

1998

# Heidi Peterson on behalf of Markelle Frei-Peterson v. Utah Department of Health, Division of Health Care Financing : Brief of Appellant

Utah Court of Appeals

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IN THE UTAH COURT OF APPEALS

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HEIDI PETERSON on behalf  
of MARKELLE FREI-PETERSON

Petitioner/Appellant,

vs.

UTAH DEPARTMENT OF HEALTH,  
DIVISION OF HEALTH CARE  
FINANCING

Respondent/Appellee.

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Case No. 98-0078-CA

Category No. 14

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BRIEF OF APPELLANT

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This is a petition for review of a final agency order,  
entered January 15, 1998 by the Director of the Division of  
Health Care Financing, Utah Department of Health, adopting a  
recommended decision which denied appellant Medicaid coverage.

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UTAH COURT OF APPEALS  
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COURT OF APPEALS

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### **JURISDICTION OF THE COURT OF APPEALS**

This is a petition for review of a final agency order issued by the Director of the Division of Health Care Financing, Utah Department of Health, on January 15, 1998. The Court of Appeals has jurisdiction pursuant to Utah Code Annot. § 78-2a-3(2)(a).

### **STATEMENT OF THE ISSUE**

Whether treatment of short bowel syndrome through use of a growth hormone is experimental and, therefore, not covered by Medicaid.

### **STANDARD OF REVIEW**

The final agency order may be reversed, if Petitioner Markelle Frei-Peterson (hereinafter "Markelle") was substantially prejudiced by a determination of fact, made or implied by respondent Division of Health Care Financing (hereinafter "DHCF") that is not supported by substantial evidence when viewed in light of the whole record before the court. Utah Code Annot. § 63-46b-16(4)(g); King v. Industrial Comm'n, 850 P.2d 1281, 1285 (Utah App. 1993). Substantial evidence is defined as "that which a reasonable person might accept as adequate to support a conclusion." When reviewing the substantiality of the evidence, the reviewing court must consider both the evidence supporting the agency's findings and the evidence negating these findings. First National Bank of Boston v. County Bd. Of Equalization, 799 P.2d 1163, 1165 (Utah 1990).

The final agency order may also be overturned, if Peterson was substantially prejudiced by DHCF's erroneous interpretation or application of the law. Utah Code Annot. § 63-46b-16(4)(d). The correction of error standard applies to agency decisions involving the interpretation or application of general law and no deference is extended to such agency rulings. Zissi v. Tax Comm'n., 842 P.2d 848, 852-53 & n. 2 (Utah 1992); Savage Indus., v. Tax Comm'n., 811 P.2d 664, 669 (Utah 1991).

**DETERMINATIVE CONSTITUTIONAL PROVISIONS, STATUTES,  
ORDINANCES AND RULES**

1. 42 U.S.C. § 1396d(a)(4)(B);
2. 42 U.S.C. § 1396d(r)(5);
3. Utah Administrative Code R414-1A-200 & 300;
4. Utah Administrative Code R414-13x-1.(5)(a).

**STATEMENT OF THE CASE**

**A. Nature of the Case**

This is a petition for review of a final agency action by the Department of Health, Division of Health Care Financing, denying Medicaid coverage for a growth hormone to treat short bowel syndrome.

**B. Course of the Proceedings**

Peterson applied for Medicaid coverage of a growth hormone for treatment of her short bowel syndrome. When coverage was denied, an administrative hearing was requested and held December 8, 1997. The hearing was held pursuant to Rule R410-14 of the Utah Department of Health and the Utah Administrative Hearings

Act, Title 63, Chapter 46b, Utah Code Annotated, 1953, as amended. Record, at 111 (hereinafter "R"). On January 7, 1998, Administrative Law Judge (ALJ) Margaret Clark issued a recommended decision, recommending that DHCF's denial of coverage be upheld. ALJ Clark concluded use of the growth hormone to treat short bowel syndrome was experimental as defined in Utah Administrative Code R414-1A-200 and, therefore, not covered by Medicaid. On January 15, 1998, DHCF Director Michael Deily adopted the recommended decision in its entirety. This petition for review followed.

C. Disposition At The Agency

The agency issued a final agency action denying coverage of a growth hormone to treat Peterson's condition.

D. Relevant Facts With Citations to The Record

Markelle Frei-Peterson is a 24-month old child who suffers from short bowel syndrome. R-126-9. She is dependent on total parenteral nutrition support (TPN), a form of intravenous feeding, for ninety percent of her nutritional needs. R-126-9, 10. TPN is covered by Medicaid. R-126-12. Markelle's treating physician is Dr. William Daniel Jackson, M.D. who is board-certified in pediatrics, pediatric gastroenterology and nutrition. R-126-9. Dr. Jackson is employed as an assistant professor of pediatrics at the University of Utah School of Medicine. R-126-9. He is the Medical Director of Nutrition Support Services at Primary Children's Medical Center. R-126-10.

