

Any Need for Treatment Options?

What PKU Patients and Caregivers are Saying

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Background: Patient Survey January 2016

CanPKU conducted an online survey of patients and caregivers, using SurveyMonkey®, from Jan 7 to Jan 27, 2016. The survey was designed to gather views on what was significant to patients and caregivers about living with PKU, with the purpose to inform a patient group submission to the Common Drug Review about Kuvan.

Survey contained the use of free-form commentary, scoring options and limited closed questions

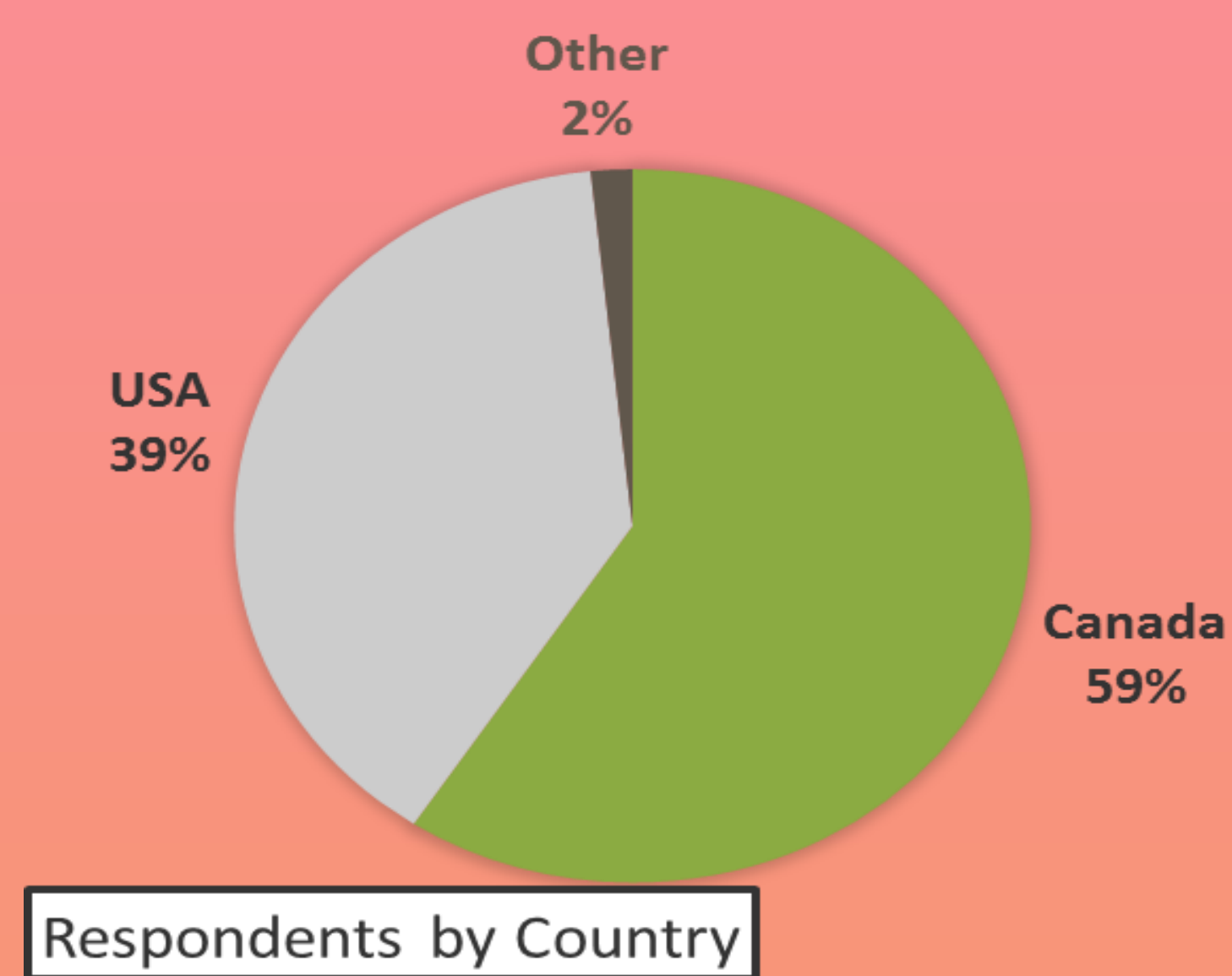
We surveyed patients and caregivers on the following:

