Gmail **Tanya Chute <tanya.chute@canpku.org>**

CanPKU+ January 2024 Newsletter

Canadian PKU and Allied Disorders <info@canpku.org> Sat, Jan 20, 2024 at 12:19 AM
Reply-To: Newseditor - Marjorie Guhl <newseditor@canpku.org>
To: Krystal Austin <krystal.austin@cencora.com>
Cc: Krystal Austin <tanya.chute@canpku.org>

Dear Readers,

As we reflect on the journey we've shared in the past year, we are grateful for your continued support. Your engagement with CanPKU+ is a driving force behind our efforts to bring you informative, inspiring, and educational content.

Thank you for being an integral part of the CanPKU+ community. We look forward to another year of shared experiences and meaningful connections. Here's to a fantastic year ahead!

Wishing you and your loved ones a joyful, prosperous, and Happy New Year!

Warm regards,

Marjorie Guhl
CanPKU+ Newseditor

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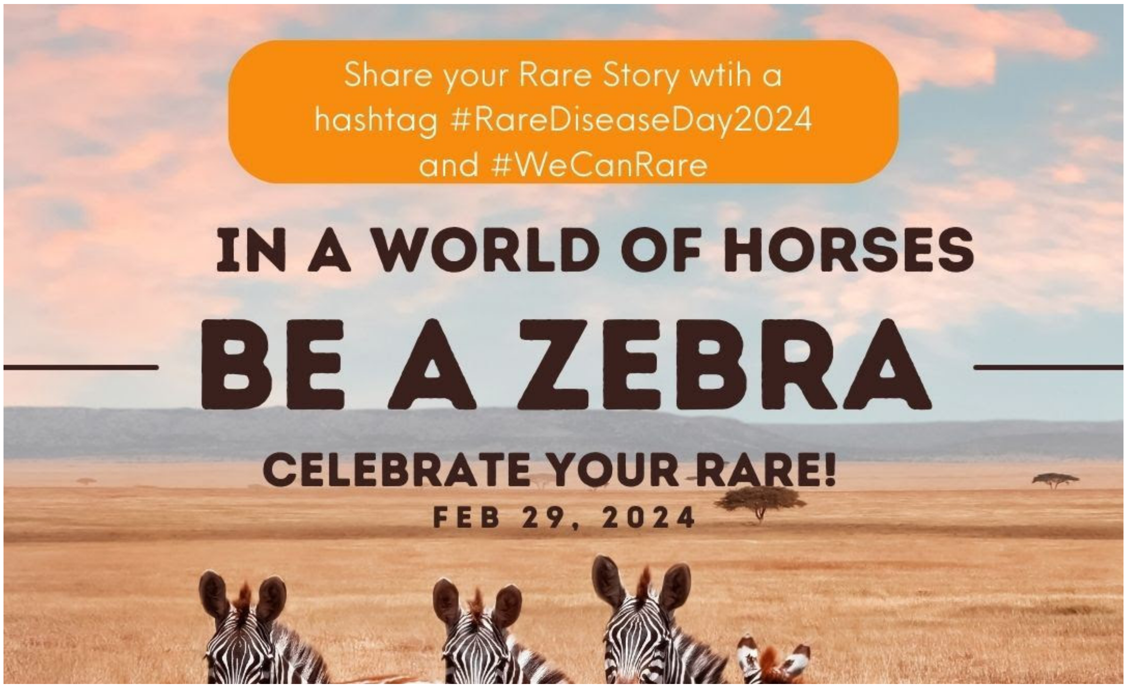
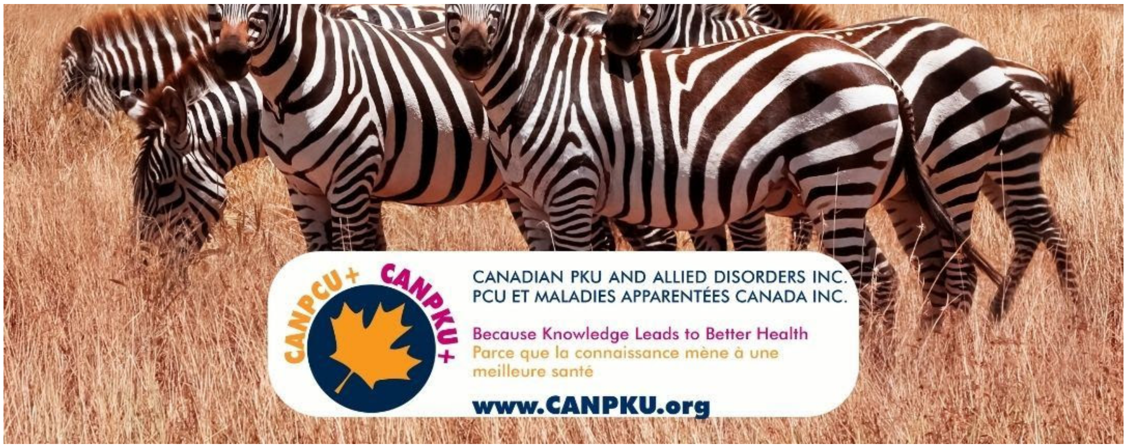
Canadian News

CanPKU+ Sponsors

We moved our Date to Mar 8 - 10, 2024 for camp MagniPHEque due to low registration. We thank those who have registered and for your continued support.

Click for more info



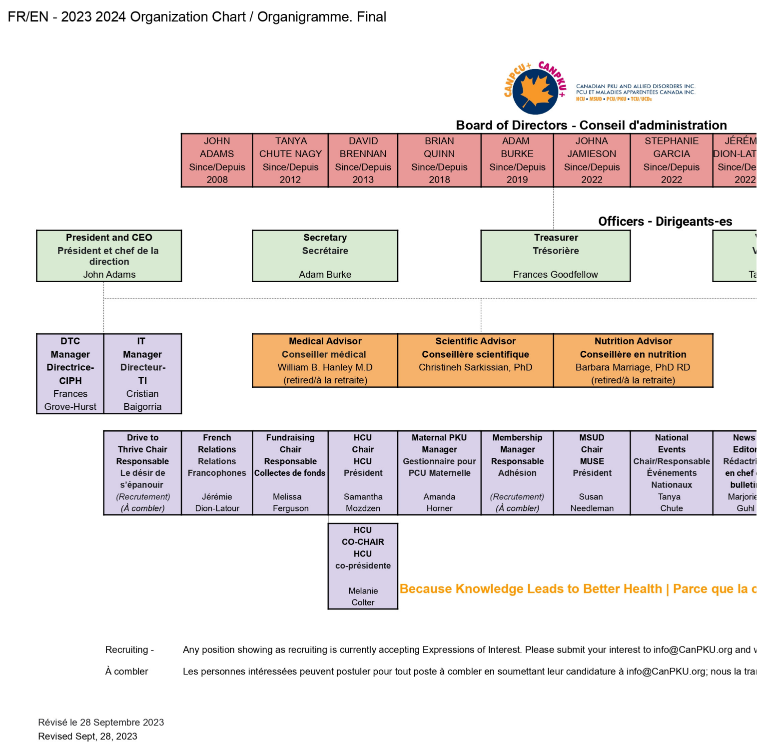


As we look ahead to celebrating Rare Disease day, we also look forward to seeing how you celebrate or recognize the day. If you share to social media, consider using the hashtag #WeCanRare and #RareDiseaseDay2024.

CanPKU+ feels it is important to represent our community using REAL faces of those who are affected by each disorder, where we can. If you would like to be featured on our promotional or educational materials, flyers, website and more here is how!