Dr. Jackson wants to get Markelle off TPN so that her

gastrointestinal tract can adapt and accommodate enteral nutrition. R-126-11. He is also concerned about central line catheter infections and loss of I.V. access sites. R-126-11. His most serious concern is that Markelle will develop progressive liver dysfunction. R-126-11. Dr. Jackson testified that patients with short bowel syndrome who are parenterally nutrition dependent "have a high risk of progressing to liver failure and require liver transplantation." R-126-12, 24. The doctor mentioned another patient with a condition similar to Markelle's who "ended up getting a liver and small bowel transplant." R-126-24. Others, he noted, had gotten liver transplants, while others not eligible for a transplant, simply died. R-126-24-25. Liver transplants are covered by Medicaid. R-126-12.

Dr. Jackson has recommended that Markelle's short bowel syndrome be treated by use of a recombinant human growth hormone, called "humatrope". R-126-13-14. He testified the growth hormone stimulates "transcription of certain regulatory genes that turn on growth of the lining of the intestine." R-126-15. The growth hormone is used, "in the lines of a pharmacologic agent to actually stimulate the kind of growth factors that would be required to try to make the small bowel develop and grow more." R-126-43.

Markelle had been treated for several months with a growth hormone, financed by Primary Childrens Hospital, and there is circumstantial evidence that it has helped her, since she has

been able to take some nutrition orally. R-126-17-18, 44. Dr. Jackson recommended a further trial up to one year. R-126-19-20. Markelle's mother, Heidi Peterson (erroneously referred to as Markelle's aunt in the hearing transcript) testified that Markelle's nutritional intake had improved since the start of using a growth hormone. R-126-21.

Medicaid covers the use of growth hormones to treat short-statured persons but will not pay for its use to treat short bowel syndrome, which it considers an "off-label use." R-126-13. Dr. Jackson testified there is a higher indication for use of the growth hormone in Markelle's case, since he is seeking approval of a treatment that may save her life. R-126-13. He further testified that the use of a growth hormone over a reasonably short course, "if it worked would be a cost effective approach in terms of the huge magnitude of the cost of lifetime TPN and/or liver transplantation." R-126-14. When asked by the ALJ to address the medical necessity of the proposed treatment, Dr. Jackson testified:

the goal would be to basically prevent premature death, reduce -- or improve quality of life by reducing dependence on total parenteral nutrition, and increasing her chances of becoming independent of that by being able to eat and consume foods normally.

Avoid the future suffering of -- well, future and things that she's already encountered of central line infections, complications of central venous catheters to provide the total parenteral nutrition. Including infection and dislodgment and vascular thrombosis. And finally to prevent chronic liver disease that plagues so many patients that are on chronic TPN, with the possibility of requirement for

a liver transplantation.

R-126-23. Dr. Jackson testified there is no other equally effective course of treatment that would be more conservative or substantially less costly. R-126-24.

The use of growth hormone to treat short bowel syndrome is supported, Dr. Jackson testified, by a number of mainstream practitioners. R-126-15. His urgency in requesting approval of the treatment arose from his concern that without it, Markelle's condition would deteriorate; he also expressed his judgment that in terms of background, training and colleagues consulted, the benefits from the proposed therapy outweighed the risks and costs. R-126-26. Dr. Jackson described the use of growth hormone for treatment of short bowel syndrome as more a matter of medical judgment than an experimental procedure. R-126-35-36.

Dr. Jackson testified that it was common in pediatric practice to use drug therapies that have not been proved or verified. R-126-37. He described this as "a fact of life" since "society has not deemed it necessary to study children in that regard." R-126-37. He concluded:

So what's happened to pediatricians is they have to take those drugs that aren't approved for pediatrics and use them. And there's a very, very long tradition of doing that, both for good and for bad for children.

R-126-38.

The request for approval of the growth hormone was denied by a utilization review committee made up of physicians, nurses and a social worker. T-48. The rationale given for the denial was

that "the documentation indicated it was an experimental procedure." T-49. According to one witness, growth hormone is only allowed under the drug criteria manual for two groups, both of them having to do with growth failure. R-126-63.

#### **SUMMARY OF THE ARGUMENT**

A number of errors were committed by the ALJ in deciding Markelle's case. The ALJ applied the wrong burden of proof and failed to give appropriate weight to the opinions of the treating physician. The conclusion that the growth hormone cannot be approved for Medicaid coverage is based on a definition of "experimental" which is too restrictive under the circumstances. While a Medicaid agency has broad discretion in deciding what treatment it will cover, its discretion is not unlimited. The agency may not apply unreasonable policies in denying medical treatment.

#### **ARGUMENT**

##### **A. Overview and Purposes of the Medicaid Program**

The Medicaid program was established by Congress in 1965 as Title XIX of the Social Security Act, "for purposes of providing federal assistance to States that choose to reimburse certain costs of medical treatment for needy persons." Harris v. McRae, 448 U.S. 297, 301, 100 S.Ct. 2671, 65 L.Ed. 2d 784 (1980). Medicaid is a joint federal-state program designed to meet some of the medical needs of low-income persons. 42 U.S.C. § 1396 (1988); Schweiker v. Hogan, 457 U.S. 569, 571 (1982). States are not required to participate in the Medicaid program; however,

once they choose to do, they must comply with the Medicaid statute and implementing regulations. Schweiker v. Gray Panthers, 453 U.S. 34, 37 (1981). A strong incentive for state participation is federal funding of a substantial portion of all Medicaid costs. In Utah, approximately seventy percent of the Medicaid budget is supplied by the federal participant.