- Patient Experience with PKU & Impact on Life
- Caregiver Experience with PKU & Impact on Life
- Dietary Restrictions and Quality of Life
- Access to Treatment Options & Experience with Kuvan
- Health Technology Assessment Standards for Drugs for Rare Disorders (DRDs)
- In addition to 30 closed-ended/scoring option questions, we also asked 5 open-ended questions resulting in 1,165 written comments.
- 297 individuals participated in the survey.

Canadian Respondents

BC: 37
AB: 26
SK: 9
MB: 10
ON: 56
QC: 7
NB: 15
NS: 7
PE: 3
NL: 1

Total 171



Ages of Patients

0-4 years of age 26.03% (76)
5-12 years of age 30.14% (88)
13-17 years of age 13.10% (38)
18-24 years of age 12.33% (36)
25+ years of age 18.49% (54)

Type of Respondent

Patient: 19% (56)
Caregiver: 73% (218)
Other*: 8% (23)

Type of PKU Represented in Survey

Mild PAH deficiency (Hyperphe) 7.89% (18)
Moderate PAH deficiency (Mild PKU) 15.35% (35)
Severe PAH deficiency (Classical PKU) 68.42% (156)
Complete PAH deficiency 0.44% (1)
I do not know 7.89 (18)

What Patients and Caregivers are Saying.....

"We don't manage Pku around our lives, we manage our lives around Pku"

"Significantly impacts my everyday work and social life. Have to plan way in advance for any activity involving food, and must sacrifice time in the evenings and on weekends preparing, cooking, packing, and ordering low protein foods"

"Social situations are extremely intensive to prepare for and I often am left out of these by others. A huge impact is the amount of time I have to spend managing my diet to protect my brain from damage..."

"Anxiety made it difficult to stay at Post Secondary school so my daughter dropped out after 3 months"

PKU Diet and PKU Management is Onerous

How has PKU had an impact on your life (or on the life of the person under your care)?

We offered 8 randomized selections, "Pick all that apply"