1. Complete a RARE FACES photo release
2. Grab a favorite photo of the person with Rare in your household
3. Email them both to info@canpku.org

Then keep your eye out and you may just see yourself featured online or in print on our materials!



FR/EN - 2023 2024 Organization Chart / Organigramme. Final

CANADIAN PKU AND ALLIED DISORDERS INC.
PCU ET MALADIES APPARENTÉES CANADA INC.
HCU • MSUD • PCU/PKU • TCU/UCDs

Board of Directors - Conseil d'administration

JOHN ADAMS Since/Depuis 2008	TANYA CHUTE NAGY Since/Depuis 2012	DAVID BRENNAN Since/Depuis 2013	BRIAN QUINN Since/Depuis 2018	ADAM BURKE Since/Depuis 2019	JOHNA JAMIESON Since/Depuis 2022	STEPHANIE GARCIA Since/Depuis 2022	JÉRÉM DION-LAT Since/De 2022

Officers - Dirigeants-es

President and CEO **Président et chef de la direction** John Adams	**Secretary** **Secrétaire** Adam Burke	**Treasurer** **Trésorière** Frances Goodfellow	**V** **V** Ta

DTC Manager **Directrice-CIPH** Frances Grove-Hurst	**IT Manager** **Directeur-TI** Cristian Baigorria

Medical Advisor **Conseiller médical** William B. Hanley M.D (retired/à la retraite)	**Scientific Advisor** **Conseillère scientifique** Christineh Sarkissian, PhD	**Nutrition Advisor** **Conseillère en nutrition** Barbara Marriage, PhD RD (retired/à la retraite)

Drive to Thrive Chair **Responsable Le désir de s'épanouir** *(Recrutement)* *(À combler)*	**French Relations** **Relations Francophones** Jérémie Dion-Latour	**Fundraising Chair** **Responsable Collectes de fonds** Melissa Ferguson	**HCU Chair** **HCU Président** Samantha Mozdzen	**Maternal PKU Manager** **Gestionnaire pour PCU Maternelle** Amanda Horner	**Membership Manager** **Responsable Adhésion** *(Recrutement)* *(À combler)*	**MSUD Chair** **MUSE Président** Susan Needleman	**National Events Chair/Responsable Événements Nationaux** Tanya Chute	**News Editor** **Rédactri en chef bulleti** Marjorie Guhl
			HCU CO-CHAIR **HCU co-présidente** Melanie Colter					

Because Knowledge Leads to Better Health | Parce que la c

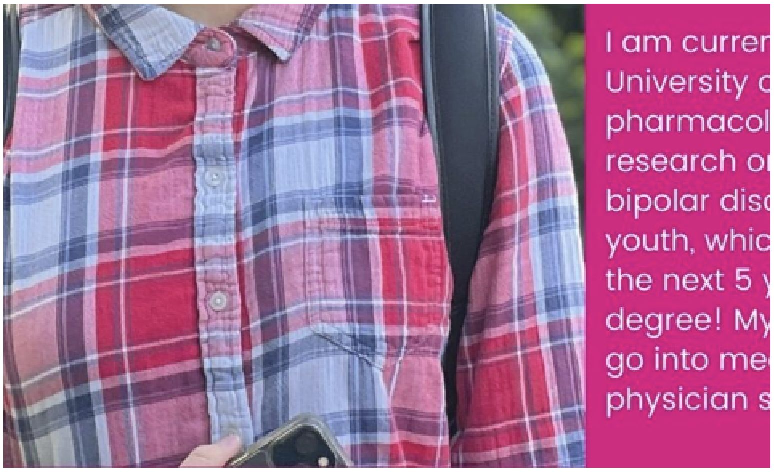
Recruiting - Any position showing as recruiting is currently accepting Expressions of Interest. Please submit your interest to info@CanPKU.org and w

À combler Les personnes intéressées peuvent postuler pour tout poste à combler en soumettant leur candidature à info@CanPKU.org; nous la tra

Révisé le 28 Septembre 2023
Revised Sept, 28, 2023

Medical Student Volunteer

CanPKU+ has been very fortuntate to be able to work with various Medical Students wh projects. From boots on the ground at events to updating documents and helping to sol We want to provide recognition for their pa



I am curren
University o
pharmacol
research o
bipolar diso
youth, whic
the next 5 y
degree! My
go into me
physician s

Connect on LinkedIn

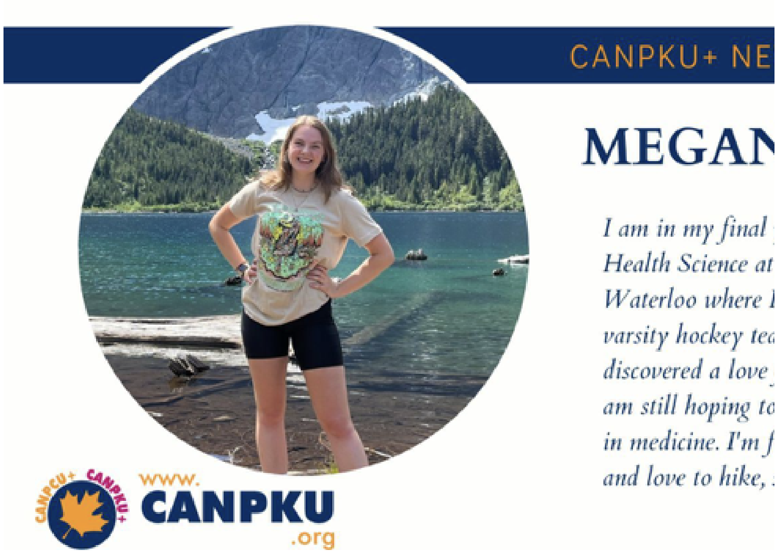
Currently interested in or working on the following:

Nationwide HCU Newborn Screening

Creation of products and resources for those living with Rare

Previously worked on:

Classwork - Updated PKU and the Brain's Treatment Pages



MEGAN

I am in my final
Health Science at
Waterloo where I
varsity hockey tea
discovered a love
am still hoping to
in medicine. I'm f
and love to hike,

Connect on LinkedIn:

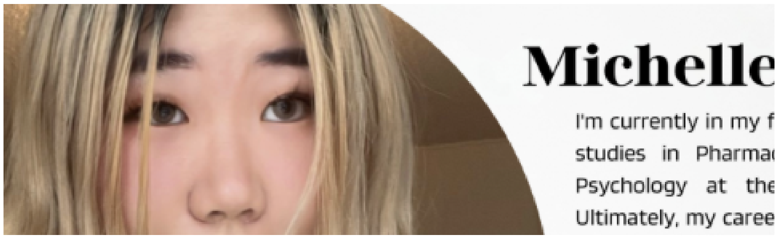
Currently interested in or working on the following:

Nationwide HCU Newborn Screening

Drive to Thrive - CME Accredited webinar

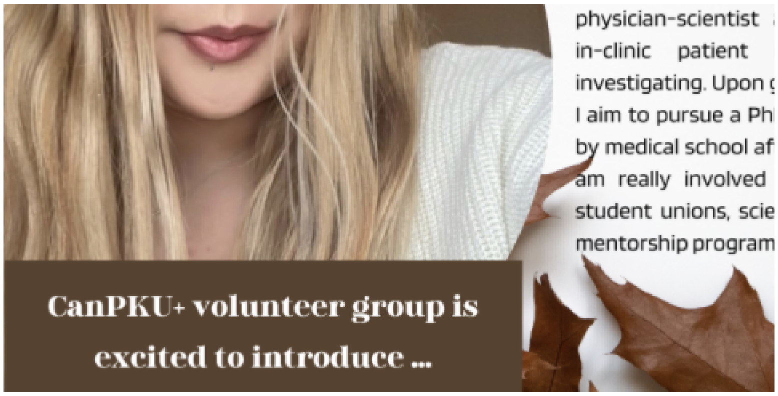
Previously worked on:

Classwork - Nationwide HCU Newborn Screening LaunchPad



Michelle

I'm currently in my f
studies in Pharma
Psychology at the
Ultimately, my caree


physician-scientist
in-clinic patient
investigating. Upon
I aim to pursue a Ph
by medical school af
am really involved
student unions, scie
mentorship program

CanPKU+ volunteer group is excited to introduce ...

