Presentation to the Federal *Advisory Committee on Heritable Disorders and Genetic Diseases in Newborns and Children*: Subcommittee on Follow-Up and Treatment

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Medical Requirements for Immediate and Long-Term Follow-Up Care

- Screen-positive infants may require specialized confirmatory testing, additional diagnostic tests, medical specialty consultations, specialized treatments (e.g., cord blood stem cell transplants) and other services that are best coordinated through a Center of Excellence.
- Evidence-based guidelines for follow-up care (e.g., ACMG) are critical.

Funding of Newborn Screening and Immediate and Long-Term Follow-Up Medical Needs

- In NYS, the NBS program is a functional unit within the Department of Health and the actual screening labs are funded through the state budget.
- Follow-up medical needs, both immediate and long-term, must be funded through the healthcare system, which can give rise to access issues.

Sources of Access Issues (both Immediate and Long-Term): NYS Medicaid Fee-for-Service

- In the Medicaid program, all medically necessary care is covered.
- However, some critical services are only available out-of-state.
- Coverage can be provided in the Medicaid FFS program through enrolled out-of-state providers.
- Enrollment in the Medicaid FFS program is voluntary on part of the provider. If a provider will not enroll, an access issue may ensue.

Sources of Access Issues (both Immediate and Long-Term): NYS Medicaid Managed Care

- Out-of-network providers may be covered only with prior authorization.
- Medical necessity of using an out-of-network provider or a Center of Excellence may be questioned.
- The need for rapid inpatient workup may be questioned.
- Lack of familiarity with the disease on the part of the plan may delay obtaining prior authorization.

Possible Solutions

- State Medicaid agencies can publish coverage guidelines emphasizing evidence-based protocols.
- Guidelines should have input from stakeholders to ensure relevance at the point of care.
- Guidelines should be coordinated with the state NBS program to ensure continuity of care.

Example: Krabbe Disease Program in NYS

- Joint effort with NYS Newborn Screening program, Medicaid program, and stakeholders, including Child Neurology Krabbe Consortium of NYS.
- Published Medicaid coverage guidelines apply to both FFS and managed Medicaid. This represents a standard that can be adopted by commercial insurance.
- Education efforts focused at managed care medical directors.
- Efforts to enroll confirmatory labs into the Medicaid program.

Krabbe Disease, NYS Medicaid Guidelines for Payment for Testing and Treatment

- **Mandated Newborn Screening and Medicaid Reimbursement**
- Because of the need for a rapid diagnosis and initiation of therapy for confirmed cases, the New York State Newborn Screening Program began testing for Krabbe Disease in August 2006 as part of the mandated newborn screening panel.
- The New York State Medicaid Program covers the testing and treatment of Krabbe Disease, which includes, but is not limited to, the following procedures:
- Confirmatory testing;
- Evaluations and consultations by a metabolic disease specialist and/or child neurologist;
- Neurological testing, including lumbar puncture, MRI, nerve conduction studies, visual evoked response and brain stem auditory evoked response;
- Myeloablative chemotherapy; and
- Newborn and young infant umbilical cord blood transplantation, which, prior to onset of symptoms, has been shown to stabilize the Disease.

Possible Solutions (con't)

- Develop lists of regional and national resources (labs, Centers of Excellence).
- Encourage enrollment of these resources in state Medicaid programs and managed care networks.
- Consider Federal designation with funding and requirement to support state Medicaid programs as well as all screen-positive children under other insurance programs.

Possible Solutions (con't)

- Further development of evidence-based guidelines that can easily be converted into coverage policies.
- Formal education programs for managed care medical directors and executives.

Coverage of Enteral Formulas

- NYS has a mandate which applies to managed care and is followed by the Medicaid program.
- Does not apply to self-insured employers

NYS Enteral Formula Mandate

- Insurance Law Section 3216(i)(21) states: "Such written order [i.e., a doctor's prescription] shall state that the enteral formula is clearly medically necessary and has been proven effective as a disease-specific treatment regimen for those individuals who are or will become malnourished or suffer from disorders, which if left untreated, cause chronic physical disability, mental retardation or death. Specific diseases for which enteral formulas have been proven effective shall include, but are not limited to, inherited diseases of amino acid or organic acid metabolism; Crohn's Disease; gastroesophageal reflux with failure to thrive; disorders of gastrointestinal motility such as chronic intestinal pseudo-obstruction; multiple, severe food allergies which if left untreated will cause malnourishment, chronic physical disability, mental retardation or death."

NYS Medicaid Coverage of Enteral Therapy

- **Enteral Therapy Services**
- Enteral nutritional therapy is covered by the Medicaid Program under the following conditions:
- The enteral nutritional therapy must be an integral component of a documented medical treatment plan and ordered in writing by an authorized prescriber.
- Enteral nutritional therapy is covered for nasogastric, jejunostomy or gastrostomy tube feeding or as liquid oral enteral nutritional therapy when there is a documented diagnostic condition where caloric and dietary nutrients from food cannot be absorbed or metabolized.
- Medical necessity for enteral nutritional therapy must be substantiated by documented physical findings or laboratory data (e.g., changes in skin or bones, significant loss of lean body mass, abnormal serum/urine albumin, protein, iron or calcium levels, or physiological disorders resulting from surgery, etc.).

NYS Medicaid Coverage of Enteral Therapy (con't)

- A Medicaid recipient will be eligible for an enteral nutritional supplement if the following conditions are met:
- There is an established diagnostic condition where the patient is unable to sustain himself/herself nutritionally by eating regular food and the condition is one where nutritional supplements are generally considered by the medical community as the treatment-of-choice to produce nutritional and medical benefit.
- A physician orders the nutritional supplement in writing.
- The physician or other appropriate health care provider has documented the patient's nutritional depletion or provided an explanation for why nutritional depletion is imminent and can be forestalled by providing a specific nutritional supplement.

Covered Pathological Processes and Associated Clinical Conditions

PATHOLOGICAL PROCESS	ASSOCIATED CLINICAL CONDITION
Inadequate food intake	Nausea and vomiting, anorexia, early satiety, wired jaw, poor dentition, stomatitis, dysphagia, severe dyspnea, periodontitis
Impaired digestion and absorption of nutrients	Vomiting/diarrhea, obstructed bowel, enteropathy, resected small bowel, bowel fistula, biliary obstruction
Defective nutrient utilization	Inherited metabolic defect, lactose intolerance, hyperglycemia, proteinuria, protein loosing enteropathy, altered lipid metabolism, chyluria
Enhanced nutrient requirement	Increased metabolic rate, fever, dyspnea