**B. The Agency Erred In Denying Approval of Growth Hormone Treatment As Being Experimental**

Medical Necessity

The Medicaid statute is clear that a participating state must provide medically necessary services. While a state Medicaid agency has discretion in deciding what services it may provide, that discretion is circumscribed by the federal statute which provides, in part:

A State plan for medical assistance must ... include reasonable standards ... for determining eligibility for and the extent of medical assistance under the plan which ... are consistent with the objectives of this [Title]....

42 U.S.C. § 1396a(a)(17)(1998 Supp.) The Supreme Court has opined that a "serious statutory question might be presented if a state Medicaid plan excluded *necessary medical treatment* from its coverage." Beal v. Doe, 432 U.S. 438, 444, 97 S.Ct. 2366, 53 L.Ed.2d 464 (1977).

Children receive additional protection against unreasonable denial of necessary medical treatment. In order for the Utah Medicaid plan to be approved by the federal government, it must provide certain mandatory medical services. 42 U.S.C. §

1396d(a)(10)(A). Included among the mandatory services are "early and periodic screening, diagnostic and treatment services" (EPSDT services) for persons under age 21. 42 U.S.C. § 1396d(a)(4)(B). EPSDT services include screening, vision, dental and hearing services as well as "[s]uch other necessary ... treatment ... to correct or ameliorate ... conditions discovered by the screening services...." 42 U.S.C. § 1396d(r)(5).

#### Unproven or Experimental Medical Practices

A state Medicaid agency may adopt reasonable standards for determining medical necessity which exclude coverage of experimental treatments. Rush v. Parham, 625 F.2d 1150, 1156 (5th Cir. 1980). Utah has done this through its administrative rules at R414-1A-300 which provide, in part:

- (1) Experimental or unproven medical practices are not covered Medicaid services.
- (2) Division staff and physician consultants shall establish criteria to determine whether a service or procedure is a covered Medicaid service.
- (3) Procedures or services proven to be medically efficacious for specific medical conditions may be provided as covered Medicaid services only for the conditions specified. Such procedures or services are not covered for any other conditions or for experimental trials.

R414-1A-300. The rules define "experimental or unproven medical practice" and "medically efficacious" as follows:

- (a) "experimental or unproven medical practice means any procedure, medication product, or service that is:
  - (i) not proven to be medically efficacious for a given procedure; or

(ii) performed for or in support of purposes of research, experimentation, or testing of new processes or products; or  
(iii) both

(b) "medically efficacious" means a medical practice that:

(i) has been determined effective and is widely utilized as a standard medical practice for specific conditions; and  
(ii) has been approved as a covered Medicaid service by division staff and physician consultants on the basis of medical necessity, as defined in R414-13x-1.(5)(a) and in accordance with R414-26-1.(2)(f)...

R414-1A-200. "Medical necessity" has been defined as follows:

a. A provider must furnish or prescribe medical services to the recipient only when, and to the extent that it is medically necessary. A service is "medically necessary" if it is (1) reasonably calculated to prevent, diagnose, or cure conditions in the recipient that endanger life, cause suffering or pain, cause physical deformity or malfunction, or threaten to cause a handicap; and (2) there is no other equally effective course of treatment available or suitable for the recipient requesting the service which is more conservative or substantially less costly. Medical services shall be of a quality that meets professionally recognized standards of health care, and shall be substantiated by records including evidence of medical necessity and quality.

R414-13x-1.(5)(a).<sup>1</sup>

#### Findings of Fact and Conclusions of Law

In her decision affirming the denial of growth hormone, ALJ Clark made the following findings of fact:

#### FINDINGS OF FACT

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<sup>1</sup>Rule R414-26-1.(2)(f) pertains to DHCF policy development process and is not pertinent to this discussion.

1. Markelle Frei-Peterson is approximately twenty four months old, suffers from short bowel syndrome, and is dependent on parenteral nutrition for approximately 90% of her nutritional needs.

2. In the past three or four months she has begun to tolerate some oral feedings, but remains dependent on parenteral nutrition.

3. Short bowel syndrome requires expensive technology and possibly a lifetime of parenteral nutrition.

4. The goal of Markelle's treating physician is to accelerate her gastrointestinal adaption by administering the growth hormone for one year.

5. Because she has short bowel syndrome, Markelle is at risk of central line catheter infections, eventual loss of intravenous access sites, and progressive liver dysfunction.

6. Markelle has been receiving growth hormone for approximately three months. The hormone has been supplied by a Primary Children's Hospital charity.

7. Usage of growth hormone for short bowel syndrome is considered an "off label" use by the Federal Food and Drug Administration.

8. The data is sufficient to motivate a number of reputable physicians to prescribe growth hormone for short bowel syndrome, but it is still considered to be controversial.

