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Q & A
"Experimental" Treatment and Medicaid

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Q. The state Medicaid agency has denied my client's request for a particular treatment as "experimental." The notice of denial does not explain how it determines whether a treatment is experimental. Are there Medicaid statutory, regulatory, or other requirements that govern the determination of whether a treatment is experimental, or do states have complete freedom to make the determination? Are the rules different for adults and children under age 21?

A. States are permitted to exclude coverage for experimental services for all beneficiaries, including children. The definition of what is an experimental service is a flexible one that is not found in statute or regulations, but has been developed through caselaw. States should, however, have specific standards for determining whether a service is experimental.

Discussion

State Medicaid programs generally will cover only services that are determined to be necessary. The Medicaid statute does not, however, define medical necessity for services; in fact, nowhere in the statute is the phrase mentioned. And, although Congress mandated the inclusion of specified services in state Medicaid programs, it did not explicitly define the minimum level of each service to be provided, nor has the federal agency done so. States have significant leeway to determine the amount, scope, and type of services that they will cover. The Medicaid Act and regulations establish broad guidelines with which states must comply when designing service coverage standards. For example:

- States must establish "reasonable standards" for determining the extent of medical assistance provided that are consistent with the objectives of the Medicaid Act.[1]

[1] 42 U.S.C. § 1396a(a)(17).

- Services must be "sufficient in amount, duration, and scope to reasonably achieve their purpose."[2]
- States may place appropriate limits on a service based upon such criteria as "medical necessity" or on utilization review criteria.[3]

The Medicaid Act does prescribe a specific standard for determining necessity for services for children and youth. The Early and Periodic Screening, Diagnosis, and Treatment (EPSDT) requirements apply to treatment services for all Medicaid beneficiaries under age 21 and require that states cover services when necessary "to correct or ameliorate" physical and mental illnesses and conditions, regardless of whether such services are covered for adults in the state Medicaid program.[4]

According to the federal agency, states are not permitted to put dollar or hourly limits on EPSDT services, or other limits unrelated to medical necessity. But, they may place tentative limits on services; require prior authorization for services; and provide services in the most economic, yet equally effective, mode.[5] In addition, states may exclude services that are unsafe or experimental.[6] The federal agency does not, however, define what "experimental" means. Accordingly, the silence of the statute and regulations and lack of specificity from the agency have left it to the courts and the states to define what "experimental" means in Medicaid.

The Leading Cases

Courts have long recognized that states may exclude coverage for experimental treatment – both for adults and children.[7] At that same time, courts have had to contend with the fact that the "experimental" is not generally recognized by health care providers as "medical" concept and that the term generally arises only in the Medicaid and Medicare context.[8]

The leading case on this subject is *Rush v. Parham,* which concerned coverage of sex reassignment surgery.[9] The Court held that states may reasonably exclude experimental treatment. In reaching this conclusion, the

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[2] 42 C.F.R. § 440.230(b).
[3] 42 C.F.R. § 440.230(d).
[4] 42 U.S.C. § 1396d(r)(5).
[5] Memorandum from Christine Nye, HCFA Medicaid Director, to Regional Administrator Region VII (1991), available from NHELP.
[6] Letter from Rozann Abato, Acting Director of Medicaid Bureau, to State Medicaid Directors (May 26, 1993); Letter from Albert Benz, Associate Regional Administrator (Region X) to Jean Schoonover, Chief of Health & Welfare Programs (April 30, 1991); Memo from Christine Nye, HCFA Medicaid Director, to Regional Administrator Region VI (Dec. 10, 1990), available from NHELP.
[7] *See, e.g., Rush v. Parham,* 625 F.2d 1150, 1156 (5th Cir. 1980).
[8] *See McLaughlin v. Williams,* 801 F. Supp. 633, 642 (S.D. Fla. 1992).
[9] 625 F.2d 1150 (5th Cir. 1980).

court looked to guidance from the federal agency on the Medicare program. According to an explanatory letter, whether a treatment is experimental should depend on

> whether the service has come to be **generally accepted by the professional medical community** as an **effective and proven treatment** for the condition for which it is being used. If it is, Medicare may make payment. On the other hand, if the service or treatment is not yet generally accepted, is **rarely used, novel, or relatively unknown**, then **authoritative evidence** must be obtained that it is safe and effective before Medicare may make payment.[10]

Based on this Medicare guidance, the Court found that Georgia could reasonably exclude treatment for experimental services in its Medicaid program.