Connect on LinkedIn

Currently interested in or working on the following:

Drive to Thrive - CME Accreditated webinar

Previously worked on:

Classwork - Drive to Thrive - CME Accreditated webinar LaunchPad


EUGENE ALFONZO II A

School: Und
Pharmacolo
the Universi

Potential Pe
- Work fo
Canada
Review
- Manage
part of a
team
- Work as
officer f
pharmac
compan

Connect on LinkedIn

Currently interested in or working on the following:

CanPKU's submission to Health Canada to contribute to the regulatory modernization of foods cat

Previously worked on:

Team lead for classwork on creation of Roadmap for Drug Development

I recently complet
Life Sciences at C
am excited to sta
healthcare field. I
opportunity to wo
research at The H
Children and worl
youth through me
am sincerely than
opportunity to vol
and continue to w
wonderful commu

Connect on LinkedIn

Currently interested in or working on the following:

Camp MagniPHEque

Previously worked on:

Camp MagniPHEque and Regional Events

Hello! I am
Honours He
Sciences s
pre-clinica
specializat
University
am passion
patient car

Connect on LinkedIn:

Currently interested in or working on the following:

Nationwide HCU Newborn Screening

Drive to Thrive - CME Accreditated webinar

Creating Resources for various Rares

Previously worked on:

Classwork - Nationwide HCU Newborn Screening LaunchPad

We thank those Medical Students who have worked with CanPKU+ in the pa

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Feature Sto

Mila, born in October 2018 in Canada, entered a challenging journey with Homocystinuria (HCU) just a week after her birth. Her parents received a phone call revealing her diagnosis of "Classical Homocystinuria," prompting a week-long stay at British Columbia Children’s Hospital. A dedicated medical team worked to stabilize Mila's elevated levels, creating a treatment plan involving B6, Folic Acid, Betaine, formula, and regular blood work. Despite concerns about her diet, potential developmental issues, and the social aspects of growing up with dietary restrictions, Mila adapted well to treatment, displaying resilience and spirit. Her parents emphasize the importance of support and community in navigating the challenges posed by HCU, and their goal is to ensure Mila's condition doesn't define her, allowing her vibrant personality to shine through in various aspects of life.

(Published in 2023) Shared with permission from full story here

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Recently received donations:

Joyce Swain

Rebecca Weatherhead

Sylvia Beairsto

Monika Joerg-Pfiffner

Melanie Colter

Bev Goorts

Paul Pietrkiewicz

Jérémie Dion-Latour

Rahm Glezos

Sherry Guhl

Cynthia Kushnier

Rebecca Goorts

Connie Cook

Barbara Marriage

Colleen O'Connell-Campbell

Canada Life Reinsurance in support of Victoria who has been impacted by PKU

Mary Cavanagh

2023 Giving Tuesday Update!

For the second year in a row we participated in Giving Tuesday. Giving Tuesday is a worldwide generosity movement. On the last Tuesday of November people from around the globe donate to causes that are near and dear to their hearts. We raised over $1400 this year and are beyond thankful to every single person who donated, shared our social media posts, and sent us words of encouragement! Without all of your support, this would not be possible. The money raised goes directly back into the CanPKU+ community to support our members and their families. Thank you again to everyone who supported this fundraising initiative.

Fundraising:

CanPKU+ is always looking for new and innovative fundraising ideas. If you or someone you know has a great fundraising idea, please reach out to Melissa at fundraising@canpku.org.

If you are interested in hosting a fundraiser, Spare for Rare is an awesome way to have a fun few hours of bowling at your local bowling Centre, and raise money for CanPKU+. You can find more information about hosting a Spare for Rare event on our website under the Events Tab then Fundraising, or you can reach out to our Fundraising Chair, Melissa.

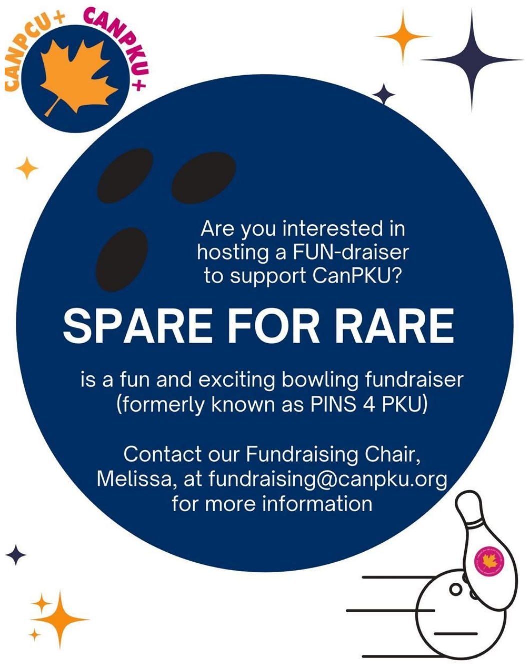
WOULD YOU LIKE TO HOST A FUN BOWLING EVENT AND SUPPORT CANPKU+ AT THE SAME TIME? THEN LET'S TALK ABOUT

SPARE FOR RARE!

CONTACT MELISSA AT
FUNDRAISING@CANPKU.ORG
TO LEARN MORE ABOUT THIS AMAZING FUNDRAISING OPPORTUNITY

CanPKU+ is looking for people who are willing to oversee a Spare for Rare event in your community in May. We will supply support; from setting up the event, advertising, pledge sheets etc.

Please contact Melissa about this exciting opportunity to support the CanPKU+ Community



Are you interested in
hosting a FUN-draiser
to support CanPKU?

SPARE FOR RARE

is a fun and exciting bowling fundraiser
(formerly known as PINS 4 PKU)

Contact our Fundraising Chair,
Melissa, at fundraising@canpku.org
for more information

Êtes-vous intéressé(e) par
l'organisation d'une collecte
de fonds amusante pour
soutenir CanPCU+ ?

ÉPARGNER POUR LE RARE.

Une collecte de fonds de bowling
amusante et excitante (anterieurement
connue sous le nom de PINS 4 PCU)

Contactez notre présidente des
collectes de fonds, Melissa, à
fundraising@canpku.org pour plus
d'informations.

HOW DO I HOST A "SPARE FOR RARE" EVENT?

CONTACT CANPKU

- REACH OUT VIA EMAIL TO OUR FUNDRIASING CHAIR, MELISSA AT FUNDRAISING@CANPKU.ORG FOR MORE INFORMATION AND ASSISTANCE WITH PLANNING THE EVENT

DEVELOP THE CONCEPT

- WILL YOU HAVE A THEME
- WILL THERE BE PRIZES?
- WILL YOU GET THE BOWLING LANES SPONSORED?
- HOW MANY TEAMS/PEOPLE WILL/CAN ATTEND?
- WE CAN HELP!

