade & Govt. Memos, 58(10) (March 4 1996)

116 ANTA § 3(3) (D)

' The FDA stated that: Allowing wide dissemination of a treatment and subsequent widespread use combined with little accountability or liability significantly reduces the incentive for manufacturers and health care practitioners to conduct studies of safety and effectiveness. The Tan Sheet, The News This Week, 4(32) :13-15 (Aug. 5, 1996).

misled into accepting unsafe treatments.<sup>18</sup> the FDA wants to insure that terminally ill people - our most vulnerable citizens - are not victims of health fraud.<sup>9</sup> The proponents respond that the bill prohibits any product labeling which is false or misleading. The FDA, of course, wants to approve each and every label. This is a degree of control which is simply not possible if we are to make alternative treatments available.<sup>20</sup>

Access to Treatments

Additionally, the FDA claims that the FDCA currently allows patients access to experimental treatments under the IND process.<sup>21</sup> The FDCA provides that new drugs and medical devices can be made available to patients early in the testing process. <sup>122</sup> The IND process also has provisions to potentially accommodate a small group of patients or even an individual patient. Treatment IND's for severely debilitating and life-threatening diseases also provide for early availability of certain treatments.<sup>23</sup>

Contrary to the FDA's assertions that medical treatment was appropriately available via the IND process, James Gordon, MD, the

<sup>118</sup> *Id.*

*Id.*

<sup>120</sup> 141 Cong.Rec. at 10002.

<sup>121</sup> 21 U.S.C.A. § 355 *et. seq.*

<sup>122</sup> The FDA stated that a patient could receive. Virtually any new medical treatment for which FDA has oversight. The Pink Sheet, In Brief, 58(32) (Aug. 5 1996).

<sup>123</sup> The Tan Sheet 4(32):13-15, The News This Week (Aug. 5, 1996)

Director of the Center for Mind-Body Medicine testified that the FDA approach often did not work within the alternative medicine approach. In treating each patient with a holistic approach, a practitioner may treat each patient with different combinations of substances. Doctor Gordon stated that if he were to ask for an IND for every modality, herb and supplement that he used, he'd be spending [his] whole life petitioning the FDA.<sup>24</sup> As noted above, the language of this bill presents several questions and concerns regarding its scope and ultimate effectiveness. Additionally, although it is certain that Congress intended some type of Practice of Medicine Exception, this bill seems to inappropriately restrict the FDA's oversight of the drug and device approval process.

#### **Kassebaum Bill**

Unlike the ANTA, Senator Kassebaum's initiative, seeks to make widespread changes to the FDCA. Although the Food and Drug Administration Performance and Accountability Act of 1996 (Kassebaum Bill) did not pass in the 104 Congress, second session, it is expected to be reintroduced this session. Although the bill does not specifically mention the Practice of Medicine Exception,<sup>25</sup> some of the proposed reform would clarify the dividing

**124** *Id.*

**125** There is also a bill in The House of Representatives, which seeks to reform the FDCA. H.R. 3119, 104th Congress, 2nd Sess. (1996) . The House Report specifically states: Nothing in the Act shall be construed to limit or interfere with the authority of a health care practitioner, licensed by law to administer drugs and devices, to prescribe or administer any legally marketed drug or device to a patient for any condition or disease within a legitimate health care practitioner-patient relationship. H.R.

3199, § 909.



line between appropriate FDA action and medical practice. One aspect of the bill addresses the issue of the availability of drugs and devices that have not yet received FDA approval and the use of approved drugs and devices for unapproved uses. The Kassebaum Bill seems to strike more of a balance between the issues set forth above: That is, the FDA's desire to retain rather stringent controls over the approval process to assure safety and efficacy of approved drugs and devices, and that of the medical profession and patients who want increased access to unapproved medical treatments.

The bill provides increased access to unapproved therapies but only if the investigational provisions of the FDCA are complied with.<sup>26</sup> A patient with a seriously debilitating disease<sup>27</sup> would have access, through a licensed health care practitioner, to such unapproved drug or device if there was no other available treatment that proved satisfactory for the patient's condition. In order to receive the treatment, the risk from the investigational drug or device must not be greater than the risk from the patient's condition.<sup>28</sup> Based on the language of the Kassebaum Bill, it is

**126** ~ 1477, § 202, *Access to Unapproved Therapies*. Unlike the ANTA which permits access to unapproved medical treatments without going through the FDA approval process. See *supra*, footnotes 74-126 and accompanying text.

