

Metabolic Food & Formula Program



Program Report (FY 2019)
July 1, 2018 – June 30, 2019





Congenital & Inherited Disorders



Iowa Department of Public Health *Advancing Health Through the Generations*

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WHAT IS THE METABOLIC FOOD AND FORMULA PROGRAM?

Babies born in Iowa are tested for a variety of disorders 24-48 hours after birth by the Iowa Newborn Screening Program. Many of the diseases tested for are life threatening and/or disabling and require immediate intervention and lifelong treatment. One category of diseases screened for are called “inborn errors of metabolism”. Patients with these types of disorders require lifelong medications and/or specialized, restrictive diets including prescribed medical formula and medical foods.

Medical foods are modified to be low protein and are critical treatments for inborn errors of metabolism, major targets for newborn bloodspot screening. The Food and Drug Administration (FDA) defines medical foods as:

“...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.” (section 5(b) of the Orphan Drug Act (21 U.S.C. 360ee (b) (3))).

The Metabolic Formula Program was created over 20 years ago to assist families of Iowan’s diagnosed with Inborn Errors of Metabolism with the cost of the metabolic formula. These patients are on very rigid, restricted diets. In order to get the nutrients they need to survive and thrive, they must drink specialized formulas and eat medically modified foods. These special foods and formulas are as essential to them as insulin is to a diabetic person. However, unlike insulin, the cost of these foods and formulas are often not covered because insurance companies consider them to be a dietary supplement and not a medical necessity. Even when insurance pays a portion of the cost of the formula, the cost to the patient is still significant. With the Metabolic Formula Program, the patients benefit from the buying power/contracts of the University of Iowa.

The Metabolic Formula Program is funded from three sources – 1) insurance reimbursement / patient payments; 2) \$4 from each Iowa newborn screen; and 3) any remaining allocation from the legislative appropriation.

The Metabolic Food Program was established in November 2005 from a State of Iowa appropriation to the Iowa Department of Public Health. The funding priority is for the purchase of medical food, with any remaining funds being used to help cover the deficit for the Metabolic Formula Program. Prior to this funding source, patients had to purchase medical food with personal funds. We did not receive any legislative appropriation in FY18. Many patients went without medical foods because the cost is so high (i.e. a loaf of low protein bread can cost as much as \$14).

WHO IS ELIGIBLE TO PARTICIPATE IN THE METABOLIC FOOD AND FORMULA PROGRAM

To be eligible for the Metabolic Food and Formula Program, the patient must:

- be a resident of Iowa with an inborn error of metabolism.
- be followed in a metabolic genetics clinic on a regular basis (as defined by the medical director).
- follow the treatment plan and recommendations.

HISTORY OF TREATMENT OPTIONS FOR PHENYLKETONURIA (PKU)

When the Iowa Newborn Screening Program started, a medical formula called Lofenalac was the only medical formula and only treatment available for PKU. Lofenalac powder was mixed with water and provided the individual amino acids, vitamins and minerals, but no phenylalanine. This therapy reduced phenylalanine levels and prevented the irreversible brain damage associated with untreated PKU, but it was not easy for the child or their parents.

Older adults with PKU have vivid memories of how difficult it was to drink Lofenalac as the taste and odor were quite unpleasant. They also remember “always being hungry” as the only medical food available in the early years of treatment was low protein bread. Parents made the bread in a coffee can and it often came out rock hard or semi-solid. It added to the stress of the family as it was very expensive and often inedible.

Fortunately, advances have been made in the types and flavors of PKU formulas and medical foods. Research has led to additional treatment options as well.

- PKU formula – multiple companies now make medically modified PKU formulas in many different flavors and forms (powder, ready to drink, juice boxes for on the go, pills, bars, etc).
- Large Neutral Amino Acids (LNAAs) – a form of treatment for adults who have difficulty following the phenylalanine restricted diet. It is taken three times a day with meals and comes in pill or powder form. It provides the amino acids that can cross over into the brain. This not only makes the patient feel better (think clearer, feel less foggy), but the LNAAs also compete with phenylalanine to cross the blood brain barrier. If you flood the system with good LNAA's, it limits the amount of phenylalanine that can cross over, thus leading to the patient feeling better.
- Kuvan (Sapropterin) – a cofactor therapy (oral medication) was approved by the FDA in 2007 for treatment of PKU. This form of treatment will decrease phenylalanine levels in about 50% of individuals with PKU. It does not replace the phenylalanine restricted diet, but, depending on the individual's response, it may lower phenylalanine levels allowing the patient to increase the amount of natural protein in their diet from food, and in some patients, decrease the amount of PKU medical formula needed.