FIND A VENUE

- FIND A BOWLING CENTER TO HOST YOUR EVENT
- CHOOSE A DATE THAT WORKS FOR YOU AND THE FACILITY
- CHOOSE A TIME FRAME THAT WORKS ON THE CHOSEN DAY

SPREAD THE WORD

- POST ON SOCIAL MEDIA
- HAVE FRIENDS AND FAMILY POST ON SOCIAL MEDIA
- PUT FLYERS UP IN LOCAL BUSINESSES
- POST IN LOCAL SOCIAL MEDIA GROUPS
- WE CAN HELP!

FUNDRAISE

- ASK LOCAL BUSINESSES TO SPONSOR LANES
- HAVE EVENT PARTICIPANTS COLLECT DONATIONS
- YOU COULD CONSIDER HAVING A SILENT AUCTION WITH DONATED PRIZES TO RAISE MONEY AT THE EVENT

THE EVENT

- THE DAY OF THE EVENT IS A TIME FOR FUN!
- REMEMBER TO ENJOY YOURSELF, YOU'VE WORKED HARD ON THIS!
- TAKE PHOTOS AND VIDEOS TO SHARE WITH CANPKU FOR OUR SOCIAL MEDIA PAGES

COMMENT HÉBERGER UN EVÈNEMENT "REPARE POUR RARE"?

CONTACTER CANPKU

- CONTACTER PAR E-MAIL NOTRE PRÉSIDENTE DE LA COLLECTE DE FONDS, MELISSA À FUNDRAISING@CANPKU.ORG POUR PLUS D'INFORMATIONS ET DE L'AIDE POUR PLANIFIER L'ÉVÉNEMENT

DÉVELOPPER LE CONCEPT

- AUREZ-VOUS UN THÈME
- Y AURA-T-IL DES PRIX?
- FEREZ-VOUS SPONSORISER LES PISTES DE BOWLING ?
- COMBIEN D'ÉQUIPES/DE PERSONNES PARTICIPERONT/POURRONT PARTICIPER ?
- NOUS POUVONS AIDER!

TROUVER UN LIEU

- TROUVER UN CENTRE DE BOWLING POUR ACCUEILLIR VOTRE ÉVÉNEMENT
- CHOISISSEZ UNE DATE QUI CONVIENT À VOUS ET À L'ÉTABLISSEMENT
- CHOISISSEZ UNE PLAGE HORAIRE QUI FONCTIONNE LE JOUR CHOISI

FAIRE CONNAITRE

- PUBLIER SUR LES RÉSEAUX SOCIAUX
- DEMANDEZ À VOS AMIS ET À VOTRE FAMILLE DE PUBLIER SUR LES RÉSEAUX SOCIAUX
- METTRE DES FLYERS DANS LES COMMERCES LOCAUX
- PUBLIER DANS DES GROUPES DE MÉDIAS SOCIAUX LOCAUX
- NOUS POUVONS AIDER!

LEVER DES FONDS

- DEMANDER AUX ENTREPRISES LOCALES DE PARRAINER DES VOIES
- DEMANDER AUX PARTICIPANTS À L'ÉVÉNEMENT DE RECUEILLIR DES DONS
- VOUS POURRIEZ ENVISAGER D'ORGANISER UN ENCAN SILENCIEUX AVEC DES PRIX DONNÉS POUR AMASSER DES FONDS LORS DE L'ÉVÉNEMENT

L'ÉVÉNEMENT

- LE JOUR DE L'ÉVÉNEMENT EST UN MOMENT DE PLAISIR !
- N'OUBLIEZ PAS DE VOUS AMUSER, VOUS AVEZ TRAVAILLÉ DUR LÀ-DESSUS !
- PRENDRE DES PHOTOS ET DES VIDÉOS À PARTAGER AVEC CANPKU POUR NOS PAGES DE MÉDIAS SOCIAUX

Join the U Crew today at CanPKU+ Open for all adults living with rare who need a listening ear, a place to talk, support each other, learn new recipes, design tools to succeed. All rare disorders are welcome (HCU,MSUD,PKU, UCDs etc)

Link: www.canpku.org/ucrewinvite

If you are looking for support with getting and staying on track, new meal ideas, a friend who understands, someone to listen, workshops, meet ups, and group activities... this group is for you!

TikTok

We're on TikTok!

Click here to find us!

Guthrie-Koch Scholarship Program

The Guthrie-Koch Scholarship Program was founded in 1997 to recognize outstanding young adults with PKU pursuing higher education and provide financial support to these efforts.

Are you or do you know a high-school senior or current student pursuing an undergraduate degree or technical school with Classical HCU, MSUD, an Organic Acidemia, PKU, Tyrosinemia, or a Urea Cycle Disorder? Applications for the 2024-2025 academic year are now being accepted at scholars.flok.org. The application deadline is March 15, 2024. scholars.flok.org

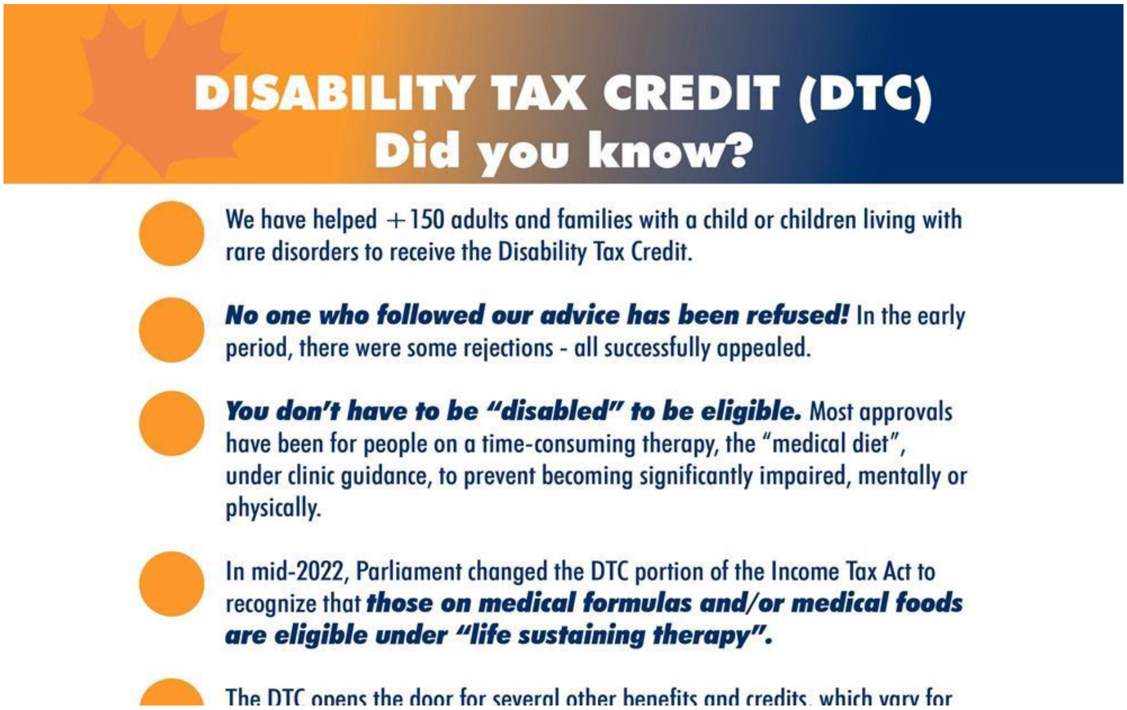
flok

www.CANPKU.org

Disabilty
TAX CREDIT

Does your dietary therapy
qualify you for the
Canadian Disability Tax Credit?

https://www.canpku.org/Disability-Tax-Credit



DISABILITY TAX CREDIT (DTC)
Did you know?

