Swiss Summary of the Risk Management Plan (RMP) for Velaglucerase alfa (VPRIV)

Version 2.0, 15-Jan-2025
Based on EU RMP version 13.4, 07-Oct-2024
Marketing Authorization Holder: Takeda Pharma AG

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 risk as well as to prevent or minimise them.

The RMP summary of VPRIV 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 authorization.

Please note that the reference document which is valid and relevant for the effective and safe use of VPRIV in Switzerland is the "Arzneimittelinformation / Information sur le médicament" (see www.swissmedicinfo.ch) approved and authorized by Swissmedic. Takeda Pharma AG is fully responsible for the accuracy and correctness of the content of the published summary RMP of VPRIV.

Summary of risk management plan for VPRIV (Velaglucerase alfa)

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

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

This summary of the RMP for VPRIV 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 VPRIV's RMP.

I. The medicine and what it is used for

VPRIV is authorised for type 1 Gaucher disease (GD) (see SmPC for the full indication). It contains velaglucerase alfa (Gene-Activated Human Glucocerebrosidase, GA-GCB) as the active substance and it is given by intravenous infusion.

Further information about the evaluation of VPRIV's benefits can be found in VPRIV'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/vpriv

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

Important risks of VPRIV, together with measures to minimise such risks and the proposed studies for learning more about VPRIV'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 PL 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 (e.g., with or without prescription) can help to minimise its risks.

Together, these measures constitute routine risk minimisation measures.

In the case of VPRIV, these measures are supplemented with additional risk minimisation measures mentioned under relevant important risks, below.

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

II.A List of important risks and missing information

Important risks of VPRIV are risks that need special risk management activities to further investigate or minimise the risk, so that the medicinal product can be safely administered. 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 VPRIV. 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 (e.g., on the long-term use of the medicine).

List of important risks and missing information	
Important identified risks	• Infusion-related reactions, including allergic-type hypersensitivity reactions
Important potential risks	• None
Missing information	• None

II.B Summary of important risks

Important Identified Risk: Infusion-related reactions, including allergic-type hypersensitivity reactions	
Evidence for linking the risk to the medicine	Clinical studies: TKT025, TKT025EXT, TKT032, TKT034, HGT-GCB-039, HGT-GCB-058, HGT-GCB-044, HGT-GCB-087, HGT-GCB-068 and HGTGCB-091.
Risk factors and risk groups	As shown in clinical studies in treatment naïve patients, the risk of infusion reactions tends to decline with the increased duration of exposure. It is not known whether patients who have previously experienced infusion-related reactions to other ERT products are at increased risk of a reaction to velaglucerase alfa.
Risk minimization measures	**Routine risk minimization measures:** SmPC sections 4.2, 4.4 and 4.8 PL sections 3 and 4 **Additional risk minimization measures:** Educational material for healthcare professionals and patients/caregivers.
Additional pharmacovigilance activities	GOS TAK-669-4018 See section II.C of this summary for an overview of the post-authorisation development plan.

Abbreviations: GOS: Gaucher Disease Outcome Survey; PL: Package Leaflet; SmPC=Summary of Product Characteristics.

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 VPRIV.

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

Study name: GOS

Purpose of the study: To monitor the safety and effectiveness of velaglucerase alfa.

Study name: TAK-669-4018

Purpose of the study: To determine and assess patients, caregivers and home infusion nurses' awareness and understanding of the EM, including infusion diary and emergency plan associated with VPRIV home infusion.

Specifically, to assess the proportion of patients/caregivers and home infusion nurses who are aware of the EM; who understand the EM; and who use the EM.