R-112. Based on these findings, the ALJ made one recommended conclusion of law:

The use of growth hormone to treat small bowel syndrome is "experimental" as defined in: Utah Administrative Code R414-1A-200, and is therefore not covered by Utah Medicaid [see R414-1A-300(1)].

R-112. Petitioner does not disagree with the findings of fact entered in the case; however, they do not direct the conclusion

of law, when the correct legal standards are applied.

**C. The ALJ Applied An Incorrect Burden of Proof**

In discussing the reasons for the conclusion of law, the ALJ stated:

As the expert witness for the moving party, the burden of proof was on Dr. Jackson to prove by the preponderance of the evidence that the growth hormone should be covered. Despite his convincing testimony regarding the medical necessity of using the drug for Markelle, he was not able to overcome DHCF's evidence that the use of growth hormone to treat short bowel syndrome is an off-label usage of the drug, and it has not yet been proven to be effective for that usage. Although treatment of short bowel syndrome with growth hormone might be more highly indicated than its usage for children of short stature, the law prohibits the use of experimental treatments, and Dr. Jackson's testimony clearly indicated that the use of growth hormone to treat short bowel syndrome was not "widely utilized as a standard medical practice," and therefore meets the criteria for an "experimental procedure."

R-113-14.

The ALJ's conclusion of law is based on the erroneous assumption that Dr. Jackson had the burden of proving by a preponderance of the evidence that the recommended treatment was not experimental. While it is harmless error to say that a witness, rather than the petitioner, had the burden of proof, it is not harmless error to apply the wrong burden of proof. Administrative agency action based on factual findings must be overturned if they are not supported by "substantial evidence." King v. Industrial Comm'n, 850 P.2d at 1285. Substantial evidence has been described as more than a mere "scintilla" of evidence

and something less than the weight of the evidence. Johnson v. Board of Review, 842 P.2d 910, 911 (Utah App. 1992). Substantial evidence is such relevant evidence as reasonable minds might accept as adequate to support a conclusion. Grace Drilling Co. V. Board of Review, 776 P.2d 63, 68 (Utah App. 1989). Of particular significance for this case is the rule that "'Evidence is not substantial "if it is overwhelmed by other evidence-- particularly certain types of evidence (e.g., that offered by treating physicians) or if it really constitutes not evidence but mere conclusion."'" Frey v. Bowen, 816 F.2d 508, 512 (10th Cir. 1987) as cited in, A.M.L. v. Department of Health, 863 P.2d 44, 47 (Utah App. 1993).

**D. The ALJ's Decision Is Not Supported By Substantial Evidence**

The ALJ's conclusion ignores the fact that Dr. Jackson is Markelle's treating physician, thereby entitling his opinion to greater weight than that of other medical consultants. A.M.L. v. Department of Health, 863 P.2d at 48. In A.M.L., this court thoroughly reviewed the treating physician rule as it applies in Medicaid cases and opined:

Further we note that several courts require state Medicaid agencies to recognize a presumption `in favor of the medical judgment of the attending physician in determining the medical necessity of treatment.' (Citations omitted).

....

In accordance, if DHCF elects not to give deference to the testimony given by the treating physician, the agency should `provide a reasoned basis for declining to do so which is consistent with the purposes of

the Medicaid Act.' *Worthington*, slip op. at 7; see also *Frey*, 816 F.2d at 513 ('If the opinion of the claimant's physician is to be disregarded, specific, legitimate reasons for this action must be set forth.')

A.M.L. v. Department of Health, 863 P.2d at 48. Other cases holding that the treating physician's opinion is entitled to greater weight include: Montoya v. Johnston, 654 F.Supp. 511, 513 (W.D. Tex. 1987) ("The best indicator for determining the medical appropriateness of treatment rests with a patient's physician.") and Weaver v. Reagen, 886 F.2d 194, 200 (8th Cir. 1989) ("The Medicaid statute and regulatory scheme create a presumption in favor of the medical judgment of the attending physician in determining the medical necessity of treatment.").

It appears from the ALJ's decision that the medical necessity of growth hormone treatment was proven. The decision refers to "convincing testimony regarding the medical necessity of using the drug for Markelle..." R-113. If a treatment is medically necessary, then it should be approved for coverage. However, the ALJ then retreats from this conclusion by saying, "he [Dr. Jackson] was not able to overcome DHCF's evidence that the use of growth hormone to treat short bowel syndrome is an off-label usage of the drug, and it has not yet been proven to be effective for that usage." R-114.

It is erroneous to conclude that a drug is not medically necessary, because it is off-label. In Weaver v. Reagen, 886 F.2d 194 (8th Cir. 1989), the court considered whether the Missouri Medicaid agency's policy of denying coverage for using

the drug AZT outside the indications published by the Food and Drug Administration was reasonable. In finding that it was not reasonable, the court opined:

Thus, the 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 of Missouri 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.

Weaver v. Reagen, 886 F.2d at 198.