**127** The committee stated that the broad language regarding serious conditions was purposeful. Illnesses that do not cause death can nonetheless destroy the lives of both patients and their families. The committee therefore intends that the seriousness of an illness be given broad consideration, to take into account of all the circumstances involved. Kassebaum Bill at 23.

**128** *Id.*

unclear what must be shown in order to satisfy the requirement that no other conventional treatment has been effective. Issues arise as to whether other treatments have to be tried first, and if so, for how long. Also whether the non-approved therapy be tried simultaneously with approved measures. The current wording of the proposed provision seems difficult for a practitioner to apply. Additionally, the Kassebaum bill provides that the FDA inform the medical community of available investigational drugs and devices. **129**

The Kassebaum bill also addresses the unapproved use by the medical profession of approved drugs. Unapproved uses of approved new drugs account for perhaps half of the use of drugs in this country today. In some specialty areas, such as cancer, of flabel uses can be 60 percent or higher.<sup>30</sup> The cumbersome and costly FDA approval process for a new use of an approved drug or device is often a disincentive for manufacturers to file supplemental applications. **131** The result is that many unapproved uses have become standard medical practice yet the drug or device label does not reflect such use. The Kassebaum bill seeks to address this problem by attempting to streamline and encourage the approval process for additional uses of an already approved drug or

**129** The Committee on Labor and Human Resources stated that [t]his provision will help to ensure that all patients will have equal knowledge of and access to investigational products. Kassebaum Bill at 23.

**130** *Id.* at 35.

*Id.*

device **132**

The Kassebaum Bill provides physicians and patients wider latitude to gain access to unapproved drugs and devices. Also by encouraging approval of non-approved uses of an approved drug or device, it gives health care practitioners increased knowledge of proper uses.

**The Current Dilemma Regarding Unapproved Uses**

Currently, once the FDA approves a drug or medical device for a particular use it may be placed on the market. However, after a drug or device is available to the public, the FDA does not regulate its use. In fact it is quite common for a physician to prescribe an approved drug for uses that are not within the indications articulated by the FDA.<sup>33</sup> Conversely, it is illegal for a physician to prescribe, use or administer an unapproved drug or medical device.<sup>34</sup> Under both scenarios, a physician is prescribing a medical treatment that has not been deemed safe and effective by the FDA, yet the first instance is legal and the second could open the doctor to criminal charges.

Unapproved Use of FDA Approved Treatments

The FDA seems to view its mandate as establishing the safety and efficacy of drugs and devices under the conditions set forth by the manufacturer,<sup>35</sup> not whether a medical treatment is safe for the

**132** *Id.* at 35-36.

*See supra*, fn.133 and accompanying text.

21 U.S.C.A. § 333.

~  
cite to 37 FR 16503?

use it is actually prescribed. The general public is likely under the impression that if their doctor is prescribing a medical treatment, it has been tested and approved for that particular use. However, based on the Practice of Medicine Exception, the FDA has specifically recognized the legality of using drugs<sup>36</sup> and devices<sup>37</sup> for purposes other than those for which they have been approved.

A drug is placed on the market with recommendations for use which are developed jointly by the manufacturer and the FDA after reviewing clinical data. A former Commissioner of the FDA stated that: The FDA's responsibility is limited to insuring that there are sufficient data to conclude that the drug is safe and effective under the conditions of use proposed by the manufacturer. The physicians of this country ... [are then free] to prescribe the drug as they see fit.<sup>38</sup> The new drug provision of the FDCA only applies when drugs are shipped in interstate commerce; they do not apply to subsequent physician action.<sup>39</sup> Once the new drug is in a local pharmacy ... the physician may, as part of the practice of medicine, lawfully ... vary the conditions of use from those

**136** *FTC v. Simeon Management Corp.*, 532 F.2d 708, 717 (1976).

**137** By analogy, the off-label use of a medical device is also a matter of medical judgment. *Klein v. Biscup*, 673 N.E.2d. 225, 231 (19 )

