



Patients celebrate B.C.'s decision to fund medical foods to protect against brain-threatening disease

- Access to Kuvan still needed as third component of PKU Comprehensive Brain Protection Strategy -

Vancouver, B.C. – November 28, 2013-- Canadian PKU and Allied Disorders (CanPKU) is delighted with the B.C. government's decision to provide funding for special low-protein medical foods for patients affected by Phenylketonuria (PKU) and similar inborn errors of metabolism (IEMs). Medical foods play a critical role in preventing devastating neurocognitive, psychiatric and physical symptoms, and in some cases even death, caused by PKU and 24 other rare, inborn metabolic disorders. The foods are one of three medical components of CanPKU's *Comprehensive Brain Protection Strategy for People with PKU* submitted to the B.C. government in February 2013.

In PKU, the body is unable to process an essential amino acid found in dietary protein, called phenylalanine (or "Phe"), and the resulting accumulation of Phe in the blood is toxic to the brain. If left untreated, symptoms can range from mild cognitive impairment to severe mental retardation. As such, patients must adhere every day of their lives to a complicated and highly-restrictive low-protein diet to stave off Phe's harmful impacts. Medical foods help PKU patients maintain their blood Phe levels at acceptable levels, protecting their brains.

Patients with other IEMs are affected by different amino acids in protein, and low-protein medical foods help them to maintain proper growth while on the severely restricted diet. One such IEM called Maple Syrup Urine Disorder (MSUD) can cause death if a similar diet is not adhered to. Medical foods, which cost on average 10 times more than regular foods, are not regulated by the Canadian Food Inspection Agency, but instead by Health Canada as medical treatment.

"We commend B.C., and particularly the leadership of Health Minister Terry Lake, for recognizing low-protein medical foods as a key treatment to improve IEM health outcomes. This is a decision that will go far to help the children and adults in this province who work hard each day to protect their brains and lives from these diseases," says Nicole Pallone, from Sparwood, B.C., who is vice president of CanPKU and mother to a five-year-old daughter with PKU. "This announcement marks B.C.'s most significant improvement in funding for PKU treatments in 50 years – and our community is so thankful to all who contributed to this decision."

According to the Ministry of Health, each patient in B.C. who requires this treatment will be entitled to a subsidy of \$250 per month to spend on special medical foods, starting January 1, 2014. Prior to this decision, B.C. only provided a nominal \$40 monthly stipend for foods, which was only available to patients on social assistance. Aside from medical foods, B.C. IEM patients receive synthetic amino acid formulas which continue to form the basis of the complicated medical diet. These formulas are high in protein, vitamins and minerals, but are specifically manufactured to exclude the amino acids that cause harm to patients with each condition.

Access to third treatment in PKU *Comprehensive Brain Protection Strategy* still needed

While this funding decision marks a monumental step forward for PKU treatment in the province, one critical treatment remains inaccessible to patients who depend on B.C. Pharmacare. Kuvan (sapropterin dihydrochloride), the first and only Health Canada approved drug therapy for PKUⁱ, is now funded in Ontario and Saskatchewan, as well as Quebec where the government funds Kuvan on a case-by-case basis for women with PKU who are pregnant or plan to become pregnant.ⁱⁱ Kuvan is covered for some patients with private health insurance.

Following the approval of Kuvan in 2010, negotiations with the drug's manufacturer began with the drug programs in Ontario, Saskatchewan and B.C. Much to the disappointment of PKU patients in B.C., it was the only province to walk away from these negotiations. It remains unclear to the patient community why B.C. funds Kuvan to treat BH4 deficiency (originally called malignant PKU) and as a diagnostic tool to determine whether newborns with high Phe levels have PKU or BH4 deficiency, but not for its Health Canada approved indication to treat PKU.

"It is our sincere hope that B.C. continues to improve PKU patients' health outcomes by providing access to the remaining essential treatment tool – Kuvan – to ensure that the brains of adults, adolescents and children are protected," says John Adams, President and CEO of CanPKU, whose adult son has PKU and has been successfully treated with Kuvan for more than six years. "We are grateful to the Ministry of Health for its renewed support for patients and families in B.C., but our mutual work is not yet done to bring treatment of PKU to national and international standards."

To date, Kuvan is publicly funded and accessible to patients with PKU in Austria, Belgium, Denmark, France, Germany, Greece, Italy, Japan, Netherlands, Norway, Slovakia, Spain, Switzerland and the United States.ⁱⁱⁱ

About PKU

PKU (phenylketonuria) is a rare inherited, brain-threatening metabolic disorder, observed when the body is unable to process phenylalanine ("Phe"), an essential amino acid found in dietary protein. The resulting accumulation of Phe in the blood is toxic to the brain, and if left untreated, symptoms can range from mild cognitive impairment to severe mental retardation. Approximately 1 in 12,000 to 15,000 infants in Canada is born with PKU. All provinces and territories, including B.C., offer newborn screening tests to determine if a child is born with PKU. If PKU is detected, the appropriate treatment must be initiated immediately and maintained throughout life to ensure normal brain development.

About Canadian PKU and Allied Disorders Inc.

Canadian PKU and Allied Disorders Inc. is a non-profit association of volunteers, dedicated to providing accurate news, information and support to families and professionals dealing with PKU and similar, rare, inherited metabolic disorders. Our mission is to improve the lives of people with PKU and allied disorders and the lives of their families. By allied disorders we mean other rare, inherited metabolic disorders also detected by newborn screening. For more information, visit www.canpku.org and download our comprehensive resource for patients and families, [PKU and the Brain](#).

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-30-

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ⁱⁱⁱ Kuvan in Europe. The European Society for Phenylketonuria and Allied Disorders Treated as Phenylketonuria.

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