Summary of Risk Management Plan for Kuvan® (sapropterin dihydrochloride)

Marketing Authorization Number 58475 (Swissmedic)

Document version: 1.0

Document date: 16 Jan 2024

Based on EU RMP version 16.0

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The Risk Management Plan (RMP) is a comprehensive document submitted as part of the application dossier for market approval of a medicine. The RMP summary contains information on the medicine's safety profile and explains the measures that are taken in order to further investigate and follow the risks as well as to prevent or minimise them.

The RMP summary of Kuvan is a concise document and does not claim to be exhaustive. As the RMP is an international document, the summary might differ from the "Arzneimittelinformation / Information sur le médicament" approved and published in Switzerland, e.g., by mentioning risks occurring in populations or indications not included in the Swiss authorisation.

Please note that the reference document which is valid and relevant for the effective and safe use of Kuvan in Switzerland is the "Arzneimittelinformation/ Information sur le médicament" (see www.swissmedicinfo.ch) approved and authorized by Swissmedic.

DRAC AG is fully responsible for the accuracy and correctness of the content of the published summary RMP of Kuvan.

PART VI: SUMMARY OF THE RISK MANAGEMENT PLAN BY PRODUCT

Summary of Risk Management Plan for Kuvan (Sapropterin dihydrochloride)

This is a summary of the risk management plan (RMP) for Kuvan. The RMP details important risks of Kuvan, how these risks can be minimised, and how more information will be obtained about Kuvan's risks and uncertainties (missing information).

Kuvan's summary of product characteristics (SmPC) and its package leaflet give essential information to healthcare professionals and patients on how Kuvan should be used.

This summary of the RMP for Kuvan should be read in the context of all this information including the assessment report of the evaluation and its plain-language summary, all which is part of the European Public Assessment Report (EPAR).

Important new concerns or changes to the current ones will be included in updates of Kuvan's RMP.

I. The medicine and what it is used for

Kuvan is indicated for the treatment of hyperphenylalaninemia (HPA) in adults and paediatric patients of all ages with phenylketonuria (PKU) who have been shown to be responsive to such treatment. Kuvan is also indicated for the treatment of HPA in adults and paediatric patients of all ages with tetrahydrobiopterin (BH4) deficiency who have been shown to be responsive to such treatment. It contains sapropterin dihydrochloride as the active substance and it is given orally, as soluble tablets (100 mg) or as powder (100 or 500 mg), to be dissolved in water and drunk.

Further information about the evaluation of Kuvan's benefits can be found in Kuvan's EPAR, including in its plain-language summary, available on the EMA website, under the medicine's webpage:

https://www.ema.europa.eu/en/medicines/human/EPAR/kuvan

II. Risks associated with the medicine and activities to minimise or further characterise the risks

Important risks of Kuvan, together with measures to minimise such risks and the proposed studies for learning more about Kuvan's risks, are outlined below.

Measures to minimise the risks identified for medicinal products can be:

- Specific information, such as warnings, precautions, and advice on correct use, in the package leaflet and SmPC addressed to patients and healthcare professionals;
- Important advice on the medicine's packaging;
- The authorised pack size — the amount of medicine in a pack is chosen so to ensure that the medicine is used correctly;

- The medicine's legal status — the way a medicine is supplied to the patient (eg, with or without prescription) can help to minimise its risks.

Together, these measures constitute *routine risk minimisation* measures.

In addition to these measures, information about adverse reactions is collected continuously and regularly analysed, including periodic safety update report (PSUR) assessment so that immediate action can be taken as necessary. These measures constitute *routine pharmacovigilance activities*.

If important information that may affect the safe use of Kuvan is not yet available, it is listed under 'missing information' below.

II.A. List of important risks and missing information

Important risks of Kuvan are risks that need special risk management activities to further investigate or minimise the risk, so that the medicinal product can be safely taken. Important risks can be regarded as identified or potential. Identified risks are concerns for which there is sufficient proof of a link with the use of Kuvan. Potential risks are concerns for which an association with the use of this medicine is possible based on available data, but this association has not been established yet and needs further evaluation. Missing information refers to information on the safety of the medicinal product that is currently missing and needs to be collected (eg, on the long-term use of the medicine).

List of Important Risks and Missing Information	
Important Identified Risks	None
Important Potential Risks	None
Missing Information	None

II.B. Summary of important risks

There are no important identified or potential risks associated with the use of Kuvan.

II.C. Post-authorisation development plan

II.C.1 Studies which are conditions of the marketing authorisation

There are no studies which are conditions of the marketing authorisation or specific obligation of Kuvan.

II.C.2 Other studies in post-authorisation development plan

There are no other studies in the post-authorisation development plan.