- We have helped +150 adults and families with a child or children living with rare disorders to receive the Disability Tax Credit.
- ***No one who followed our advice has been refused!*** In the early period, there were some rejections - all successfully appealed.
- ***You don't have to be "disabled" to be eligible.*** Most approvals have been for people on a time-consuming therapy, the "medical diet", under clinic guidance, to prevent becoming significantly impaired, mentally or physically.
- In mid-2022, Parliament changed the DTC portion of the Income Tax Act to recognize that ***those on medical formulas and/or medical foods are eligible under "life sustaining therapy".***
- The DTC opens the door for several other benefits and credits, which vary for

children or adults.

The DTC can be made ***retroactive for up to ten years.*** ***<u>You may get a bunch of tax rebates!</u>***

Clinics and other patient groups are asking for our help with DTC applications for other conditions... and we are helping them get approved!

Have you applied for the DTC?
What are you waiting for?
We can help!

L'autre côté français.

www.CanPKU.org
Because Knowledge Leads to Better Health

CANADIAN PKU AND ALLIED DISORDERS INC. **PCU ET MALADIES APPARENTÉES CANADA INC.**

DTC2023_03

If you've had a successful DTC application, we'd love to hear from you!

Please connect with Marjorie, our CanPKU+ news editor at newseditor@canpku.org. so we can feature you and your story in our next issue.

CRÉDIT D'IMPÔT POUR PERSONNES HANDICAPÉES (CIPH)
Le saviez-vous ?

Nous avons aidé plus de 150 adultes et familles avec un enfant ou des enfants vivant avec la PCU à demander et à recevoir le crédit d'impôt pour personnes handicapées.

Aucune personne ayant suivi nos conseils n'a été refusée! La plupart des demandes approuvées concernaient des personnes qui suivent, sous supervision médicale, un régime alimentaire exigeant en temps, qui vise à prévenir les déficiences physiques ou intellectuelles.

Au milieu de 2022, le Parlement a modifié la partie sur le CIPH de la Loi de l'impôt sur le revenu afin de reconnaître ***que les personnes qui consomment des formules médicales et/ou des aliments médicaux sont admissibles dans le cadre de la « thérapie de maintien de la vie ».***

L'admissibilité au CIPH ouvre la porte à plusieurs autres avantages et crédits qui varient pour les enfants ou les adultes. Le DTC peut devenir ***rétroactif jusqu'à dix ans. Vous pourriez bénéficier de nombreuses remises fiscales!***

Les cliniques et autres groupes de patients demandent notre aide pour les demandes de CIPH pour des personnes souffrant d'autres conditions, et nous aidons à les faire approuver!

Avez-vous demandé le CIPH?
Qu'attendez-vous?
Nous pouvons aider!
English on other side.

www.CanPKU.org
Parce que la connaissance mène à une meilleure santé

CANADIAN PKU AND ALLIED DISORDERS INC. PCU ET MALADIES APPARENTÉES CANADA INC.

DTC2023_03

synlogic

Happy new year from Synlogic! The Synpheny-3 is a clinical trial enrolling **now.** Synpheny-3 is a clinical trial for adults living with PKU who are unable to maintain blood Phe levels below 360 micromol/L. Canadians can participate in any of the locations listed below, including a few new sites that have opened recently:

Calgary, Alberta: MAGIC Clinic

- Principal Investigator: Aneal Khan, MD

Hamilton, Ontario: Hamilton Health Sciences Corporation

- Principal Investigator: Murray Potter, MD

Ottawa, Ontario: Children's Hospital of Eastern Ontario

- Principal Investigator: Danielle Bourque, MD

Toronto, Ontario: University Health Network

- Principal Investigator: Graeme Nimmo, MD

Different trial formats may be available depending on the site. **Have questions?** Complete the questionnaire on www.pkuresearchstudy.com to speak with Leyla, Synlogic's Clinical Case Manager.

COME JOIN US!!!

FEBRUARY 29, 2024

9:00AM - 12:00PM

Calgary Central Library
800 3 Street SE
Calgary AB, T2G 2E7

INFO@RDNA.CA

Raising Awareness through Diagnosis and Treatment!

CALGARY, AB

RARE DISEASE DAY 2024

Cick for more info!

University of Manitoba | Rady Faculty of Health Sciences

Parents and Caregivers Needed

Do you have a child with a rare genetic diagnosis or is suspected to have a rare genetic diagnosis?

We want to hear from you!

Our Goal
Understand the experiences, needs, and preferences among Canadian caregivers during their child's diagnostic journey

Your Role
Check your eligibility using our screening survey, then complete an anonymous 15-20 minute online survey

Who Can Participate
Caregivers whose child(ren) have gotten a genetic diagnosis **OR** genetic test for a suspected genetic condition in 2018 or later

OR GO TO:
www.redcap.link/rd-caregiver

Scan for Screening Survey

Eligible participants can be entered a Gift-Card draw in return for their participation

If you would like a direct link to the survey or more information on this study opportunity, please email **Vivien** at **truongv2@myumanitoba.ca**

This study has been approved by the Bannatyne Health Research Board: **HS26049**

University of Manitoba | Rady Faculty of Health Sciences

Besoin de parents et de

soignants

Avez-vous un(e) enfant atteint(e) d'un diagnostic génétique rare ou chez lequel(laquelle) un diagnostic génétique rare est soupçonné?

Nous voulons connaître votre expérience!

Notre Objectif

Comprendre les expériences, les besoins et les préférences des soignants canadiens durant le parcours diagnostique de leur enfant.

Votre Rôle

Vérifiez votre éligibilité à l'aide de notre enquête de sélection, puis répondez à une enquête en ligne anonyme de 15 à 20 minutes

Qui Peut Participer

Les soignants dont l'enfant/les enfants a/ont reçu un diagnostic génétique ou un test génétique pour une maladie génétique soupçonnée en 2018 ou plus tard.