**138** *New Drugs Used for Nonapproved Purposes (Methotrexate for Psoriasis)*; *Hearings Before a Subcommittee of the Committee on Government Operations*, 92nd Cong., 1st Sess at 133 (July 29 & 30, 1971)

*Id.*

approved in the package insert.<sup>17</sup>

Arguably, if the FDA's mandate is to protect the public from harmful drugs and medical devices, it should test and approve all proposed medical treatments. For example, a manufacturer receives FDA approval to market a drug for the treatment of AIDS. Although during the testing process some of the patients experiences severe side effects to the medication, the FDA approves the drug, because the overall benefits experienced by the terminally ill patients outweighed the potential harm. Physicians began noticing that AIDS patients with acne who used this drug, had marked improvement in their skin condition. Thereafter, dermatologists began using the drug to treat their acne patients. Some of the patients who were being treated for acne were seriously injured from the side effects of the medication.

In the above hypothetical, the FDA approved use of the drug for the treatment of AIDS patients was determined safe and effective given the circumstances of use. If the manufacturer had sought to receive approval for this drug merely for the treatment of a cosmetic problem, the FDA might have determined that the severe side effects made the drug unsafe given the nonessential nature of the treatment. However, the FDA has taken the position that the Practice of Medicine Exception prevents the agency's intrusion into a physician's unapproved use of an approved drug

<sup>17</sup> 37 Fed. Reg. at 16503; *see also*, *Bristol-Myers Squibb Co.*  
*V. Shalala*, 91 F.3d 1493, 1496 (D.C. Cir. 1996)

within the confines of the doctor-patient relationship.<sup>41</sup> The FDA's position was seriously questioned in a congressional

subcommittee that was holding hearings on the issue of new drugs used for nonapproved purposes, specifically the use of methotrexate (approved as a cancer drug) for the treatment of psoriasis. **142** Senator Fountain stated that use of a drug should not be permitted until its safety and effectiveness is proven with respect to its intended use. ' He went on to comment, I believe with certainty, that is one of the things which Congress had in mind back in 1962 when it enacted the drug amendments.' [H]uman life is precious and one human life lost needlessly by unauthorized drug therapy is one too many. Particularly when the disease does not threaten life.<sup>45</sup> Although physicians may be liable for malpractice if they use a drug for an unapproved use, the obvious reality is an after

**141** Courts have also supported this position. The Act does not . . . limit the manner in which a physician may use an approved drug. Once a product has been approved for marketing, a physician may prescribe it for uses or in treatment regimens or patient populations that are not included in approved labeling. *Klein*, at 864 (quoting Use of Approved Drugs for Unlabeled Indications, 12 FDA Drug Bulletin 4 (April 1982)).

Although the FDA has promulgated regulations that purport to regulate the unapproved use of approved drugs, the regulations have never been finalized or otherwise enforced. *See supra*, fn. 36-46 and corresponding text.

**142** *See generally, New Drugs Used for Nonapproved Purposes (Methotrexate for Psoriasis); Hearings Before a Subcommittee of the Committee on Government Operations, 92nd Cong., 1st Sess (July 29 & 30, 1971)*

*Id.* at 104.

**144** *Id.*

*Id.*

the fact award of money damages

for a sacrificed life . . . is small solace to those affected.<sup>146</sup> In a highly industrialized and technical society with

biotechnical advances almost daily, it is difficult for the average consumer to protect herself from unsafe or ineffective products.<sup>147</sup> Thus the FDA's purpose is to protect the general public in areas that are largely beyond self-protection.<sup>148</sup> If a drug, that has never been tested and proven safe and effective for a use for which it is prescribed, how is the public protected?