Answered: 196

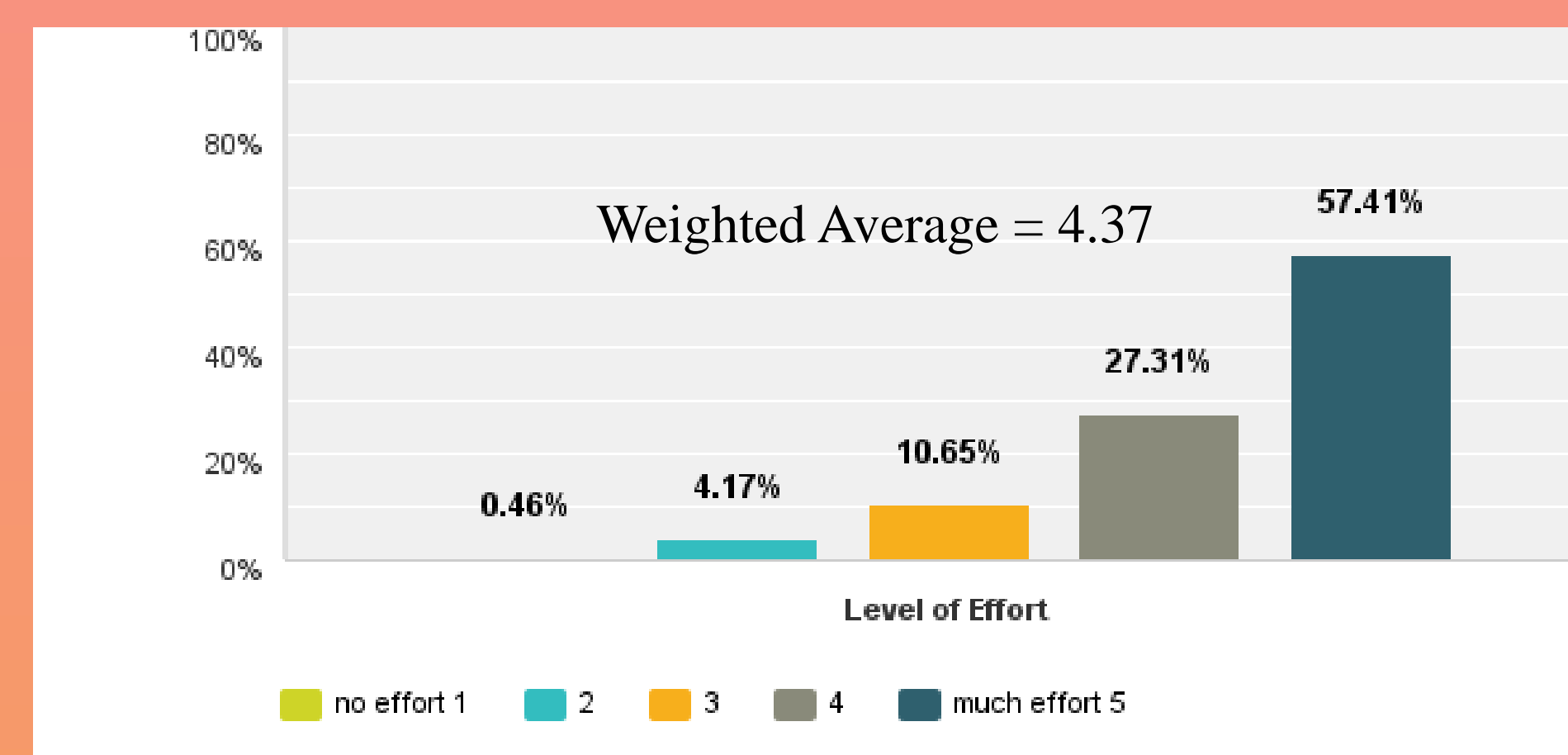
"Difficulties with diet" was selected by 71% of all respondents and was the #1 selected **Impact on life**followed by:

- Difficulty with focus/attention deficit - 50%
- Exclusion or difficulties in social settings - 47%
- Depression or Anxiety – 41%
- Problems at school related to diet compliance – 30%
- Learning disabilities – 26%
- Difficulty forming interpersonal relationships – 18%
- Difficulties maintaining employment – 9%

What level of effort is required to adhere to a diet restricted in Phe as prescribed by your physician?

(With 1 being "no effort" and 5 being "much effort")

Answered: 216



In a typical week, how much time do you spend on the following PKU-related tasks?

Answered: 231

Total Time (average) all tasks = 39 hrs/wk. More than five hours a day.

- Cooking for Phe-restricted diet = 4 hrs/wk
- Supervising protein intake = 4 hrs/wk
- Planning daily Phe intake = 3+ hrs/wk
- Baking bread/low protein foods = 3 hrs/wk
- Weighing foods = 3 hrs/wk
- Keeping records = 3 hrs/wk
- Food research = 2.5 - 3 hrs/wk.
- Preparing for social events = 2.5 - 3 hrs/wk
- Researching PKU = 2.5 hrs/wk
- Blood testing = 1.5+ hrs/wk
- Ordering low proteins = 1.5+ hrs/wk
- Clinic appointments = 1.5+ hrs/wk
- PKU events = 1.5+ hrs/wk
- Ordering amino acids = 1.5+ hrs/wk