OU VISITEZ:
www.redcap.link/rd-caregiver

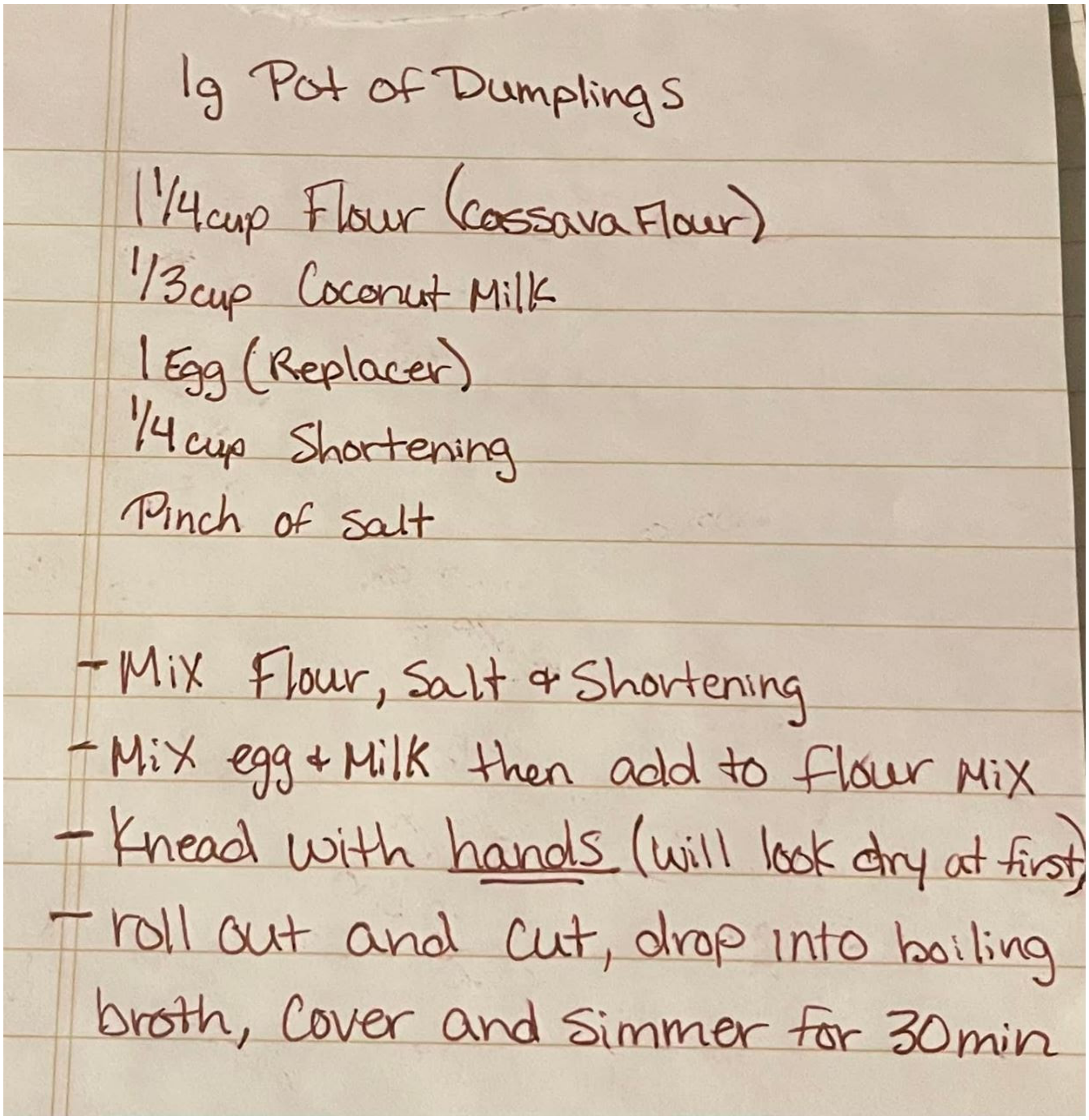
Numérisez pour l'enquête de sélection

Les participants éligibles peuvent participer au tirage au sort d'une carte-cadeau en échange de leur participation.

Si vous souhaitez obtenir un lien direct vers l'enquête ou plus de renseignements sur cette occasion d'étude, veuillez envoyer un courriel à **Vivien** à l'adresse suivante **truongv2@myumanitoba.ca**

Étude approuvée par le Comité d'éthique pour la recherche du campus Bannatyne: **HS26049**

Feature Recipe



lg Pot of Dumplings

1 1/4 cup Flour (cassava Flour)
1/3 cup Coconut Milk
1 Egg (Replacer)
1/4 cup Shortening
Pinch of salt

- Mix Flour, Salt & Shortening
- Mix egg + Milk then add to flour Mix
- Knead with <u>hands</u> (will look dry at first)
- roll out and cut, drop into boiling broth, Cover and Simmer for 30 min

Thank you to Jessica from the *PKU Worldwide Support Group* on Facebook for this recipe! With the recent cold snap across Canada, a recipe like this is perfect for those cold days. Such a versatile recipe. Add it to soups and more.

Thank you for sharing, Jessica!

Updates and Articles of Interest

PTC Therapeutics to Present Recent Data for Sepiapterin at the Society for the Study of Inborn Errors of Metabolism (SSIEM) Annual Symposium

https://www.globenewswire.com/news-release/2024/01/04/2803824/0/en/Synlogic-Provides-Corporate-Update-and-Outlook-for-2024.html

Pharmac Expands Funded Supplement Options For People With Inherited Metabolic Diseases

https://www.scoop.co.nz/stories/GE2401/S00005/pharmac-expands-funded-supplement-options-for-people-with-inherited-metabolic-diseases.htm

Revolutionizing treatment: Tandem Bio taps into placental cell power to combat rare metabolic disorders

Christianeh Sarkissian, PhD, mentioned here, also volunteers as a scientific advisor to CanPKU+ and has for many years.

https://www.digitaljournal.com/tech-science/revolutionizing-treatment-tandem-bio-taps-into-placental-cell-power-to-combat-rare-metabolic-disorders/articlehttps://www.linkedin.com/posts/christinehsarkissian_revolutionizing-treatment-tandem-bio-taps-activity-7150878423555391488-hGV3?utm_source=share&utm_medium=member_ios

Canadian News

Opinion: Spend less on dental care and pharmacare, more on treatments for rare medical disorders

Three million Canadians suffer from rare illnesses. Although drugs exist for some of them, they are often prohibitively expensive

Nigel Rawson and John Adams, Special to Financial Post

https://financialpost.com/opinion/opinion-spend-less-on-dental-care-and-pharmacare-more-on-treatments-for-rare-medical-disordershttps://www.pennmedicine.org/news/news-releases/2023/august/penn-team-seeks-to-create-treatments-for-3-rare-genetic-diseases

ATTENTION THOSE LIVING IN BC!!

WE NEED YOUR HELP!!

CanPKU+ is joining The Gastrointestinal Society, as a member of the MedAccessBC Coalition, unveiled a groundbreaking analysis shedding light on the concerning inequity in British Columbia's public drug plan. The findings underscore that BC not only allocates the least per capita but also provides coverage for fewer prescription medicines compared with all other provinces in Canada.

While provinces nationwide have greenlit numerous medications in their public drug plans, BC stands apart by denying coverage for crucial treatments. This denial has far-reaching consequences for patients grappling with conditions such as acne, bone fractures, migraines, multiple sclerosis, schizophrenia, short bowel syndrome, skin diseases, and more. Those affected are left with limited alternatives, unless equipped with private drug insurance, the financial means for out-of-pocket expenses, or the ability to relocate to another province.

More information on how you can help and join the efforts can be found at canpku.org/MedAccess-BC

MedAccess BC

Excerpt from the Garrod Association Newsletter, December 2023

TORONTO, CANADA's bid to host the ICIEM 2029
16th International Congress on Inborn Errors of Metabolism
Monday – Friday, August 27 to 31, 2029

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DESTINATION TORONTO

In partnership with:

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ICIEM 2029 Bid Chair
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Medical Director, Newborn Screening
The Hospital for Sick Children
Professor, Departments of Paediatrics and Biochemistry
University of Toronto
E: andreas.schulze@sickkids.ca

SickKids THE HOSPITAL FOR SICK CHILDREN

TEMERTY FACULTY OF MEDICINE UNIVERSITY OF TORONTO

Please mark your calendars for 2029!

Six years from now, the 16th International Congress on Inborn Errors of Metabolism (ICIEM) will take place in Toronto, August 27-31, 2029.

Earlier this year, Dr. Andreas Schulze decided to place a bid to host the ICIEM in 2029 in Toronto.

The ICIEM brings together key opinion leaders in the field of Inborn Errors of Metabolism from all over the world. The meeting is held every four years.