The ALJ is incorrect in saying that DHCF introduced evidence that the growth hormone had not been proven effective for the treatment of short bowel syndrome. The only evidence offered by DHCF was brief testimony by Dr. John Hylen, who is not an expert in the area. He testified briefly as to his concerns, which included approval of an off-label use, toxicities and expense. T-126-60. In light of Dr. Hylen's limited expertise and the vagueness of his testimony, it cannot be said that DHCF offered substantial evidence on this point.

In contrast, substantial evidence of the growth hormone's effectiveness in limited trials was offered by petitioner. Dr. Jackson acknowledged that the proposed use of the growth hormone was off-label, as determined by DHCF; however, that acknowledgment does not detract from the treating physician's opinion that its use would be effective in treating his patient. He testified:

[T]here are a number of other physicians around the country, many much smarter than I am, including Dr. Book who's a division chief, who feel that in certain situations using growth hormone which is commonly used in many, many children for the indications of just somatic growth, it's not going to threaten their life, it doesn't threaten much of anything except for their -- it threatens self esteem, et cetera. But growth hormone for short-stature patients, that is covered by Medicaid as an on-label use.

In this case we're using it off that label but what I think is a higher indication which is -- and obviously has to do with judgment and making a guess of probabilities, but for saving someone's life.

R-126-13. The treating physician testified further:

However, the relationship between the patient and this physician at least in terms of background, training, and the colleagues that I've consulted and worked with has been -- have basically led me to advocate the use of this therapy. And at least in my judgement and tying together the benefits outweigh the risks and the costs.

R-126-26.

**E. The ALJ Applied An Overly Restrictive Definition of "Experimental"**

The ALJ further based her conclusion that the drug was experimental on her assessment that "Dr. Jackson's testimony clearly indicated that the use of growth hormone to treat short bowel syndrome was not `widely utilized as a standard medical practice.'" A careful review of the record shows this is not a fair summary of Dr. Jackson's testimony. While acknowledging that the treatment might under some circumstances be considered experimental, Dr. Jackson did not consider it so in this case. Again, he testified:

[T]he strict interpretation of experimental in my mind would be is she on an experimental protocol of like one patient in a study. And I guess I don't consider it that. I consider it more of a situation of taking a therapy that's been -- that is in use, that is new, that is not 100 percent validated and may very well have certain subjects, certain patients in which it works in and certain patients that it doesn't. ...

I don't know whether you call it the art of medicine but it's the idea of taking data, what data is available, and a desperate clinical situation and adding up pluses and minuses and trying to come to a judgment. And that judgment could be deemed erroneous by peers or other people, but on the other hand there are a number of different peers and superiors to me who agree with trying this kind of therapy, so they have been my guide.

The standard "widely utilized as a standard medical practice" does not automatically direct the conclusion that growth hormone treatment for short bowel syndrome is experimental. As the court in Miller by Miller v. Whitburn, 10 F.3d 1315, 1320 (7th Cir. 1993) noted:

Clearly, the best indicator that a procedure is experimental is its rejection by the professional medical community as an unproven treatment. The quoted passage<sup>2</sup> suggests,

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<sup>2</sup> The court in Miller by Miller v. Whitburn, and many other cases discussing alleged experimental treatments, frequently cite the following quotation from Rush v. Parham:

"The clearest articulation of the considerations that go into determining whether a particular service is experimental is found in a letter Medicare uses to explain to its clients and providers why a service is ineligible for reimbursement:

`In making such a decision [whether to provide payment for a particular service], a basic consideration is whether the service has come to be generally accepted by the professional medical community as an effective and proven treatment for the

however, that different definitions of 'experimental' may be necessary depending upon the notoriety of the treatment under review. Indeed, certain procedures may be so new and, as a result, relatively unknown, that the medical community may not yet have formed an opinion as to their efficacy. We agree with the court in *Rush* that such procedures are not *per se* experimental. If 'authoritative evidence' exists that attests to a procedure's safety and effectiveness, it is not 'experimental.'

A number of courts have followed the lead of Rush v. Parham and have found Medicaid agency definitions of experimental to be defective. In Weaver v. Reagan, the court found the Missouri Medicaid agency's definition of "experimental," which limited the use of AZT to those cases meeting published FDA indications, to be overly broad. The Missouri agency defined experimental as:

a treatment not 'generally accepted by the professional medical community as an effective and proven treatment for the condition' or 'rarely used, novel or relatively unknown.'

Weaver v. Reagan, 886 F.2d at 198. Relying on the definition in Rush, the court found Missouri's definition to be "unreasonable" in light of the widespread recognition by the medical community and the scientific literature that AZT was an effective drug. Similarly, in Montoya v. Johnston, 654 F.Supp. 511 (W.D. Tex. 1987), the court, after considering affidavits from physicians saying liver transplants were not experimental, held that the

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condition for which it is being used. If it is, Medicare may make payment. On the other hand, if the service is rarely used, novel or relatively unknown, then authoritative evidence must be obtained that it is safe and effective before Medicaid may make payment.'" *Rush v. Parham*, 625 F.2d 1150, 1156 (5th Cir. 1980).

treatment was medically necessary.