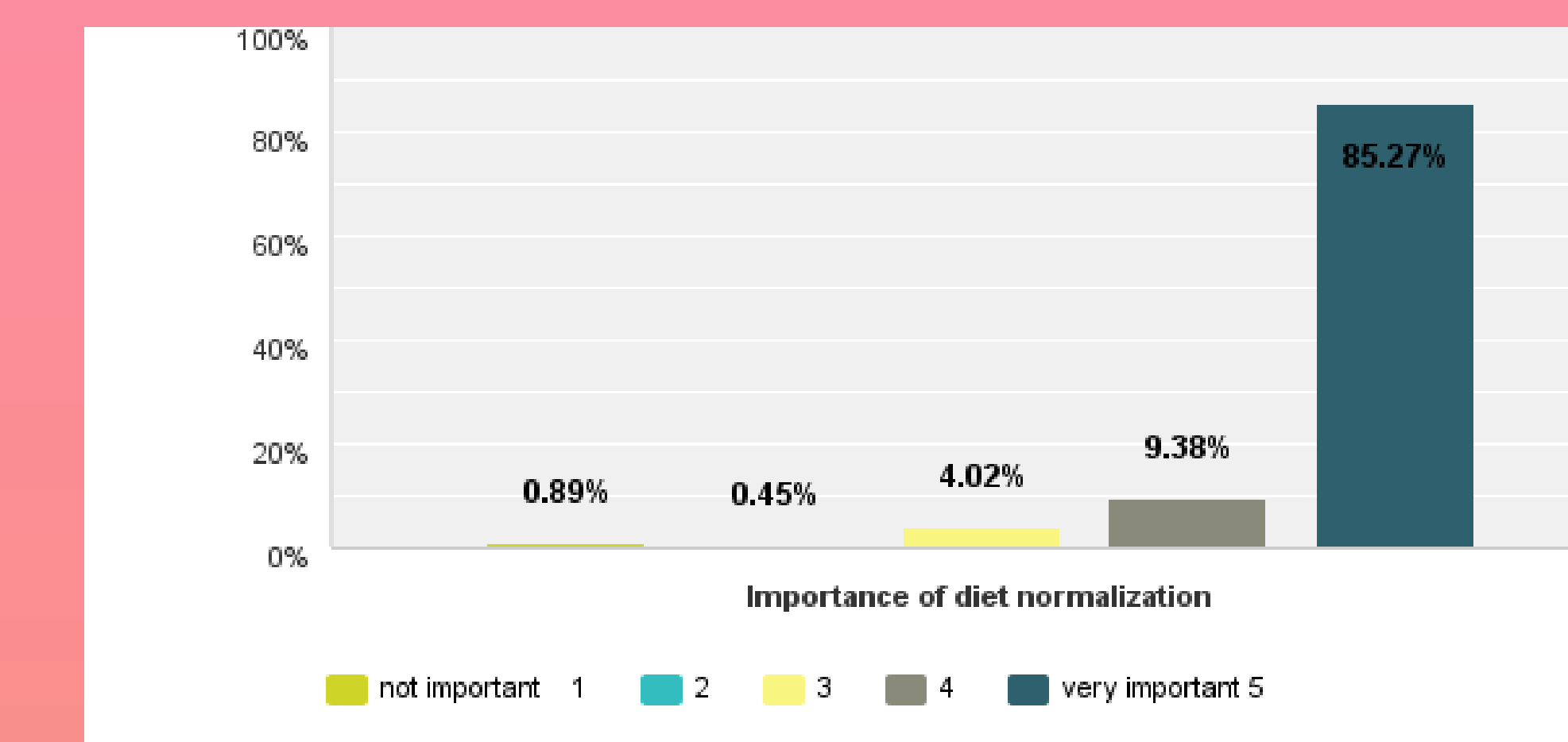
Diet normalization is critical to improving

Quality of Life

How important would the opportunity to normalize your diet have on improving your Quality of Life?

(With 1 being "not important" and 5 being "very important")

