

Challenges in Health Economic Modeling of Phenylketonuria (PKU): A Targeted Review of HTA Evaluations

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Supplementary materials:

Websites of the following HTA agencies were searched:

- Pharmaceutical Benefits Advisory Committee, Australia
 - [Pharmaceutical Benefits Advisory Committee. Public Summary Document – March 2018 \(Sapropterin\)](#)
 - [Pharmaceutical Benefits Advisory Committee. Public Summary Document – July 2022 \(Sapropterin\)](#)
 - [Pharmaceutical Benefits Advisory Committee. Public Summary Document – July 2022 \(Pegvaliase\)](#)
- Canada Drug Agency (formerly Canadian Agency for Drugs and Technologies in Health), Canada
 - [CADTH Canadian Drug expert committee final recommendation – July 20, 2016](#)
 - [CADTH Reimbursement recommendation Pegvaliase \(Palynziq\)](#)
- National Institute for Health and Clinical Excellence, England
 - [Single Technology Appraisal – Sapropterin for treating phenylketonuria \[ID1475\] - Committee Papers – March 2021](#)
 - [Final appraisal document – Sapropterin for treating hyperphenylalaninaemia in phenylketonuria – August 2021](#)
- National Centre for Pharmacoeconomics, Ireland
 - [NCPE summary – Sapropterin \(Kuvan\) for the treatment of phenylketonuria \(PKU\) – June 2009](#)
 - [NCPE summary – Pegvaliase \(Palynziq®\) for the treatment of patients with phenylketonuria \(PKU\) – September 2022](#)
- Scottish Medicines Consortium, Scotland
 - [Scottish Medicines Consortium final advice – Sapropterin \(Kuvan\) – July 2018](#)
- Federal Joint Committee / Institute for Quality and Efficiency in Health Care, Germany
 - [GBA benefit assessment procedure for the active ingredient pegvaliase \(phenylketonuria\) – July 2019](#)
- Haute Autorité de Santé, France
 - [Transparency Committee opinion – KUVAN – February 2009](#)
- National Health Care Institute, The Netherlands
 - [Sapropterin \(Kuvan\) with hyperphenylalaninemia – March 2009](#)