Medical Foods – Provider Perspective – SIMD

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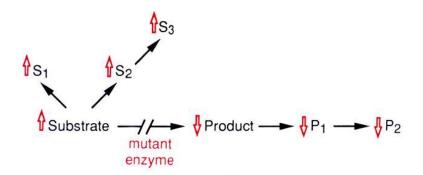
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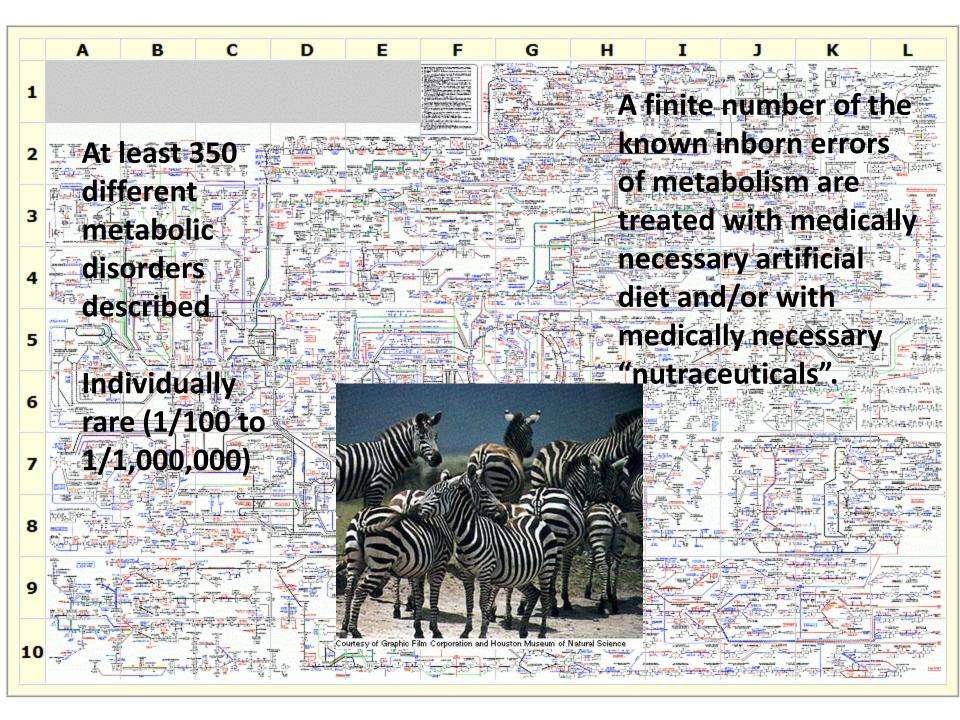
The Society for Inherited Metabolic Disorders is a professional organization of physicians, nutritionists, nurses and other health care professionals involved in the diagnosis and treatment and researchers who study inborn errors of metabolism.

What is IEM?

- Potential for (immediate and chronic) treatment
 - Patients with IEM can die in hours or less, or suffer permanent neurologic damage -BUT
 - Treatment can permit normal growth and development

Genetic alterations of biochemical pathways that affect physiologic function





EXAMPLE: Classic PKU

OUTCOME:

- Untreated: IQ of 20 or 30, autism, seizures.
- Treatment stopped in childhood: IQ drops, behavior problems, mental health problems.
- Treatment stopped in adult life: irritability, judgment and mental health problems, white matter (can be irreversible) brain disease, and for women birth defects in their children.
- Treatment early and continuous: IQ and behavior in normal range, and affected women have healthy children. For this reason, newborn screening was developed
- Cornerstone of treatment: restrict the amino acid phenylalanine, and monitor blood levels and growth with constant adjustment of diet by physician and dietician. Since phenylalanine is a component of all natural protein, treatment requires artificial diet using "Medical Food". This diet would be fatal for a person without PKU. There is no natural substitute for the Medical Food. Medical food provides typically 70% to 90% of the protein in the diet for classic PKU and therefore most of the calories.