Answered: 224



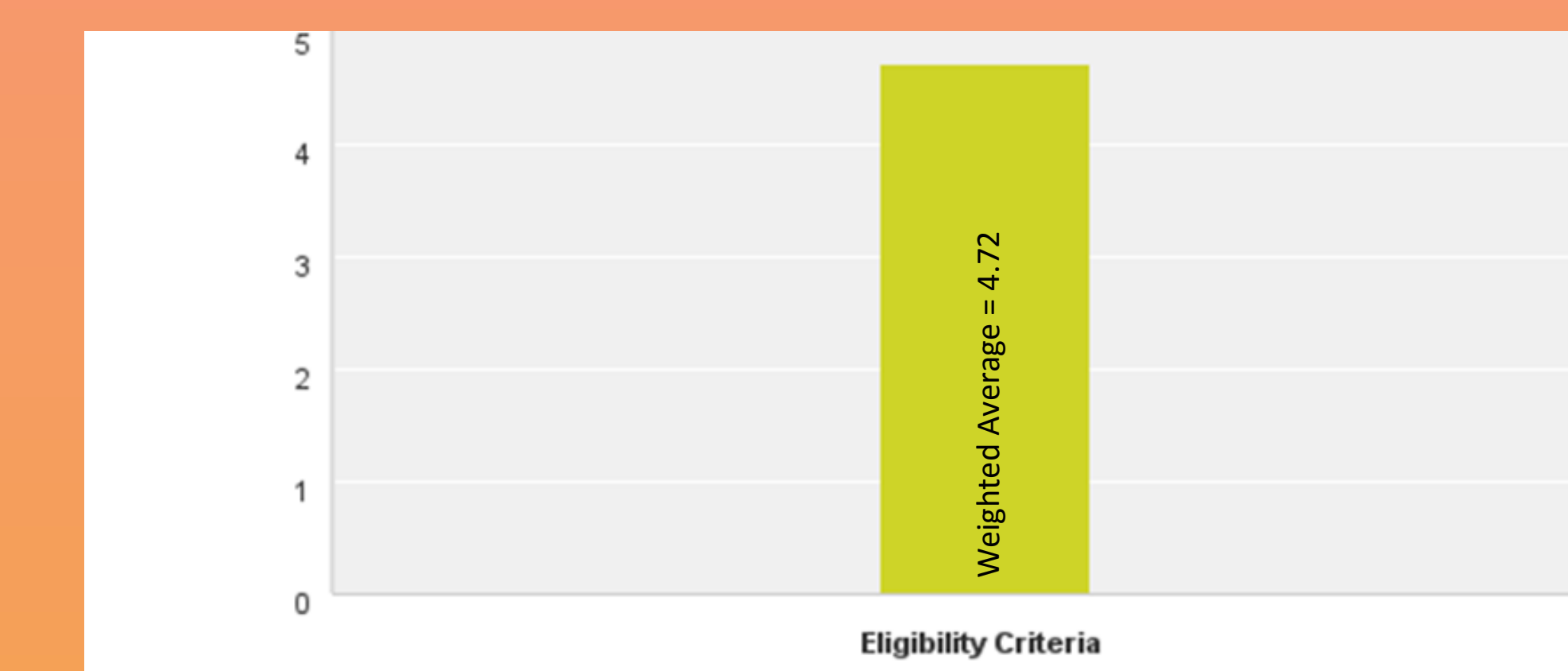
Treatment Option (KUVAN) is

Difficult to Access

We asked Ontario, Saskatchewan and Quebec Patients and Caregivers: *To what extent you find current reimbursement eligibility criteria for Kuvan in your province restrictive in achieving the best health outcomes for you?*

