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## INHERITED METABOLIC DISEASE BENEFITS

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Select formula, low protein food and drugs may be included as an Inherited Metabolic Disease Benefit for clients who meet the established criteria.

### A. Formula and Low Protein Food

In addition to being an eligible Saskatchewan Health beneficiary, clients must:

1. be diagnosed with an inherited metabolic condition (may or may not be screened through newborn screening),
2. be prescribed treatment that includes formula that is used to replace an element of a typical diet (e.g. protein),
3. be prescribed formula that is only a component of their diet and is/will be less than 90% of their caloric intake after age one,
4. be prescribed formula that is not available through local suppliers; and,
5. follow a strict diet. If the diet is not followed, symptoms will be severe and include, but are not limited to, cognitive impairment and/or physical disability such as blindness.

Eligible clients receive the following benefits at no charge:

1. Formula from the list of approved products.\*
2. Low protein food from the list of approved products.\*

\* The legislative authority for the formula and the low protein food is The Saskatchewan Aids to Independent Living Regulations, 1976 (Saskatchewan Regulation 292/76).

## 1. Formula

<b>FORMULA</b>			
<b>Abbott</b>		<b>Nutricia (continued)</b>	
Cyclinex 1	400g	Glutarade Junior GA-1 Drink Mix	400g
Cyclinex 2	400g	MSUD Aid	200g
Glutarex-1	400g	MSUD Analog	400g
Glutarex-2	400g	MSUD Maxamaid	454g
Hominex-1	400g	MSUD Maxamum	454g
Hominex-2	400g	Periflex Advance	454g
I-Valex 1	400g	Periflex Infant	400g
I-Valex 2	400g	Periflex Junior	454g
Ketonex-1	400g	Periflex Junior Plus	400g
Ketonex-2	400g	Phenylade 60	454g
Phenex 1	400g	Phenylade	454g
Phenex 2	400g	Phenylade Amino Acid Blend	454g
Pro-Phree	400g	Phenylade Essential	454g
Propimex 1	400g	Phenylade GMP	400g
Propimex 2	400g	Phenylade MTE Amino Acid Blend	454g
Tyrex-1	400g	TYR Anamix Next	400g
Tyrex-2	400g	UCD Anamix Junior	400g
<b>Cambrooke</b>		XLYS Xtrp Analog	400g
Bettermilk	49g-52g	XLYS Xtrp Maxamaid	454g
Camino Pro PKU	140ml	XLYS Xtrp Maxamum	454g
<b>Mead Johnson</b>		XMET Analog	400g
BCAD-1	454g	XMET Maxamaid	454g
BCAD-2	454g	XMET Maxamum	454g
PFD-1	454g	XMTVI Analog	454g
PFD-2	454g	XMTVI Maxamaid	454g
Phenyl-Free 1	454g	XMTVI Maxamum	454g
Phenyl-Free 2	454g	XPhe Maxamaid	454g
Phenyl-Free 2 HP	454g	XPhe Maxamum	454g
Tyros-1	454g	XPHE Xtyr Analog	400g
Tyros-2	454g	XPHE Xtyr Maxamaid	454g
<b>Nutricia</b>		XPHEN TYR Maxamaid	500g
Acerflex	454g	XPHEN TYR Maxamum	500g
Complex Essential MSD Drink Mix	454g	XPTM Analog	400g
Complex Junior MSD Drink Mix	400g	<b>Vitaflo</b>	
Complex MSUD Amino Acid Blend	454g	EAA Supplement	12.5g
Duocal	400g	HCU Cooler	130ml
Glutarade Essential	454g	HCU Cooler 20	174ml
Glutarade GA-1 AA Blend	454g	HCU Express	25g