It is a joint meeting of the members of the continental metabolic societies - SSIEM (Europe), SIMD (North America), ASIEM (Australasia), JSIMD (Japan), and SLEIMPN (Latin America).
The continental societies alternate in hosting the ICIEM event;
in 2029, it is SIMD's turn.

The SIMD executive agreed to the proposal to hold the event in Toronto and asked Dr. Schulze to be the lead organizer.

The proposal was enthusiastically endorsed by the Executive and Board of Directors of the Garrod Association.

"Delayed and denied: The challenges faced by Canadians with rare disorders due to ill-advised drug policies"

- **YouTube**: https://www.youtube.com/watch?v=VVun0ujKMtk
- **MLI website:** https://macdonaldlaurier.ca/event/delayed-and-denied/

John will be presenting a poster at the SIMD scientific conference in April about a rare diseases patient registries project called INFORM-RARE.

Poster abstract:

CO-DEVELOPING LONGITUDINAL PATIENT REGISTRIES FOR PHENYLKETONURIA AND MUCOPOLYSACCHARIDOSES IN CANADA

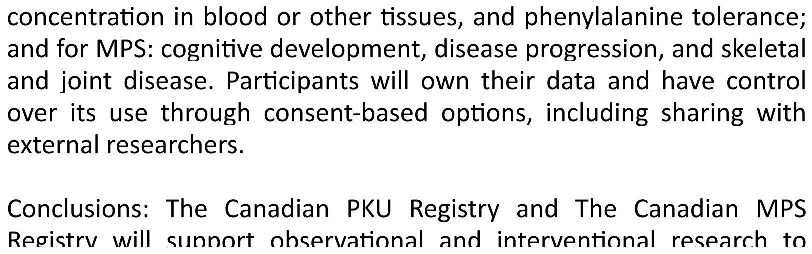
John Adams[1], Kim Angel[2], John Mitchell[3], Pranesh Chakraborty[4], Beth K Potter[5], Michal Inbar-Feigenberg[6], Sylvia Stockler[7], Monica Lamoureux[4], Alison H Howie[5], Alex Pace[4], Nancy J Butcher[6], Cheryl Rockman-Greenberg[8], Robin Hayeems[6], Anne-Marie Laberge[9], Thierry Lacaze-Masmonteil[10], Jeff Round[11], Martin Offringa[6], Maryam Oskoui[3], Andreas Schulze[6], Kathy Speechley[12], Kednapa Thavorn[13], Yannis Trakadis[3], Kumanan Wilson[13]

[1]Canadian PKU and Allied Disorders Inc., [2]The Canadian MPS Society, [3]McGill University Health Centre, [4]Children's Hospital of Eastern Ontario, [5]University of Ottawa, [6]The Hospital for Sick Children, [7]BC Children's Hospital, [8]University of Manitoba, [9]Centre de recherche CHU Sainte-Justine, [10]University of Calgary, [11]Institute of Health Economics, [12]University of Western Ontario, [13]Ottawa Hospital Research Institute

Background: Phenylketonuria (PKU) and mucopolysaccharidoses (MPS) are inherited metabolic diseases. Given their rarity, there is a paucity of evidence to inform treatment decisions. The rigorous collection of standardized outcomes within a patient registry can facilitate research to enhance understanding of natural history and evaluate long-term intervention effectiveness; yet, such registries do not exist for Canadian PKU and MPS patients. Patient organizations – Canadian PKU and Allied Disorders Inc. and the Canadian MPS Society – partnered with the INFORM RARE research network to co-develop novel patient registries for PKU and MPS, respectively.

Methods: To facilitate a shared vision among registry partners, a core set of values was co-developed for The Canadian PKU Registry and The Canadian MPS Registry: sustainability to support long-term relevance; accessibility within and outside current networks, while maintaining participant privacy; scalability to other disease groups; recognition of the patient registries as a public benefit; direct benefit to patients and their families through access to and control over their own data; and building capacity for research that responds to patient priorities. The registries are consent-based with ethical oversight centred at the Children's Hospital of Eastern Ontario research ethics board (REB). Decisions about data collection, access, and use are made by the Canadian Metabolic Patient Registries Steering Committee, a multi-disciplinary team with experts from both disease groups. Special attention is given to factors that facilitate registry based interventional research.

Results: The patient registries are hosted on the National Organization for Rare Disorders IAMRARE® Platform and are available in English and French. Initially, eligible participants are individuals diagnosed with PKU or MPS, aged 18 years or younger, and receiving disease-specific care in Canada; expansion to adult populations is a priority for both registries. Phased data collection is informed by core outcome sets that have been developed by patients, caregivers, health care providers, and policy makers. Following consent and confirmation of eligibility, participants are asked to complete a demographic questionnaire and patient- and/or caregiver-reported questionnaires to measure children's health-related quality of life (Patient-Reported Outcome Measurement Information System) and activities of daily living (Adaptive Behavior Assessment System). Additional patient/caregiver-reported outcomes will be added as part of the phase I launch, including, for PKU: child understanding of and self-efficacy with disease management, and adherence to treatment; and for MPS: pain, mobility, sleep, and caregiver/family impact. In phase II, clinician-reported data will be integrated into the patient registries with site-specific REB approvals and linked, with participant consent, to phase I data. Clinician-reported outcomes include, for PKU: overall child development and functioning, phenylalanine



concentration in blood or other tissues, and phenylalanine tolerance; and for MPS: cognitive development, disease progression, and skeletal and joint disease. Participants will own their data and have control over its use through consent-based options, including sharing with external researchers.

Conclusions: The Canadian PKU Registry and The Canadian MPS Registry will support observational and interventional research to registry will support observational and interventional research to ultimately improve care and outcomes for individuals with PKU and MPS, respectively. The framework and methods described are scalable to other rare diseases.

CanPKU+'s Drive to Thrive Committee

As mentioned earlier this year, CanPKU is committed to helping those who were previously misguided (with information that at the time was believed to be correct) that it would be ok to come off their medical diet. This may have been as the medical community believed that the brain had developed enough and would not incur further brain damage by lifting the protein restriction. It may be due to the fact some provinces restricted coverage of therapeutic products (and some still do) so it was impossible to maintain the previous "diet" therapy. Lastly, it may be due to the fact the adult opted for their own reasons to come off "diet" and stop therapy.

We now know that this is not the best course of action. We owe it to those who were previously diagnosed, but are now lost to treatment to help them return to clinic and resume treatments (which have dramatically improved since their time on therapy).

To do this, we need your help. We are looking to form a committee that will help locate and support these individuals. You do not have to be an adult with PKU, just a passion to help. We hope to have representatives from many provinces.

Are you up for it? (Can you join both committees mentioned in this newsletter - Absolutely!) Email to Tanya.Chute@canpku.org to sign up!

Email Tanya

CAMBROOKE

Glytactin COMPLETE 10 Bars are being *DISCONTINUED*

Dear Customer,

This notification is to inform you that we are discontinuing Glytactin COMPLETE 10 Bars with this estimated schedule:

Middle of February: Peanut Butter
End of March: Fruit Frenzy

Cambrooke will support those who like our bars by continuing to offer the COMPLETE 15 Bars.

COMPLETE 15 Bars are the same as COMPLETE 10 Bars in taste, nutrition and cost per bar section. The only difference is the 15 g PE Bar has **3** x 5 g PE sections and the 10 g PE Bar has **2** x 5 g PE sections.

