

May 2, 2017

John Adams
President & CEO
Canadian PKU & Allied Disorders Inc.

Dear John,

Thank you for providing patient input again for Kuvan (sapropterin) to the CADTH Common Drug Review.

Your group's input helped CADTH reviewers develop the review protocol by identifying important outcomes (quality of life, neurophysiologic and neurocognitive benefits, Phe tolerance) and is discussed in the clinical report. The clinical report will shortly become available on CADTH's website.

A member of the Canadian Drug Expert Committee presented your input to provide the whole committee with patients' perspectives and to underscore unmet needs. Committee members reflected on how or whether the evidence under review addressed the issues identified by your group. A summary of the reasons for the recommendation is now available on our website.

CADTH reviewers and expert committee members found your input useful, particularly with regards to:

- Clearly outlining your extensive collection of both qualitative and quantitative data from 298 individuals. We noted that the clinical trials enrolled 206 and 56 patients for 13 and 26 weeks.
- Demonstrating how liberalization of diet would improve quality of life. The studies of sapropterin did not indicate a direct effect on improving quality of life. Instead, the committee accepted that evidence provided by a CADTH review of the relationship between Phe levels and diet liberalization, in conjunction with patient input, suggested that diet liberalization is likely associated with an improvement in quality of life.
- Detailing and explaining the side effects experienced by those using sapropterin and how side effects waned over time.

CADTH reviewers and the Canadian Drug Expert Committee members thank you for your time and effort in providing patients' perspectives. Patient input is an integral part of our review process and we appreciate your contribution.

Sincerely,



Sarah Berglas
Patient Engagement Officer, CADTH

cc: Frank Gavin, Public Member, CDEC; Allen Lefebvre, Public Member, CDEC; Trevor Richter, Director, CDR; Ken Bond, Director, Patient Engagement.