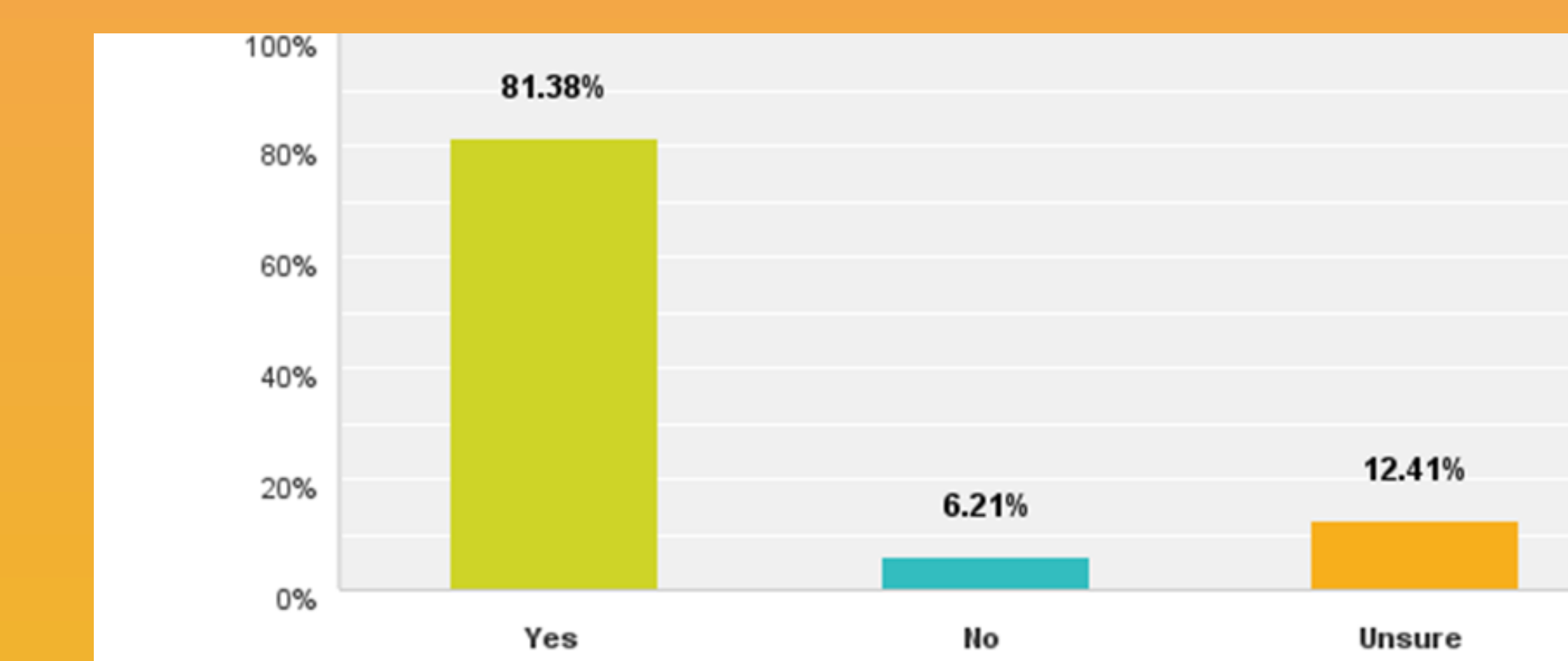
(With 1 being "not restrictive" and 5 being "very restrictive")

Answered: 58



We asked patients and caregivers in all other provinces & territories: *If funding was not an issue, is Kuvan a treatment option that you would like to investigate for the treatment of your PKU?*

Answered: 145



What Patients and Caregivers are Saying about Kuvan

"She went from an average student through 3 yrs of college to a 3.9 GPA in her 4th yr because of the ability to focus and she is able to absorb and process new information. Drastically lowered anxiety, very little shaking and no irritability. She is sleeping much better too. Kuvan is a Godsend, I wish she could have had it at a younger age. who knows what her potential may have been."

"KUVAN has been life changing, his protein intake tripled and his blood phe dropped in half. If we had a guarantee access to Kuvan for life he would no longer require formula. Kuvan is at cure level for my son, but without belief in access for life we have no choice but to continue him on the diet."

"I felt better on Kuvan, more stable and steady. My doctors increased my Phe intake by double greatly easing the difficulty involved in managing the diet."

"Kuvan was the best thing to happen to me. My levels stayed high no matter what I did. After starting kuvan my levels came down and stayed down and my allowance of phe tripled. ..."

"Enhanced memory short term and long term increased confidence in social atmospheres. Increased marks in post secondary education. Promotions at work and improvement of ability to carry out job responsibilities. Flexibility in managing diet. No restrictions whatsoever. Ability to improve physical fitness and conditioning. Higher level of focus and attention to detail Improvements in multi tasking ability. Higher energy levels in day to day activities. Critical thinking benefits."

Conclusions

- The highly restrictive diet is a heavy burden of treatment from both a **Quality of Life and financial** perspective.
- Dietary compliance needs to be recognized as a serious disease management issue.
- Existing diet therapy is not completely effective.
- Patients/Caregivers urgently require treatment options in conjunction to the prescribed diet.
- Kuvan is a highly effective treatment options for certain patients.
- Kuvan is difficult to access (with the exception of patients with excellent private insurance).

About CanPKU

Canadian PKU and Allied Disorders Inc. is a non-profit association dedicated to providing information and support to families and professionals dealing with PKU and similar, rare, inherited metabolic disorders.

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