10 GLYTACTIN COMPLETE

- Contains 2 x 5 g PE sections
- Calories: 210 - 220
- PE: 10 g
- Phe: 22 - 24 mg

15 GLYTACTIN COMPLETE

- Contains 3 x 5 g PE sections
- Calories: 320 - 330
- PE: 15 g
- Phe: 32 - 37 mg

PRODUCT INFO & INGREDIENTS

We are here to support you. If you have any additional questions, contact **Customer Service** at **866-456-9776, option # 2** or **info@cambrooke.com.**

CAMBROOKE

Glytactin COMPLETE 10 des barres *ARRÊT DE PRODUCTION*

Chers clients,

Cette notification est pour vous faire part que nous discontinuons les Barres Glytactin COMPLETE 10 selon l'échéancier estimatif suivant:

Mi-février : Beurre d'arachide
Fin mars : Frénésie de fruits

Cambrooke continuera à soutenir ceux qui aiment nos barres en leur proposant les barres Glytactin COMPLETE 15.

Les barres COMPLETE 15 sont identiques aux barres COMPLETE 10 en matière de goût, d'apport nutritionnel et de prix unitaire de la barre. La seule différence, c'est que les barres de 15 g PE sont composées de **3 sections** de 5 g de PE et celles de 10 g PE comportent **2 sections** de 5 g PE.

10 GLYTACTIN COMPLETE

- Comporte 2 sections de 5 g de PE
- Calories : 210 - 220
- PE : 10 g
- Phe : 22-24 mg

15 GLYTACTIN COMPLETE

- Comporte 3 sections de 5 g de PE
- Calories : 320 - 330
- PE : 15 g
- Phe : 32 - 37 mg

INFORMATIONS ET INGRÉDIENTS DU PRODUIT

Nous sommes ici pour vous soutenir. Pour toute question supplémentaire, veuillez contacter notre **Service Client** au **866-456-9776, option # 2** ou à l'adresse électronique **info@cambrooke.com**.

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COLTS

THE NUTRITION THEY NEED,
THE TASTE THEY DESERVE

DHA
Include DHA, a nutrient typically lacking in the PKU diet

FIBERS
Each serving provides 1.5 g of soluble fiber to help meet daily fiber needs.

GREAT TASTE
PKU formula made with GMP are known for their mild taste and may help with feeling full

EASY TRANSITION
Vanilla or Plain flavor for easy transition from other Nutrica products

Only Nutricia provides GMP-based PKU formula in your choice of can or single-serve pouch

NUTRICIA

Must Be Used Under Medical Supervision

L'ALIMENTATION DONT ILS ONT BESOIN,
LE GOÛT QU'ILS MÉRITENT

ADH
Inclus de l'ADH, un nutriment important souvent manquant dans le régime alimentaire PCU

FIBRES
Chaque portion contient 1,5 g de fibres solubles pour aider à satisfaire les besoins quotidiens en fibres.

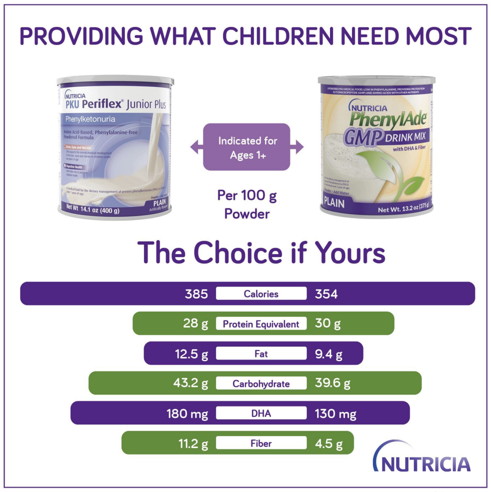
EXCELLENT GOÛT
Préparations pour la PCU contenant des glycomacropeptides (GMP) reconnues pour la douceur de leur goût et pouvant aider à se sentir rassasié.

TRANSITION FACILE
Saveur de Vanille ou Nature (sans arôme) assurant une transition facile à partir d'autres produits Nutricia

Seule Nutricia offre une préparation pour la PCU à base de GMP en boîtes ou en sachets de portion individuelle – À vous de choisir!

NUTRICIA

Doit être utilisé sous surveillance médicale



PROVIDING WHAT CHILDREN NEED MOST

Indicated for Ages 1+

Per 100 g Powder

The Choice if Yours

385	Calories	354
28 g	Protein Equivalent	30 g
12.5 g	Fat	9.4 g
43.2 g	Carbohydrate	39.6 g
180 mg	DHA	130 mg
11.2 g	Fiber	4.5 g

NUTRICIA

Must Be Used Under Medical Supervision

PROCURER L'ESSENTIEL AUX ENFANTS

Convient aux enfants de plus de 1 an

Pour 100 g de poudre

À vous de choisir

385	calories	354
28 g	équivalents en protéines	30 g
12,5 g	lipides	9,4 g
43,2 g	glucides	39,6 g
180 mg	ADH	130 mg
11,2 g	Fibres	4,5 g

NUTRICIA

Doit être utilisé sous surveillance médicale


NUTRICIA PROVIDES *two great* OPTIONS FOR CHILDREN WITH PKU
PIXIE, PKU
NUTRICIA PhenylAde GMP DRINK MIX with DHA & Fiber PLAIN
NUTRICIA PKU Periflex Junior Plus Phenylketonuria PLAIN
NUTRICIA
Must Be Used Under Medical Supervision


NUTRICA OFFRE *deux excellents* CHOIX AUX ENFANTS ATTEINTS DE PCU
PIXIE, PCU
NUTRICIA PhenylAde GMP DRINK MIX with DHA & Fiber PLAIN
NUTRICIA PKU Periflex Junior Plus Phenylketonuria PLAIN
NUTRICIA
Doit être utilisé sous surveillance médicale

RESOURCES

CanPKU+ has a number of resources which can be downloaded or printed at

www.canpku.org/Downloadable-educational-resources

Getting the most from the CanPKU website and emails!

Did you know you can get tailored emails and event invitations (like a cafe chat between folks like you! That could be a parent of a PKUer, Child, Teen or Adult with PKU!)

To do this we need some help with updating your profile.

Find out how to do this with information found at

www.canpku.org/Update-your-Profile and www.canpku.org/how-to-use-the-website

CanPKU+ Sponsors

Our sponsors are listed in the order that they began to support CanPKU+'s efforts.

This order does not represent the dollars they may contribute.

Eat Well, Live Well. AJINOMOTO.

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Sponsorship in Kind. These companies provide assistance other than financial.

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CanPKU+ News

Canadian PKU and Allied Disorders Inc. is a non-profit association of volunteers, first organized in the Spring of 2008 based in Toronto, Ontario. We are dedicated to providing accurate news, information and support to families and professionals dealing with phenylketonuria 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 the modern miracle of newborn screening.

La « Canadian PKU and Allied Disorders Inc. » est une association à but non lucratif de bénévoles créée au printemps 2008 à Toronto, en Ontario. Nous nous engageons à fournir des nouvelles, des informations et un soutien précis aux familles et aux professionnels travaillant avec la phénylcétonurie et des troubles métaboliques héréditaires rares similaires.

Notre mission est d’améliorer la vie des personnes atteintes de PCU et de troubles apparentés ainsi que la vie de leurs familles. Par troubles apparentés, nous entendons d’autres troubles métaboliques héréditaires rares, également détectés par le miracle moderne du dépistage néonatal.

“Education is the passport to the future, for tomorrow belongs to those who prepare for it today.”

—Malcolm X

Privacy Statement:

Please note that CanPKU+ maintains member/participant/registration information for its own use and does not rent, sell or otherwise provide any identifying information to outsiders.

www.canpku.org

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