Many other courts have taken their cue from this decision and applied what some refer to as the "*Rush* definition." For example, in *Weaver v. Reagen,* the Eighth Circuit cited the *Rush* definition to hold that prescribing AZT for use beyond its FDA-approved uses was not experimental.[11] In so holding, the court rejected the state's arguments concerning the lack of scientific data from clinical trials documenting the efficacy and safety of AZT outside of the FDA-approved guidelines indicated that the treatment at issue was experimental. The court held that

> [T]he fact that FDA has not approved labeling of a drug for a particular use does not necessarily bear on those uses of the drug that are established within the medical and scientific community as medically appropriate. It would be improper for the [State] to interfere with a physician's judgment of medical necessity by limiting coverage of AZT based on criteria that admittedly do not reflect current medical knowledge or practice.[12]

See also Ruth v. Kizer, 8 Cal. App. 4th 380 (1992) (upholding denial of coverage of oxygen treatment for individuals with chemical sensitivity as investigative and not supported by complete and specific documentation, citing *Rush*).

Though *Rush* concerned coverage of a service for adults, courts have also applied its reasoning in EPSDT cases. For example, *McLaughlin v. Williams* also applied the *Rush* definition in the EPSDT context, but used it to develop a flexible framework for determining whether a procedure is experimental. In that case, the district court granted a preliminary injunction compelling the Florida state Medicaid agency to cover a liver-small bowel transplant for a 12-month-old.[13]

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[10] Id. at 1156, fn. 11 (emphasis added), quoting Enclosure # 2 to Intermediary Letters Nos. 77-4 & 77-5, (1976 Transfer Binder), Medicare & Medicaid Guide (CCH) ¶ 28,152 (1976).
[11] 886 F.2d 194, 198-99 (8th Cir. 1989).
[12] *Id.* at 198.
[13] 801 F. Supp. 633 (S.D. Fla. 1992).

The court acknowledged that "the term experimental is difficult to define precisely," and followed the "rubric" articulated in *Rush.*[14] Accordingly, in considering whether a rarely used, novel or relatively unknown treatment is medically necessary, authoritative evidence can be used to show that it is "safe and effective."[15] Further, the court identified the following factors that should be considered:

- The mortality of patients over the period in which the procedure has been performed;
- How frequently the procedure has been performed and where it has been performed;
- The success or failure of the procedure;
- The reputation of the doctors and medical centers who are performing the procedure;
- The long-term prognosis of the patients who have had the procedure preformed; and to what extent medical science in that area has developed rapidly.[16]

The Court also rejected the defendant's contention that as a matter of law, until a particular, but unspecified, amount of time has passed, and until the new procedure is accepted generally, the procedure must be deemed experimental. "On the record before us, this view appears to be both too narrow and too imprecise, and it ignores the rapid rate of advancement of medical science in the field of transplants."[17] Acceptance in the medical community, the court cautioned, should not be dispositive when the procedure at issue is performed in only a few locations by a few specialists. In addition, the fact that rapid advances are being made in this area means that there will be a significant lag between knowledge about the efficacy of a particular testimony and publishing of a report in the medical press.[18] Moreover, the fact that a procedure is in clinical trials is not dispositive.[19]

Similarly, in *Miller v. Whitburn*, a case involving a liver-bowel transplant for a child, the Seventh Circuit applied *Rush* to determine whether services are experimental. The court held that federal courts may review a state's definition of "experimental" to ensure that it is consistent with the *Rush* formulation.[20]

In *Oklahoma Chapter of the American Academy of Pediatrics v. Fogarty*, the district court found that the Oklahoma Medicaid agency could exclude

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[14] *Id.* at 639.
[15] *Id.*
[16] *Id.* at 639. Notably, these factors were later cited by the Seventh Circuit in *Miller v. Whitburn,* 10 F.3d 1315 (7th Cir. 1993).
[17] *Id.*
[18] *Id.* at 640.
[19] *Id.*
[20] *Miller v. Whitburn,* 10 F.3d 1315, 1319-20 (7th Cir. 1993).

coverage of a particular medication to treat asthma for children under age 12, because its use was neither approved by the FDA nor generally accepted by the medical community as effective and proven treatment.[21] The court was careful, however, to acknowledge the holding in *Weaver* that FDA approval could not be an absolute prerequisite for coverage.