The difficulty presented by a policy which denies coverage of a treatment because it is not "widely utilized as a medical practice" was taken up in McLaughlin v. Williams, 801 F.Supp. 633 (S.D. Fla. 1992), where the court reviewed Florida's Medicaid policy of denying coverage for liver-bowel transplants as being experimental. One of the shortcomings of DHCF's definition of experimental in this case is that it does not demarcate how widely used a practice must be in order to no longer be experimental. The McLaughlin court focused on this same weakness in Florida's policy, which it summarized as follows:

The Defendant's contention, stated simply, is that as a matter of law, until a given amount of time has passed, and until the new procedure is accepted generally, the procedure must be deemed experimental, and the state need not pay. 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.

McLaughlin v. Williams, 801 F.Supp. At 639. In addressing Florida's argument that a fixed period of time must pass before a practice is generally accepted in the medical community and, therefore, not experimental, the court followed the suggestion in Rush that such cases can be found medically necessary if "authoritative evidence" shows the treatment to be "safe and effective." McLaughlin v. Williams, 801 F.Supp. at 639. The court then went on to suggest several factors which should be considered in determining the effectiveness of a treatment, including: patient mortality; frequency, success or failure of

the treatment; the reputations of the doctors and facilities performing the treatment; long term prognoses of other patients receiving the treatment and the extent to which medical science in the area has developed rapidly. McLaughlin v. Williams, 801 F.Supp. at 639.

The truly authoritative evidence in this case is the testimony of Dr. Jackson, together with extensive medical literature submitted at the hearing, establishing that the use of a growth hormone to treat short bowel syndrome is safe and effective. Dr. Jackson's testimony on these points can be summarized as follows:

1. There is a higher indication for using the growth hormone to possibly save Markelle's life than for using the drug to treat persons of low stature R-126-13;
2. It would be more cost effective, since the alternative is possible lifetime TPN or a liver transplant R-126-14;
3. There are studies by a number of "mainstream" professionals that support use of the growth hormone R-126-15;
4. Short term, three-week studies, have shown some increase in absorption and amino acid uptake R-126-16;
5. Markelle's ability to take solid foods appeared to have improved while she was taking the growth hormone R-126-18;
6. Use of the growth hormone is reasonably calculated to prevent premature death and improve quality of life by reducing dependence on TPN R-126-23;
7. The growth hormone would lessen future suffering from catheter line infections, dislodgment and vascular thrombosis R-126-23;

8. There is no good alternative treatment that would be more cost effective R-126-24.

A sampling of the medical literature submitted by Dr. Jackson shows further support for the conclusion that growth hormone treatment for short bowel syndrome is safe and effective:

1. D. Wilmore, "Short Bowel Rehabilitation Program: A Unique Approach Including Glutamine, Growth Hormone and Special Diet"-- 12 children treated showed enhanced growth velocity and improved absorption... R-17;

2. Theresa A. Byrne, Dsc, RD, CNSD, et. al., "A New Treatment Option for Patients with Short Bowel Syndrome..." Support Line, Feb. 1996--"These data suggest that treatment with GH+GLN+Diet offers an effective alternative to long-term TPN for some patients with severe SBS." R-25;

3. Theresa A. Byrne, et. al. "A New Treatment for Patients with Short-Bowel Syndrome..." Annals of Surgery, Sept. 1995-- "The initial balance studies indicated improvement in absorption of protein by 39% accompanied by a 33% decrease in stool output with the GH+GLN+DIET. In the long-term study, 40% of the group remained off TPN and an additional 40% have reduced their TPN requirements, with follow-up averaging a year and the longest being over 5 years." R-28.

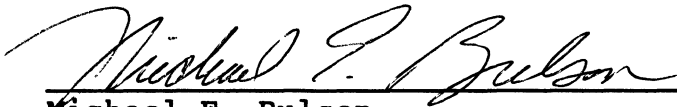
The evidence from Dr. Jackson and the medical literature is both authoritative and substantial. Dr. Jackson's opinions as Markelle's treating physician are entitled to great weight. Further, Dr. Jackson has the reputation of the University Medical Center and Primary Childrens Hospital behind him. Since this is an area in which the science seems to be developing rapidly, Dr. Jackson's opinions are especially relevant. DHCF has not offered evidence by a comparable expert which would overcome Dr. Jackson's testimony, nor has the agency given any specific,


legitimate reasons for not giving controlling weight to Dr. Jackson's testimony.

#### CONCLUSION

For the reasons discussed herein, the final agency decision, dated January 15, 1998, should be reversed and DHCF ordered to provide Medicaid coverage for the growth hormone treatment.

DATED this 12<sup>th</sup> day of May, 1998.

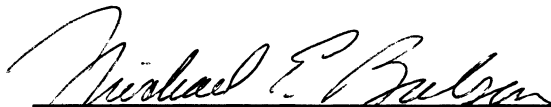
  
Michael E. Bulson  
Attorney for Petitioner/Appellant

  
W. Paul Wharton  
Attorney for Petitioner/Appellant

#### CERTIFICATE OF SERVICE

The undersigned hereby certifies that two true and correct copies of the foregoing **Brief of Appellant** were mailed this 12 day of May, 1998, via first class mail, postage prepaid, to the following:

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## **ADDENDUM**



DIVISION OF HEALTH  
CARE FINANCING

# State of Utah

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MARKELLE FREI-PETERSON

Petitioner

vs.