FDA Definition: Medical Food

"a food which is formulated to be consumed or administered enterally under the supervision of a physician and which is intended for the specific dietary management of a disease or condition for which distinctive nutritional requirements, based on recognized scientific principles, are established by medical evaluation."

as defined in section 5(b) of the Orphan Drug Act (21 U.S.C. 360ee (b) (3)) This legal definition of a medical food was added to the Orphan Drug Amendments of 1988

EXAMPLE: Biotinidase deficiency

Outcome:

- Completely untreated: death.
- Partially treated: mental retardation, cerebral palsy, deafness.
- Completely treated: normal (although some may have deafness).
- Treatment: Biotin, 10,000 to 20,000 micrograms per day (normal biotin requirement for person without the disorder is about 300 micrograms per day).
- Is biotin a "Medical Food"?

Other "nutraceuticals"

- Carnitine for carnitine transporter defects (covered on many forumlaries)
- CoQ for CoQ synthetic defects
- Arginine for MELAS
- Arginine and/or citrulline for urea cycle
- OH-cobalamin for CBL A, B, C, etc (covered on many formularies)
- MCT for VLCADD
- Valine and/or isoleucine for (most) MSUD

HRSA supported survey of families

- Nearly all families surveyed had some type of health care coverage (even if it did not pay for these products)
- Most children needed more than one category of food/supplies
- Coverage was variable but there were at least some outof-pocket expenses
 - For about 20% of families using Medical Foods
 - For about 30% of families using "supplements" ("nutraceuticals")
 - ALSO evaluated in the survey:
 - For about 35% of families using feeding supplies
 - For about 60% of families using Modified Low Protein Foods

Summary/Problems

- Medical Foods prescribed for treatment of IEM are not optional: they are medically necessary treatment
- Treatment is life-long
- "Nutraceuticals" prescribed for treatment of IEM are not optional or supplemental: they are medically necessary treatment
 - Always recognizing the need for ongoing research, there are clearly examples for which the necessity for treatment is clearly established
- Medical Foods are substantially more expensive than traditional foods and "Nutraceuticals" can be extremely costly
- Many insurers consider both categories "foods" or "supplements" and exclude them from coverage despite agreement by their medical directors that the treatment is "medically necessary".
- Costs may be prohibitive; coverage is at best variable
- My colleagues and I cannot care for affected persons without Medical Foods and "nutraceuticals" -- but our patients often do not have access to them

Decades of Support for Access to Tx

- FDA definition of Medical Foods recognizes a distinct and important category that are not "drugs" and that are critical to prevention of mental retardation and death in people with inborn errors of metabolism
 - I am NOT suggesting that Medical Foods should be recategorized as drugs; there are many advantages to the category defined by FDA
- The American Academy of Pediatrics first supported coverage for what would later be called Medical Foods in the 1960s, and reaffirmed this support in 2003.
- Also in the 1960s, Champus and Kaiser supported coverage of Medical Foods.
- See 2007 SIMD statement of support for coverage of Medical foods and related nutritional ("nutraceutical") therapies at: http://www.simd.org/Statements/MedicalFoods2007.htm

SIMD Statement on "Coverage for Medical Foods"

 "The Medical Foods ordered by the metabolic medical team for an individual with an inborn error of metabolism, such as the artificial foods and the amino acids for the treatment of maple syrup urine disease, should be regarded as equal to, and covered to the same extent as, medications critical to life – such as insulin for diabetes – are covered."

Conclusion

- Medical Foods and "nutraceuticals" are critical elements of therapy for IEM
 - Many of the conditions requiring this treatment are detected by NBS beginning with PKU, for which NBS was originally developed
- With access to these therapies we can reduce or prevent disability and death
 - Without access to these therapies, we lose much of the benefit of NBS, without access to these therapies there are also increased medical costs e.g. hospitalization of those with MSUD, psychotropic and anticonvulsant drugs for those with PKU
- Access to the therapies depends on
 - Research (and the SIMD also has a statement in support of research)
 - Development, manufacturing, marketing of the therapies
 - Coverage by payors