## 2. Low Protein Food

<b>LOW PROTEIN FOOD</b>	
<b>Baking Mixes</b>	<b>Breads</b>
Aproten Flour Mix (Farina)	Cambrooke Artisan Bread
Cambrooke Baking Mix	Cambrooke Bagels
Cambrooke Mix Quick	Cambrooke Breakfast Bars
Cambrooke Wheat Starch	Cambrooke Brookelyn Dog Buns
Country Sunrise Muffin Mix	Cambrooke Camburger Buns
Country Sunrise Pancake Mix	Cambrooke Cinnamon Raisin Swirl Bread
Dietary Specialties Bread Machine Mix	Cambrooke Homestyle Sliced White Bread
Dietary Specialties Low Protein Baking Mix	Cambrooke Pita Pockets
Juvela Low Protein Mix	Cambrooke Scones
Nutricia Loprofin All Purpose Baking Mix	Cambrooke Southwestern Biscuits
Nutricia Maddy's Homestyle Muffin Mix	Cambrooke The Bigger Bagels
Nutricia Wel-Plan Wheat and Corn Starch Baking Mix	Cambrooke Tortilla Wraps
Taste Connections Low Protein Bread Mix	Cambrooke Tuscan Pizza Crusts
Taste Connections Low Protein Versa Mix	<b>Cereals</b>
Taste Connections Multi-Baking Mix	Dietary Specialties Porridge
Taste Connections Wheat Starch	Nutricia Loprofin Breakfast Cereal Loops
<b>Rice</b>	Nutricia Milupa LP-Flakes
Aproten Chicchi/Riso Rice	Nutricia Milupa LP-Fruity Cereal Mix
Cambrooke Short Grain Rice	Promin Pasta Meal (Porridge)
Dietary Specialties Imitation Rice	<b>Pastas</b>
Nutricia Loprofin Rice	Aproten Pasta, Assorted
Promin Pasta Cous Cous	Cambrooke Pasta, Assorted
Promin Pasta Imitation Rice	Dietary Specialties Pasta, Assorted
<b>Meat Substitutes and Cheese</b>	Nutricia Loprofin Pasta, Assorted
Cambrooke Brookelyn Dogs	Promin Pasta, Assorted
Cambrooke Camburgers	<b>Snacks</b>
Cambrooke Cheese Shreds	Aproten Biscotto
Cambrooke Cheese Singles	Aproten Cracker Toast/Fette Tostate
Cambrooke EGGZ	Aproten Crispbread/Rusks
Cambrooke GO! Pockets	Nutricia Loprofin Crackers
Cambrooke Meatballs	<b>Miscellaneous</b>
Cambrooke Mini Pockets	Cambrooke Alfredo Sauce Mix
Cambrooke Pierogi	Cambrooke Chicken Consomme and Seasoning
Cambrooke Ravioli	Cambrooke Shake N'Cheese
Canbrands PaneRiso Egg Replacer	Country Sunrise Cheese Flavoured Sauce Mix
Country Sunrise Burger Mix	Country Sunrise Instant Mashed Potato Mix
Country Sunrise Imitation Scrambled Egg and Omelette Mix	Dietary Specialties Cheese Flavoured Sauce Mix
Country Sunrise Peanut Butter Spread Mix	Dietary Specialties Tomato Flavoured Sauce Mix
Country Sunrise Vegetable Chicken Nugget Mix	La Tiara Taco Shells
Country Sunrise Vegetable Hot Dog Mix	Nutricia Milupa LP-Drink Mix
Dietary Specialties Peanut Butter Flavoured Spread	Washington Golden Seasoning and Broth
Ener-G Egg Replacer	Washington Rich Brown Seasoning and Broth
Walden Farms Whipped Peanut Spread	

## B. Drug Benefits

Please note, physicians may make individual case by case requests for medications to treat inherited metabolic diseases. Requesting physicians are asked to provide the Drug Plan with clinical information to support the request.

### 1. saproterin dihydrochloride (Kuvan-BPC) - Criteria For Coverage

For more information on how to obtain coverage, or if you have questions regarding the criteria or billing procedures, please contact the Drug Plan.

#### Initial Inclusion Criteria:

#### Non-Pregnant Patients and Patients actively planning pregnancy:

- For the management of patients with the diagnosis of Phenylketonuria (PKU) who meet **ALL** of the following criteria:
  1. Compliance with low protein diet and formulas.
  2. Baseline blood phenylalanine (Phe) levels > 360 µmol/L despite compliance with low protein diet (require at least 2 levels during 3 to 6 month time frame).
  3. Baseline protein intake assessment by a dietitian.
  4. Ability to comply with medication regimen.
  5. Managed by a physician specialized in metabolic/biochemical diseases.

#### Pregnant Patients:

- For the management of patients with the diagnosis of Phenylketonuria (PKU) who meet **ALL** of the following criteria:
  1. Managed by a physician specialized in metabolic/biochemical diseases.
  2. Baseline blood phenylalanine (Phe) levels > 360 µmol/L despite compliance with all recommendations for dietary intervention and monitoring.