UTAH DEPARTMENT OF HEALTH

DIVISION OF HEALTH CARE FINANCING,

Respondent.

FINAL AGENCY ORDER

Case No. 97-209-11

IF YOU ARE NOT SATISFIED WITH THIS DECISION, YOU MAY REQUEST A RECONSIDERATION FROM THE DIRECTOR OF HEALTH CARE FINANCING WITHIN TWENTY (20) DAYS AFTER THIS DECISION IS SIGNED. IF YOU WOULD LIKE TO APPEAL THIS DECISION, YOU MAY FILE A PETITION IN THE UTAH COURT OF APPEALS WITHIN THIRTY (30) DAYS AFTER THIS DECISION IS SIGNED. IF YOU DECIDE TO APPEAL, YOU ARE NOT REQUIRED TO ASK FOR A RECONSIDERATION FIRST, BUT YOU MAY DO SO IF YOU WISH. IF YOU HAVE QUESTIONS, CALL (801) 538-6576.

The enclosed Recommended Decision has been reviewed pursuant to Section 63-46b-12 Utah Code Ann. 1953, as amended, entitled "Agency Review - Procedure," and Department of Health Administrative Rule R410-14, entitled "Division of Health Care Financing Administrative Hearing Procedures for Medicaid/UMAP Applicants, Recipients, and Providers."

I hereby adopt Recommended Decision No. 97-209-11 in its entirety.

## RIGHT TO JUDICIAL REVIEW

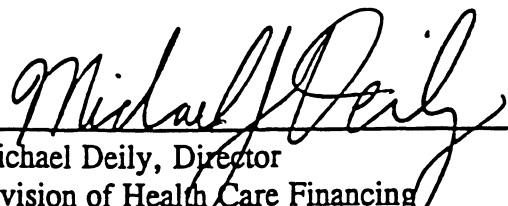
Within twenty (20) days after the date that this Final Agency Order is issued, you may file a written request for reconsideration with the Director of the Division of Health Care Financing. Any request for reconsideration must state the specific grounds upon which relief is requested. The filing of such a request is not a prerequisite for seeking judicial review.

Judicial review may be secured by filing a petition in the Utah Court of Appeals within thirty (30) days of the issuance of this Final Agency Action or, if a request for reconsideration is

filed and denied, within thirty (30) days of the denial for reconsideration. The petition shall be served upon the Director of Health Care Financing and shall state the specific grounds upon which review is sought. Failure to file such a petition within the 30-day time limit may constitute a waiver of any right to appeal the Final Agency Order.

A copy of this Final Agency Order shall be sent to Petitioner or representative at the last known address by certified mail, return receipt requested.

DATED this 15 day of January 1998

BY:   
Michael Deily, Director  
Division of Health Care Financing  
UTAH DEPARTMENT OF HEALTH

BEFORE THE UTAH DEPARTMENT OF HEALTH

DIVISION OF HEALTH CARE FINANCING

STATE OF UTAH

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MARKELLE FREI-PETERSON :  
Petitioner, :  
vs. : RECOMMENDED DECISION  
UTAH DEPARTMENT OF HEALTH :  
DIVISION OF HEALTH CARE : Case No. 97-209-11  
FINANCING, : Margaret J. Clark  
Respondent. : Administrative Law Judge

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Pursuant to Rule R410-14 of the Utah Department of Health and the Utah Administrative Hearing Procedures Act, Title 63, Chapter 46b, Utah Code Annotated, 1953, as amended, a formal administrative hearing for the above captioned case was held on December 8, 1997, at 8:00 a.m., in Room 344, Cannon Health Building, 288 North 1460 West, Salt Lake City, Utah 84116, Margaret J. Clark, Administrative Law Judge, presiding. Daniel Jackson, M.D., testified on behalf of the petitioner. The petitioner's mother was present at the hearing. Steven Gatzemeier represented the Division of Health Care Financing ("DHCF"). John C. Hylen, M.D., and Duane Parke testified on behalf of DHCF.

ISSUE

SHOULD UTAH MEDICAID COVER GROWTH HORMONE TO TREAT SHORT BOWEL SYNDROME FOR MARKELLE FREI-PETERSON?

## FINDINGS OF FACT

1. Markelle Frei-Peterson is approximately twenty four months old, suffers from short bowel syndrome, and is dependent on parenteral nutrition for approximately 90% of her nutritional needs.
2. In the past three or four months she has begun to tolerate some oral feedings, but remains dependent on parenteral nutrition.
3. Short bowel syndrome requires expensive technology and possibly a lifetime of parenteral nutrition.
4. The goal of Markelle's treating physician is to accelerate her gastrointestinal adaptation by administering the growth hormone for about one year.
5. Because she has short bowel syndrome, Markelle is at risk of central line catheter infections, eventual loss of intravenous access sites, and progressive liver dysfunction.
6. Markelle has been receiving growth hormone for approximately three months. The hormone has been supplied by a Primary Children's Hospital charity.
7. Usage of growth hormone for short bowel syndrome is considered an "off-label" use by the Federal Food and Drug Administration.
8. The data is sufficient to motivate a number of reputable physicians to prescribe growth hormone for short bowel syndrome, but it is still considered to be controversial.