Although the FDA claims that it does not have authority to regulate in this area, the Practice of Medicine Exception does not appear to prevent the opposite conclusion. There is no bright-line distinction between the mandate of the FDA and the practice of medicine, in fact, the broad reaching jurisdiction of the FDA guarantees that medical practice will be regulated to some extent. The *9/1 kg Containers* court stated that if the FDA did not regulate the practice of medicine - what does the statute do? Congress gave the FDA comprehensive powers to license the manufacture of drugs and limit their sales. To regulate drugs is to be 'involved' in the 'practice of the healing arts.'<sup>149</sup> In order to protect the general public, [s]omeone must draw on knowledge about risks and

*Id.*

- See generally, *U.S. v. Dotterweich*, 320 U.S. 277, 280 (1943) *reh'g den.* 320 U.S. 815.

**148** *Id.*

*U.S. v. 9/1 kg. Containers*, 854 F.2d 173, 176 (7th Cir. 1988) , *cert den.* 489 U.S. 1010 (1989)

needs and decide how much risk is too much; that someone is the Executive Branch of the government.<sup>50</sup> The FDA's task in enforcing the FDCA is undeniably involves making judgments about medical subjects.

The *9/1 kg Containers* court, in narrowly interpreting the scope of the Practice of Medicine Exception, stated that it should be restricted to the ability of the states to license and discipline the medical professions. The court noted that the states have the power to set appropriate medical qualifications and practitioners who do not use drugs (i.e., chiropractors) may go on as before.<sup>51</sup> Although the court's comments were related to the compounding of unapproved drugs, the rationale also supports the position that the FDA has authority to assure the safety and effectiveness of all drug and medical device usage.

#### Use of an Unapproved Drug

Health care practitioners are strictly prohibited from prescribing or otherwise using an unapproved drug when treating a patient. Obviously, an unapproved drug has not been tested for safety and efficacy by the FDA. However, the basis for and rationale of the FDA decision to allow the unapproved use of an approved drug or device but not the use of an unapproved medical treatment is not clear. Arguably, the approval process of a drug

so *Id.*

The court noted that the Senate in 1935 stated the statute is not intended as a medical practices act and [would] not interfere with the practice of the healing art by chiropractors and others. *Id.* at 176-77.



or device determines some level of safety for human use. But if such treatment is used in a completely different context from its initial approval, the safety of the public is put into question.

The *Weaver v. Reagen*<sup>152</sup> court stated that FDA approved uses were not intended to interfere with the practice of medicine and should not be construed to preclude physicians from using their best judgment in the interest of the patient.<sup>53</sup> If this is the case, then why should physicians' best judgment to use an unapproved drug or device be criminalized?

A recent example of the FDA's position against the unapproved use of drugs or devices is the criminal trial of a Texas doctor which began on January 7th of this year.<sup>54</sup> Dr. Burzynski, a Houston physician is being criminally prosecuted for violating the FDCA by his use of an unapproved drug to treat cancer patients. The doctor invented, patented, and manufactured an antineoplaston treatment which he claims serves as a biochemical switch to turn off cancer genes and prevent cancerous cells from multiplying in the human body.<sup>55</sup>

Burzynski has only treated six of his patients under FDA approved clinical trials and on the day his trial was to begin,

**152** 886 R.2d 194 (8th Cir. 1989)

*Id.* at 198.

**154** The Dallas Morning News, *Houston Cancer Doctor's Trial Delayed*, 1SA (January 7, 1997)

**55** Chicago Sun-Times, *Rebel Cancer Doctor To Go On Trial For Fraud*, 19 (Jan 6, 1997)

approximately three hundred patients were receiving the drug.<sup>156</sup> The U.S. Attorney stated that Burzynski was first informed that he needed FDA approval in 1977.<sup>157</sup> Over a two decade span, the doctor treated over three thousand patients with his drug, yet never complied with the FDA's approval requirements.<sup>158</sup>

The FDA finally ordered Burzynski to not distribute the drug in interstate commerce.<sup>159</sup> The doctor was also informed by the Texas Department of Health that use of the drug within the state was also illegal because it did not have FDA approval for use.<sup>160</sup> The defense states that Dr. Burzynski's actions were to fulfill his oath as a physician, which states that doctors do no harm, and he believes that to withhold (antineoplaston) is to do harm.<sup>161</sup> Interestingly, the indictment against Burzynski does not allege that the administration of the antineoplaston treatment itself has caused any harm.<sup>162</sup> In fact, many of Burzynski's patients swear

<sup>156</sup> Chicago Sun-Times, *Rebel Cancer Doctor To Go On Trial For Fraud*, 19 (Jan 6, 1997)

<sup>157</sup> The Dallas Morning News, *Jury Told Doctor Delivered Drug Illegally*, 19A (January 8, 1997).