#### Exclusion Criteria:

- Known hypersensitivity to saproterin or its excipients.
- Any other contraindications.
- Baseline Phe Levels <360 µmol/L for non-pregnant patients.
- Baseline Phe Levels <360 µmol/L for pregnant patients.
- Women who are nursing/breast feeding.
- Patients not on diet or not compliant with diet.

#### Caution:

- Taking medication known to inhibit folate synthesis (e.g., methotrexate).
- Any condition requiring treatment with levodopa or any PDE-5 inhibitor (e.g., sildenafil).

#### Test for Eligibility: 72 hour Kuvan Challenge

- 72 hour challenge with Kuvan at 20 mg/kg/day - blood Phe concentrations are measured at the following timepoints (in hours): T -48 to T -24, T0, T4 to T12, T24, T 48 to T 72, OR
- As per clinic's protocol.

Dose of Kuvan to establish clinical benefit is 20 mg/kg/day.

## **Definition of Responder to Kuvan Challenge:**

### **For Non-Pregnant Patients and Patients actively planning pregnancy:**

Criteria to determine if patients have “responded” to 72 hour Kuvan challenge:

- Reduction in Phe blood level of at least 30% compared to baseline; AND
- *Baseline assessment of neurobehavioural or neurocognitive impairment\* and quality of life assessment due to PKU after 72 hr Kuvan challenge but before start of Kuvan therapy (this assessment does not apply to pregnant women).*

Note:

- *Baseline Phe tolerance level must be documented as well as Phe tolerance levels documented at months 1-2 and 4-6 while on Kuvan therapy.*

Approval Duration: 6 months **(KUVAN THERAPY TO BE FUNDED BY THE MANUFACTURER FOR 6 MONTHS)**

\* For children less than 4 years of age, clinically validated age-appropriate neurobehavioural, neurocognitive, or developmental tests may be selected at the clinician's discretion rather than PKU specific tests.

### **Pregnant Patients:**

Criteria to determine if patients have “responded” to 72 hour Kuvan challenge:

1. Reduction in Phe blood level of at least 30% compared to baseline after 72 hours.

For patients who meet “responder” definition to Kuvan Challenge, may be eligible for Kuvan funding if the following criteria are met:

- A decrease in Phe concentration to less than 360 µmol/L to be maintained for the duration of pregnancy to be eligible for continued funding.

Approval Duration: Until end of pregnancy, no renewals **(KUVAN THERAPY TO BE FUNDED BY THE MANUFACTURER FOR 6 MONTHS)**

## **INITIAL REQUESTS FOR FUNDING AND RENEWAL REQUESTS:**

**(AFTER INITIAL SIX-MONTH TRIAL DEMONSTRATING RESPONSE TO KUVAN THERAPY)**

### **Non-Pregnant Patients and Patients actively planning pregnancy:**

- For the management of patients with the diagnosis of Phenylketonuria (PKU) who meet **ALL** of the following criteria:
  1. Compliance with low protein diet, formulas, and Kuvan; AND
  2. Achieve:
    - a) normal sustained Blood Phe levels [ > 120 µmol/L and < 360 µmol/L] (At least 2 levels measured at least 1 month apart); OR
    - b) sustained blood Phe reduction of at least 30% (At least 2 levels measured at least 1 month apart) compared to baseline if the Phe baseline level is < 1200 µmol/L; OR
    - c) sustained blood Phe reduction of at least 50% (At least 2 levels measured at least 1 month apart) compared to baseline if the Phe baseline level is > 1200 µmol/L;

AND

  3. Demonstrated increase of dietary protein tolerance based on target set between the clinician and patient; AND

4. Clinically meaningful age-appropriate improvement in:
  - Neurobehavioural or neurocognitive function or impairment for patients with such impairments as determined by peer reviewed clinically validated scales; OR
  - Demonstrated improvement in Quality of Life using peer reviewed validated scales; AND
5. Managed by a physician specialized in metabolic/biochemical diseases.

*Dosage: Up to a maximum of 20 mg/kg per day.*

Approval Duration: 1 year

**Pregnant Patients:**

***No renewals will be considered.***

***Note: Patients enrolled in sapropterin clinical trials PKU 015, PKU 016 and Kuvan Assistance Program would have to meet provincial coverage criteria in order to qualify for funding.***

**1. Coverage for Fabry Disease**

Saskatchewan participates in the Canadian Fabry Disease Initiative (CFDI). Prescribers, pharmacists or patients with questions regarding coverage of enzyme replacement therapy for the treatment and management of Fabry Disease are asked to call the Drug Plan and Extended Benefits Branch for more information.