## RECOMMENDED CONCLUSIONS OF LAW

The use of growth hormone to treat small bowel syndrome is "experimental" as defined in: Utah Administrative Code R414-1A-200, and is therefore not covered by Utah Medicaid [see R414-1A-300(1)].

## REASONS FOR PRESIDING OFFICER'S DECISION

DHCF denied reimbursement for growth hormone for Markelle because it contended that the drug is experimental for usage in treating short bowel syndrome, and it has not been approved by the Federal Drug Administration for that purpose.

DHCF's policy regarding experimental or unproven medical practices is contained in Utah Administrative Code R410A. R410A-300 states in relevant part:

- (1) Experimental or unproven medical practices are not covered Medicaid

services.

R410A-200 defines "experimental or unproven medical practice" as "(2)(i) not proven to be medically efficacious for a given procedure." "Medically efficacious" is defined in R410-1A-200(iii)(b) as "a medical practice that has been determined effective and is widely utilized as a standard medical practice for specific conditions."

W. Daniel Jackson, M.D., Markelle's treating physician testified on her behalf. Dr. Jackson is board certified in pediatrics and pediatric gastroenterology and nutrition. He is an Assistant Professor of Pediatrics at the University of Utah School of Medicine, and Medical Director of Nutrition Support Services at Primary Children's Hospital.

Dr. Jackson testified that growth hormone for short bowel syndrome is being used by a number of reputable physicians in the United States. He testified that it was controversial, but it had promise.

Dr. Jackson testified that growth hormone is commonly used in many children to treat short stature, and since Medicaid covers the drug for that use, it should also cover its usage for short bowel syndrome, which he believes has a "higher indication."

Dr. Jackson made compelling arguments for the medical necessity of using growth hormone for Markelle. He testified that he thought that use of growth hormone in this case would be a cost effective approach when compared to the potential cost of lifetime parental nutrition or liver transplantation, both of which could result from small bowel syndrome. He testified that more conservative approaches to treat Markelle were not successful, and as her treating physician, he had weighted the pluses and minuses of using the growth hormone.

Dr. Jackson testified that the impetus for trying the growth hormone was the fact that Markelle was showing signs of accelerated liver disease. Upon cross examination, John C. Hylen, M.D., and Physician Consultant for DHCF asked Dr. Jackson if he could provide documentation of whether or not Markelle's liver function had normalized as a result of receiving the growth hormone. Dr. Jackson replied that he did not know why her liver functions had improved, but he thought that it had normalized "independent of growth hormone." He testified that Markelle had improved after receiving growth hormone, but that improvement could also have come from the maturation process and the oral feedings Markelle has recently begun to tolerate.

Dr. Jackson conceded that the use of growth hormone for short bowel syndrome is an area where there is active work and controversy, and, "The indications are not in your code for using it this way."

As the expert witness for the moving party, the burden of proof was on Dr. Jackson to prove by the preponderance of the evidence that the growth hormone should be covered. Despite his convincing testimony regarding the medical necessity of using the drug for Markelle, he was not able to overcome DHCF's evidence that the use of growth hormone to treat short bowel

syndrome is an off-label usage of the drug, and it has not yet been proven to be effective for that usage. Although treatment of short bowel syndrome with growth hormone might be more highly indicated than its usage for children of short stature, the law prohibits the use of experimental treatments, and Dr. Jackson's testimony clearly indicated that the use of growth hormone to treat short bowel syndrome was not "widely utilized as a standard medical practice," and therefore meets the criteria for an "experimental procedure."


#### RECOMMENDED AGENCY ACTION

I recommend that DHCF's action be UPHELD.

#### RIGHT TO REVIEW

This Recommended Decision will be automatically reviewed by the Department of Health, Division of Health Care Financing, prior to its release. Both the Recommended Decision and a Final Agency Action, which represent the results of that review, will be released simultaneously by the Department of Health, Division of Health Care Financing.

DATED this 7 day of January 1998

  
Margaret J. Clark  
Administrative Law Judge

**The following exhibits were admitted into evidence:**

**RESPONDENT'S EXHIBIT 1: Off-Label Drug Policy**

**PETITIONER'S EXHIBIT 1: Medical Literature Regarding Short Bowel Syndrome and Growth Hormone**

**PETITIONER'S EXHIBIT 2: Billing Records for Markelle**

No: 97-209-11

CERTIFICATE OF MAILING

I hereby certify that on the 15 day of January 1998, I mailed a true and correct copy of the foregoing FINAL AGENCY ORDER AND RECOMMENDED DECISION, to the following parties:

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MICHAEL DEILY, DIRECTOR  
DIVISION OF HEALTH CARE FINANCING  
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CHRIS SMITH