Swiss Summary of the Risk Management Plan (RMP) for ADZYNMA® (recombinant ADAMTS13 [rADAMTS13])

Version 1.0, 31-Mar-2026
Based on EU RMP version 1.0, 17-June-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 ADZYNMA 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 ADZYNMA 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 ADZYNMA.

Summary of risk management plan for ADZYNMA (recombinant ADAMTS13 [rADAMTS13])

This is a summary of the risk management plan (RMP) for ADZYNMA. The RMP details important risks of ADZYNMA, how these risks can be minimised (through routine pharmacovigilance [PV], clinical studies and signal detection), and how more information will be obtained about ADZYNMA's risks and uncertainties (missing information).

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

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

I. The medicine and what it is used for

ADZYNMA is authorised for Congenital TTP (see SmPC for the full indication). It contains ADZYNMA as the active substance and it is given by intravenous route.

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

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

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

Important risks of ADZYNMA, together with measures to minimise such risks and the proposed studies for learning more about ADZYNMA'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 (e.g., 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 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 ADZYNMA 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 ADZYNMA. 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	None
Important potential risks	• Neutralizing (inhibitory) antibodies to rADAMTS13 • Hypersensitivity reactions
Missing information	• Risks in case of pregnancy and lactation • Long-term safety

II.B Summary of important risks and missing information

The safety information in the proposed product information is aligned to the reference medicinal product.

Important potential risk: Neutralizing (inhibitory) antibodies to rADAMTS13	
Evidence for linking the risk to the medicine	Clinical trials and literature
Risk factors and risk groups	In iTTP patients with existing anti-ADAMTS13 antibodies, administration of rADAMTS13 may induce an anamnestic response while in cTTP patients there could be lack of efficacy.
Risk minimization measures	Routine risk minimisation measures: Sections 4.4 in the SmPC Section 2 of the PL Additional risk minimisation measures: No additional risk minimisation measures.
Additional pharmacovigilance activities	Additional pharmacovigilance activities: TAK-755 PASS study See section II.C of this summary for an overview of the post-authorisation development plan.

Important potential risk: Hypersensitivity reactions	
Evidence for linking the risk to the medicine	Clinical trials and literature
Risk factors and risk groups	Patients with known hypersensitivity to the parent molecule, any component of rADAMTS13 or hamster proteins are at increased risk of developing potentially life-threatening reactions.
Risk minimization measures	Routine risk minimisation measures:

	Sections 4.4 in the SmPC Section 2 of the PL Additional risk minimisation measures: Educational Tools for HCP Educational tools for patients and caregivers (for home and self-administration)
Additional pharmacovigilance activities	Additional pharmacovigilance activities: TAK-755 PASS study See section II.C of this summary for an overview of the post-authorisation development plan.

Missing information: Risks in case of pregnancy and lactation	
Risk minimization measures	Routine risk minimisation measures: Sections 4.6 in the SmPC Section 2 of the PL Additional risk minimisation measures: No additional risk minimisation measures.
Additional pharmacovigilance activities	Additional pharmacovigilance activities: TAK-755 PASS study See section II.C of this summary for an overview of the post-authorisation development plan.

Missing information: Long-term safety	
Risk minimization measures	Routine risk minimisation measures: Sections 4.4 and 4.6 in the SmPC Section 2 of the PL Additional risk minimisation measures: No additional risk minimisation measures.
Additional pharmacovigilance activities	Additional pharmacovigilance activities: TAK-755 PASS study See section II.C of this summary for an overview of the post-authorisation development plan.

II.C. Post-authorisation development plan

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

TAK-755 PASS Study

To evaluate specific safety concerns potentially related to TAK-755 in patients with cTTP, including the important potential risks and missing information delineated in the EU RMP, including neutralizing (inhibitory) antibodies to rADAMTS13, hypersensitivity reactions, risks in case of pregnancy, and long-term safety following TAK-755 administration. This is designed to meet an EMA specific obligation.

Objectives:

- Estimate risk of neutralizing antibodies to rADAMTS13 and hypersensitivity reactions
- Characterize long-term safety
- Describe pregnancy exposures and outcomes, as available

Study 281102

Purpose of the study: To assess safety and efficacy of BAX 930 in the prevention and treatment of acute thrombotic thrombocytopenic purpura (TTP) events in subjects with severe congenital deficiency of ADAMTS13 (cTTP; defined as plasma ADAMTS13 <10%, as measured by the fluorescent resonance energy transfer-VWF73 assay).

Study TAK-755-3002

Purpose of the study: To evaluate the long-term safety and tolerability of TAK-755 (rADAMTS13) in terms of related treatment-emergent adverse events (TEAEs) and related serious adverse events (SAEs) in both the prophylactic and the on-demand cohorts.

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

There are no studies required for ADZYNMA.