In a Utah state court appeal from an administrative hearing, *Peterson v. Utah Dep't of Health, Div. of Health Care Financing,* the court upheld the state Medicaid agency's refusal to cover growth hormone treatment for an infant with short bowel syndrome.[22] At the agency hearing, the petitioner's doctor testified that the child's condition had improved while undergoing treatment, but was unable to definitively state that the treatment was responsible for this improvement. Moreover, he testified that the growth hormone treatment was not yet widely used for this purpose. Accordingly, the Administrative Law Judge (ALJ) applied the state regulation defining experimental treatment and found that the treatment was experimental.[23] The court upheld this determination on appeal, noting that the decision that a treatment is experimental is "highly fact dependent" and would be upheld if supported by substantial evidence.[24] In addition, the court found that the state's definition of experimental treatment was substantially identical to the formulation in *Rush* and therefore permissible.

Other EPSDT Cases

Notably, none of the preceding cases suggested that there was anything significant about the fact that *Rush* concerned services for adults. In another EPSDT case, however, the Georgia Court of Appeals took a different approach to determining whether a treatment should be covered. While these cases do not use the term "experimental," the principles at issue are fundamentally similar.

First, in *Georgia Dep't of Community Health v. Freels,* the court addressed whether EPSDT required coverage of hyperbaric oxygen therapy (HBOT) for a child with cerebral palsy. [25] Used for many years to treat individuals suffering from diving accidents and other problems, HBOT has only recently been used to treat children with cerebral palsy. The Freels family paid for their son to receive a limited number of HBOT treatments, and the treating providers noted improvements in his speech and motor activity. The state Medicaid program, however, refused to cover HBOT. The State based its decision on a finding that HBOT was not an acceptable standard of medical practice.

The court reversed and remanded. It held that the EPSDT statute required only that treatment be necessary to correct or ameliorate a condition.

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[21] 366 F. Supp. 2d 1050, 1117 (N.D. Okla. 2005).
[22] 969 P.2d 1 (Utah 1998).
[23] *Id.* at 3-4.
[24] *Id.* at 4.
[25]258 Ga. App. 446, 576 S.E. 2d 2 (Ct. App. 2002).

The "federal statute does not require that a treatment also be 'an acceptable standard of medical practice' to be eligible for reimbursement."[26] Thus, the Court found that the State had applied the wrong standard of proof:

> Instead of requiring proof that HBOT is the accepted standard medical practice, or that it meets the definition of medical necessity reserved for adult Medicaid recipients, the [Department] should have focused its inquiry on whether HBOT was necessary to correct or ameliorate [Freels'] physical condition.[27]

The Court then remanded the case for a determination of whether HBOT was necessary under the EPSDT standard.

This case is in tension with *Rush* and the cases following it. While it does not concern a decision that a service is experimental, the question of whether a particular treatment is an accepted medical practice is part of the *Rush* formulation and, therefore, widely used by states to determine whether a service is experimental. *Freels* suggests that the *Rush* formulation is impermissible in EPSDT cases. Unfortunately, as noted above, CMS has repeatedly stated that states do not have to cover experimental treatment. So, it is questionable how far this argument may be pushed.

States

Most states have medical necessity definitions imposing limitations on coverage of experimental services. Some, including California and Tennessee, also exclude "investigational" services.[28] Some, such as Delaware, refer to the Medicare definition of experimental.[29] The caselaw suggests that if these definitions conform with the *Rush* formulation, they are probably legal. *McLaughlin,* as well as due process principles, suggests that states should have a specific standard for determining whether a treatment is experimental.

Points to remember:

- The definition of what is experimental should be a flexible one, particularly for children and youth under age 21 – the *McLaughlin* factors should be a guide.
- Advocates should argue for significant deference to the treating provider when determining whether a particular treatment is

[26] *Id.* at 450.
[27] *Id. See also Jackson v. Millstone,*
[28] Cal. Code Regs. tit. 22, § 51303(g), (h); Tennessee Admin. Rules & Regs. 1200-13-13-.01(1)(f), Rule 1200-13-16-.05(6).
[29] Delaware Medicaid Provider Policy Manual – General Policy §1.15.

accepted or effective.[30]

- A service is not experimental simply because it is rarely performed.
- If a state says that a service is experimental, advocates should investigate this claim.
  - ✓ Discuss the issue with the treating provider
  - ✓ Review the medical literature
  - ✓ Determine whether other state Medicaid programs cover the procedure (particularly when dealing with children and youth)
  - ✓ Determine whether Medicare and/or private insurers cover the procedure

[30] *See* Sarah Somers, *Q & A: Medicaid and Deference to Treating Providers* (Nov. 2008), available on the TASC website at http://www.ndrn.org/TASC/pub/qa/2008/0812MedDef.pdf.