

Medical Foods Policy

Voice of the Patient on State Response to Unmet Needs

THE LIFEBLOOD FOR INBORN ERRORS IS A GHOST TOWN — JENNIFER PAYNE

Although recent issue of NORD's 2015 State Policy Report Card shows some progress to assure access to medical foods consumers living with rare, inborn errors of metabolism, the inconsistency and arbitrary interpretations of the Orphan Drug Act Amendment (otherwise known as the federal definition of medical food) remain a public outrage worthy of attention and scrutiny by some of the nation's most vulnerable citizenry. Even in the era of the Affordable Care Act, many states and insurance regulators pose threats to public health in usurping a patient voice and eliminating the one and only treatment choice – medical food – which has saved countless lives for over 6 decades and given birth to the most successful, preventative public health policy ever - known as the institution of newborn screening. There is much room for improvement to deal with the machinations of the government and such unlimited discretion in application of potentially transformative, health care initiatives at both the state and federal level. The checkered pattern of coverage as reflected in NORD's State Policy Report Card is not only a reflection of discriminatory provision of lifesaving treatment, but state endorsement for the oppressive regime enshrined in federal statute should awaken the nation to action.

While the federal definition as amended to the Orphan Drug Act may be perceived as heroic, repeated patterns of contradictory governmental behaviors and lack of alignment in public health priorities and civil duties have proven to be a disaster. The lack of standards in both the government and private sector (especially with self-insured corporate entities) affords incentive and profit to big industry players, caters to less than altruistic motives at the expense of public health, and violates the spirit for which the Orphan Drug Act was originally passed. Patients living with rare, genetic diseases like me are left to navigate a state policy environment rife with loopholes in medical foods coverage that result in the discriminatory provision of costly treatment based upon age, gender, state of residence, employer, and any combination of the above.

Although my home state of Maryland scored an "A" in national overview of key policy arenas, I think the poor uptake and payer reluctance as reflected in research findings of NORD's State Policy Report Card serve as a graphic reminder to address poor outcomes and the need to open the dialogue on changing medical foods policy. While Maryland leadership should be commended for setting a precedent in duty to respond to those 1) diagnosed by newborn screening, and 2) dependent upon affordable,

equitable medical foods coverage for survival, it behooves the federal government to follow suit of the states that serve as a model for excellence as opposed to leaving federal employees like myself subject to abuse of institutional controls and profit-driven policies dictated by self-insured corporations. My family and I have had to fend on our own since the inception of the category of medical foods and deal with the consequences of 1972 landmark policy decision making that permanently banned the category of legitimate and beneficial medical foods from the nation's health care system and further marginalized the greater community touched by inborn errors who require treatment with medical foods.

In the decades that have followed FDA reclassification of what was once a prescription drug, the culmination of government inadvertence and reluctance to change policy on medical foods is analogous to life in a ghost town. It is my position of that top leadership officials (Congress and FDA) having simply "checked out" of the lifeblood for inborn errors. And, this is reflected in both policy and policy enforcement posture and the apportioning of responsibility under poor faculty of choice that has perpetuated risk and compromised patient access and efficiency. The findings of NORD reinforce the need to stop allowing this inappropriate, predatory behavior that specifically targets a rare, minority population and rewards private interest groups. My efforts have focused in addressing system responsibilities to prevent adverse outcomes and invoke professionalism in the practice of pharmacy. Success in having introduced a first-ever historic right and breakthrough in pharmacy benefits administration was achieved on medical foods coverage effective January 2015. The implementation of a 2015 BCBS service benefit plan marked an evolutionary milestone – which provided timely and affordable access to the latest 21st century orphan innovation in the category of medical foods used in the treatment of PKU. Paradoxically, in the backdrop of PKU Awareness Month (national observance commemorated in May), the federal government reversed their decision and rescinded my coverage on medical foods, thereby setting back years of progress made possible through nongovernment participation and partnership in tireless, persistent advocacy efforts. (The existing plan structure continues to afford medical foods coverage to children only as eligible, reimbursed, durable medical equipment and supplies). The federal government has never classified medical foods as fit for their intended purpose, which is appropriately used as a drug in the clinical practice setting and under conditions of safe use in the treatment and mitigation of rare, inborn errors of metabolism like PKU. As of May 2015, the US Office of Personnel Management and the Blue Cross and Blue Shield Association changed the contracted terms of agreement on coverage of PKU treatment for FEHB employees on the basis that my prescription for medical food now "is properly categorized as an over the counter product and FEP does not currently cover OTC products except in limited circumstances," (meaning up to age 22). Medical foods are a separate and distinct category that fall in the spectrum of food and drugs - not Rx, not OTC; but clearly warrant exceptional treatment from the larger, overarching shadows of the nutraceutical world. Thus, the

lack for a uniform standard across the state and federal level and changing political environment is making it even harder now to convince health plans and policymakers on the need for robust coverage.

I continue to champion policy appeals at the national level to bring this lifelong, necessary and critical treatment back into the hands the medically disenfranchised and hope to set a new precedent for restoring a positive change. A shared sense of responsibility in obligatory, legal and ethical requirements for the provision, dissemination and sustainability of policies and practices on safe, compassionate care necessitates compliance by all parties in resolutions processes. Engagement with the primary stakeholder – the patient, must be included in defining a reaffirmation to the mission of caring and setting a new course for success and promoting a healthy diversity in adoption of public health policies.

More on contributing author, Jennifer Payne, and related works [*Solving the Satisficing Cost of Medical Food Misbranded*](#) are published to the [Rare Disease Report](#)

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