<sup>158</sup> Los Angeles Times, *Cancer Pioneer on Trial After Bucking the System*, A1E (January 12, 1997).

<sup>159</sup> Los Angeles Times, *Cancer Pioneer on Trial After Bucking the System*, A16 (January 12, 1997)

*U.S. v. Burzynski*, Indictment p.9 (filed Nov. 20, 1995)

<sup>160</sup> The Dallas Morning News, *Jury Told Doctor Delivered Drug Illegally*, 19A (January 8, 1997)

<sup>161</sup> See generally, *U.S. v. Burzynski*, Indictment (filed Nov.

20, 1995). This article distinguishes harm flowing from the medical treatment from injury that may result from a patient forgoing other potentially effective treatments. See *supra*, fn.

that his treatment has saved their lives when practitioners who provided more conventional treatment told them to prepare for death.<sup>163</sup> One father, testifying to a congressional committee about his son's treatment by Burzynski stated: Burzynski is guilty of saving lives illegally. If helping sick people live is irrelevant to enforcing rules, than the police should start pulling over ambulances and fire trucks for speeding.<sup>164</sup>

If Burzynski's drug had received approval for any type of treatment, even if completely unrelated to the treatment of cancer, he would not be being prosecuted. In either instance however, the issue of safety and efficacy would not have been determined by the FDA for the use of the treatment in cancer patients. This inconsistency, in oversight and enforcement for essentially the same action, should be reconciled.

#### **Reconciling FDA Action Regarding Unapproved Uses**

It should be noted that under both of the above scenarios, patients have malpractice claims against their health care

114-115 and accompanying text.

<sup>163</sup> Los Angeles Times, *Cancer Pioneer on Trial After Bucking the System*, A16 (January 12, 1997). The Oversight and Investigations Subcommittee of the Commerce Committee heard testimony from Burzynski patients who testified that his treatment cured them or helped them. Government Press Releases, *Background on Access to Medical Treatment Hearing*, (February 22, 1996). A group of 100 patients and their families gathered outside the federal courthouse in support of Burzynski on the first day of his criminal trial. Houston Chronicle, *Cancer Doctor Says Patients Need Him* 13 (January 7, 1997).

<sup>164</sup> Cong. Testimony, *Access to Medical Treatment Act S1035* (July 30, 1996)

practitioner if the drug or device causes harm. **165** In contrast to the tort system which merely attempts to compensate for tragedies that have already occurred, the FDA is empowered by Congress to intervene and prevent harm to the general public. Therefore, the FDA should not defer to a retrospective solution when its mandate is prospective in nature.

As indicated, the FDA currently permits some unapproved uses of drugs and medical devices, without proof of safety and efficacy, yet prohibits others. This inconsistent enforcement scheme does not appear to be required by Congress based on the Practice of Medicine Exception. If the FDA's goal is to protect the general public from harm, not merely approve a drug or device for a specific use - all uses should arguably be approved. The *U.S. v. Article*<sup>7</sup> court stated that the FDA's responsibility was to protect the ultimate consumer, which included protection of the ignorant, the unthinking and the credulous.<sup>67</sup> In this case, the ultimate consumer is the patient and therefore it should be the FDA's responsibility to protect patients, even from their doctor if

<sup>7</sup> See e.g., *Klein*, 673 N.E.2d at 231 (an off-label use of a medical device may subject a physician to malpractice liability) It has been argued that FDA involvement in this area is not necessary because market forces are sufficient to control physician's unapproved uses via malpractice (deviation from FDA approved use may be evidence of negligence) and insurance reimbursement (insurer may not reimburse unapproved medical treatments) . Walsh & Pyrich, *Rationalizing the Regulation of Prescription Drugs and Medical Devices: Perspectives on Private Certification and Tort Reform*, 48 Rutgers L. Rev. 883, 1041 (1996)

<sup>67</sup> 409 F.2d 734 (2nd Cir. 1969)

**167** *Id.* at 740.

28 (1995)

necessary. By this statement I am not implying that physicians or other practitioners intentionally harm patients. However, doctors often do not have extensive training in pharmacology and may be ill equipped to decide whether a prescription for an unapproved use of an approved medication is proper.