- Pegvaliase (Palynziq) – in May 2018 the FDA approved the first enzyme replacement therapy for PKU for adults who have uncontrolled phenylalanine levels. It is given by subcutaneous injection once each day. There are however, possible side effects associated with this form of treatment and this would not be a treatment option for women of child bearing age.

Ongoing research continues for potentially new and curative therapies for PKU, including:

- Recent approval of a Phase 1/2 trial for single dose administration of a PKU gene therapy. The trial will be for adults with PKU, ages 18-55.
- Phase 1b trials in Australia of a therapeutic enzyme in the GI tract to treat PKU.
- A Phase 1b trial, the first ever clinical investigation of genetically engineered red blood cells to treat PKU.
- A Phase 1/2a clinical trial with a synthetic biotic medicine for treating PKU. This therapy uses bacteria that are genetically engineered to consume phenylalanine in the intestine.

PATIENT STATISTICS

Patient Ages

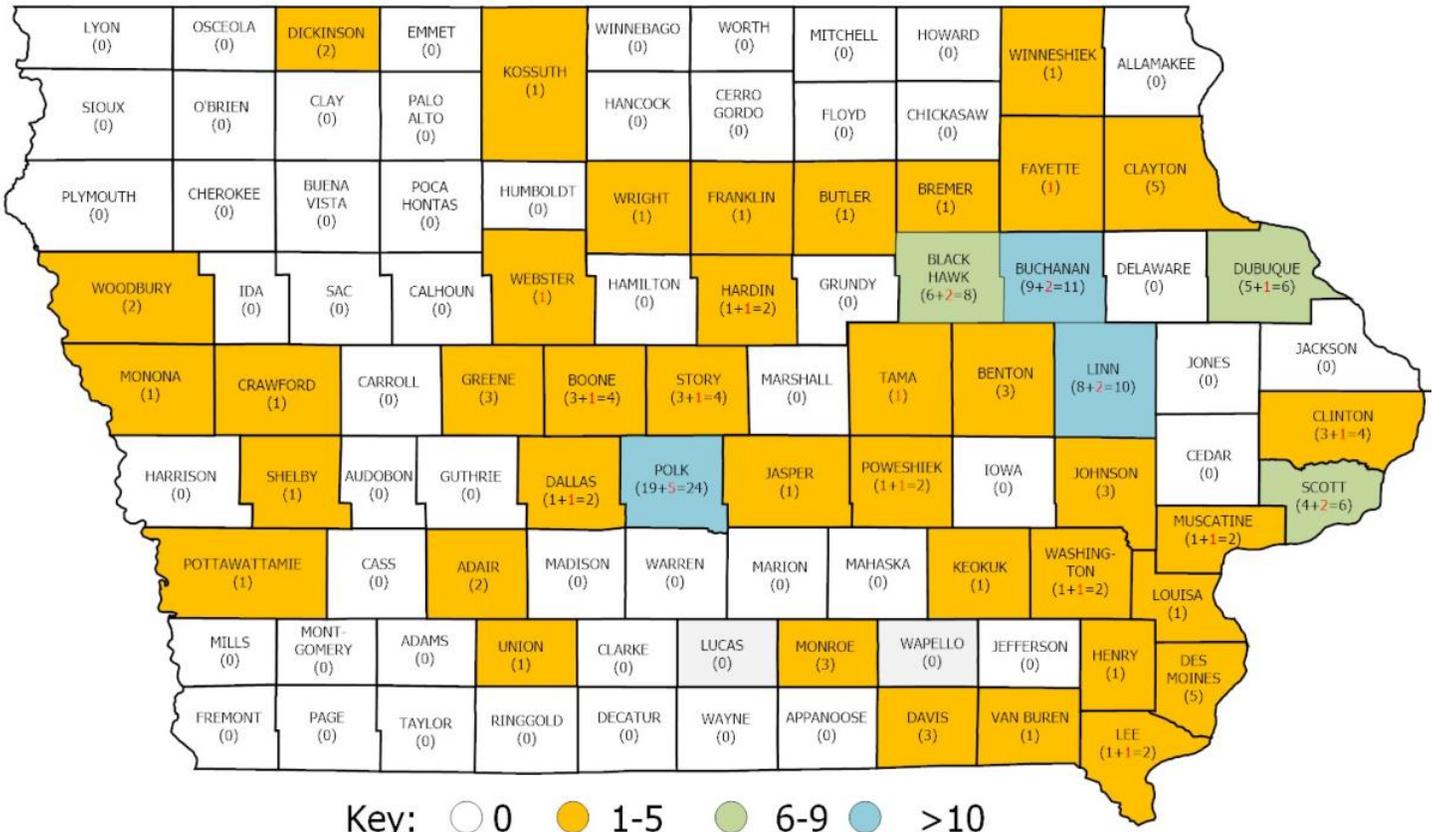
Age/Category	Number of Patients in Formula/Food Program FY 16	Number of Patients in Formula/Food Program FY 17	Number of Patients in Formula Program* FY 18	Number of Patients in Formula/Food Program FY 19
Children 0-17	72	73	60	78
Adults 18-on	57	59	71	61
TOTAL	129	132	131	139

This table shows enrollment numbers for FY16, FY17 and FY19. The number is from the program's patient spreadsheet. *We did not receive legislative appropriation in FY18. There were 8 new patients added to the program since FY18.

