RISK MANAGEMENT PLAN

ELFABRIO
[PEGUNIGALSIDASE ALFA]

RMP Version Number: **1.0**

Data Lock Point for this RMP: **08 October 2021**

Date of final sign-off: **31 January 2023**

Marketing Authorisation Holder: Chiesi SA, Villar-sur-Glâne, Switzerland

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 Elfabrio 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 Elfabrio in Switzerland is the "Arzneimittelinformation / Information sur le médicament" (see www.swissmedic.ch) approved and authorized by Swissmedic. Chiesi SA is fully responsible for the accuracy and correctness of the content of the published summary RMP of Elfabrio.

Summary of the risk management plan

This is a summary of the risk management plan (RMP) for Elfabrio, (hereafter PRX-102). The RMP details important risks of PRX-102, how these risks can be minimised, and how more information will be obtained about PRX-102 's risks and uncertainties (missing information). PRX-102 summary of product characteristics (SmPC) and its package leaflet give essential information to healthcare professionals and patients on how PRX-102 should be used. This summary of the RMP for PRX-102 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 PRX-102 RMP.

I. The medicine and what it is used for

PRX-102 is an alpha- galactosidase A (α-Gal A) enzyme replacement therapy (ERT) indicated for the treatment of adults with a confirmed diagnosis of Fabry disease. PRX-102 is indicated for long-term enzyme replacement therapy in adult patients with a confirmed diagnosis of Fabry disease (deficiency of alpha-galactosidase). where the level of α-galactosidase enzyme activity is absent or lower than normal. In subject suffering from Fabry disease a fat substance, called globotriaosylceramide (GL-3), is not removed from the cells of the body and starts to accumulate in the walls of the blood vessels. PRX-102 is indicated for use as long-term enzyme replacement therapy in patients with a confirmed diagnosis of Fabry disease (α-galactosidase A deficiency). PRX-102 is indicated in adults, it contains Pegunigalsidase alfa as its active substance and is given by infusion.

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

Important risks of PRX-102, together with measures to minimise such risks and the proposed studies for learning more about PRX-102 risks, are outlined below.

Measures to minimise the risks identified for PRX-102 will be:

- Specific information, such as warnings, precautions, and advice on correct use, in the package leaflet and SmPC addressed to patients and healthcare professionals

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

In the case of PRX-102, these measures are supplemented with *additional risk minimisation measures* (HCP brochure and patient/caregiver/HCP guide) mentioned in section V.2 as well as under relevant important risks, below.

In addition to these measures, information about adverse reactions will be collected continuously and regularly analysed (including PSUR assessment and signal detection) 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 PRX-102 is not yet available, it is listed under 'missing information' below.

II.A List of important risks and missing information

Important risks of PRX-102 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 PRX-102. 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	• Hypersensitivity Reactions (infusion related)
Important potential risks	• Medication errors in the home infusion setting
Missing Informations	• None

II.B Summary of important risks

Important identified risk: Hypersensitivity Reactions (infusion related)	
Evidence for linking the risk to the medicine	Clinical trials included in the development programme
Risk factors and risk groups	Patients undergoing IV infusion and/or subjects that had previous ERTs (Fabrazyme or Replagal).
Risk minimisation measures	Routine risk minimisation measures: • SmPC section 4.4. • PL section 2 Additional risk minimisation measures: • HCP brochure • Patient/caregiver/HCP guide

Important potential risk: Medication errors in the home infusion setting	
Evidence for linking the risk to the medicine	Clinical trials included in the development programme
Risk factors and risk groups	• Patients receiving PRX-102 infusions in the home setting by an HCP

Risk minimisation measures	Routine risk minimisation measures: • SmPC section 4.2 • SmPC section 6 Additional risk minimisation measures: • HCP brochure • Patient/caregiver/HCP guide

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 for PRX-102

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

There are no studies required in the post-authorization plan for PRX-102.