Additionally, under the current system, a potentially uninformed physician may have a difficult time in obtaining information about the proper unapproved uses of an approved medical treatment. The FDA prohibits a manufacturer from advertising an unapproved use of its product.<sup>7</sup> If the FDA permits unapproved uses, the general public is better protected if physicians have access to as much truthful information about drug or medical device usage. If the FDA refuses to mandate safety and efficacy testing for all uses of a drug or device, it should not leave the public even more unprotected by preventing health care practitioners access to all available truthful information regarding proper usage of the unapproved use. Therefore, a situation may arise where a doctor is prescribing an approved drug or device for an unapproved use, with very little information at her disposal.

To leave the general public even more unprotected, all states do not mandate that the patient be informed of this situation prior to deciding whether to consent to treatment. Patients do not have to be informed that the medication or device they are being

*Washington Legal Foundation v. Kessler*, 880 F.Supp. 26, 43

prescribed is not approved by the FDA for the patients' intended use. The *Klein* court stated that [o]f f-label use of a medical device is not a material risk inherently involved in a proposed therapy which a physician should disclose to a patient prior to the therapy. ... Accordingly, we conclude that failure to disclose FDA status does not raise a material issue of fact as to informed consent.<sup>169</sup> The patient is therefore potentially at greater risk once a device or drug has been approved than if the same item merely had investigational status. When a drug or device is still being tested but is available to human subjects, the FDA provides for stringent controls on informed consent.<sup>170</sup>

However, from a practical standpoint, the FDA approval process does not dovetail with the immediacy of treatment needs within the doctor-patient relationship. If the FDA controlled all uses of a drug or medical device, medical practice as we know it would grind to a halt. Additionally, enforcement of such a policy would be virtually impossible, especially given already strained agency resources. To rectify this problem, the FDA seems to have two reasonable choices: Require approval for all uses but significantly expedite the review and approval of new uses of

**169** *Klein*, 673 N.E.2d at 231 (doctor failure to inform patient that device implanted in spine did not have FDA approval for such use did not violate informed consent requirements); *see also, In re Orthopedic...*, 1996 WL 107556 (1996) (The FDA labels given to a medical device do not speak directly to the medical issues surrounding a particular surgery, They are not, therefore, required to be disclosed pursuant to the law of informed consent)

**170** See *e.g.*, 21 U.S.C.A § 355(i) (3).

already approved drugs and devices, or allow unapproved uses under certain circumstances.

The issue essentially boils down to a safety verses efficacy issue. At present, the FDA appears satisfies that a drug, once approved for some use is relatively save, even if used for an unapproved use. It seems to follow that if an unapproved medical treatment is not harmful, the FDA should allow its use until efficacy is proven or disproven. Under either scenario, the FDA retains the power to prohibit the use of use of a drug or device, whether approved or unapproved, when incidents of unreasonable harm are shown.

### **Conclusion**

The FDA has recognized a Practice of Medicine in the FDCA although it is not specifically referred to within the statutory scheme. This exception has not been well defined by the agency and its implementation currently leads to inconsistent an inappropriate results. The FDA should reconcile its position on the unapproved uses of approved medical treatments and its prohibition regarding the unapproved use of drugs and devices.

Practicality dictates that it would be virtually impossible for the FDA to approve every use of a drug or device before allowing its use by the medical community and the public at large. Therefore, it seems reasonable to permit unapproved treatments under controlled, FDA supervised, circumstances. The Kassebaum Bill begins this process.

Allowing greater medical access to treatments is likely in the

public interest and is reasonably enforceable. However, it should be a requirement that a patient is informed of the lack of FDA approval, in either the case of an unapproved use of an approved drug or device, or the use of an unapproved medical treatment. In both situations, safety and efficacy has not been ascertained and a patient should have a right to this important information prior to consenting to medical treatment. The FDA within its existing mandate has the authority to rectify this inappropriate inconsistency.