PATIENTS SERVED BY COUNTY

FY 2019 (139 patients)

FY 2019 Metabolic Food & Formula Program Patient Breakdowns by County



Key: ○ 0 ● 1-5 ● 6-9 ● >10

Numbers in red = Formula Only (26 pts)

Numbers in black = Both Food & Formula (113 pts)

Total # Pts = 139

PROGRAM OPERATIONS

Patients are enrolled into the program by self-referral or by being identified with an inborn error of metabolism in our metabolic clinic, usually through a positive newborn screening result.

Patients receive dietary prescriptions for the appropriate amount and type of low protein food and formula through the Metabolic Genetics Clinic at the University of Iowa. The Metabolic Food and Formula Program is discussed during the initial visit to the metabolic clinic. Patients are then enrolled in the program. After the first visit, families/patients call the dietitian to initiate the order for formula and fax or email their food orders to the program staff.

All metabolic formulas are purchased through special purchasing arrangements with the University of Iowa. The dietitian places the order with the appropriate vendor for formula and the formula is delivered directly to the patient. The program is billed for the cost of the formula. The University of Iowa Hospitals and Clinics then processes insurance claims when applicable. Once the claim process has been completed, the sliding fee schedule is applied if the patient is enrolled in the University' (see below) and the patient is billed for the balance of the formula when applicable.

The food portion of the program is administered somewhat differently. The IDPH legislative appropriation for medical food was divided equally amongst program participants. For FY19, the allocation was \$740 per patient.

Order forms for the low protein food vendors are given to each patient. The patient fills out the forms and sends the order forms to the program staff. Our staff reviews and then places the orders. The total cost of the orders is then entered into a spreadsheet. Food is delivered directly to the patients and the program is billed for the cost of the medical food ordered. Once the patient spends their allotted amount of money on low protein food, no more orders are accepted. At that time the patients must order and pay for the low protein food out of their personal funds.

This year, any unspent funds reverted back to the program to help offset the deficit for metabolic formula.

University of Iowa Hospitals and Clinics Financial Assistance Program

Patients who need assistance with the cost of formula after insurance is billed may apply to this program and if they qualify, there is a sliding fee scale to help with the cost of metabolic formula. This replaced the former Iowa Metabolic Food and Formula Program application for financial assistance.

Insurance

We provide letters of medical necessity for families to use in their efforts to get coverage for the prescribed metabolic formula and medical foods.

Our program continues to have ongoing difficulties receiving insurance coverage. Currently, it is unknown whether the Managed Care Organization (MCO) will cover the cost of metabolic formula. Medicare and some private insurance companies will not pay for metabolic formula.

With much work, some private insurances and Medicaid(s) are now covering the cost of low protein food. We have partnered with University of Iowa Home Healthcare in this endeavor and this has gone well.

Fiscal Information

Detailed financial information for our program can be found in a separate fiscal report submitted to the Iowa Department of Public Health.

Education

We provide assistance and education to school nurses, teachers and school lunch programs regarding the disorders and assist them in making low protein lunch menus and give them information on how to order low protein foods.

Patient education is continuously provided for patients during each clinic visit, as well as when patients contact program staff regarding additional educational needs. Program staff also attend meetings related to metabolic conditions and special dietary needs so that they can provide the most current information and education to the patients they serve.

Meetings Attended:

Name	Title	Meeting Name	Dates	Location
Cheryl Stimson	Dietitian	Metabolic Nutrition Webinar	October & November 2018 February & April 2019	Iowa City, IA
Cheryl Stimson	Dietitian	Genetic Metabolic Dietitians International	May 2019	Memphis, TN

Patient Education Meetings:

Name	Title	Meeting Name	Dates	Location
Cheryl Stimson	Dietitian	Metabolic Patient Meeting	October 2018	Des Moines, IA
Judy Miller	ARNP	Metabolic Patient Meeting	October 2018	Des Moines, IA

Summary

The biggest challenge for the Iowa Metabolic Food and Formula Program continues to be the lack of reimbursement and/or low reimbursement for medical food & formula from certain insurance providers. The National PKU alliance continues to work on a federal level to mandate coverage for prescribed metabolic formulas.

Submitted by Lisa Neff-Letts, Program Coordinator