

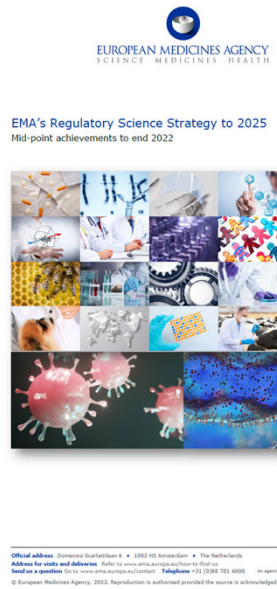
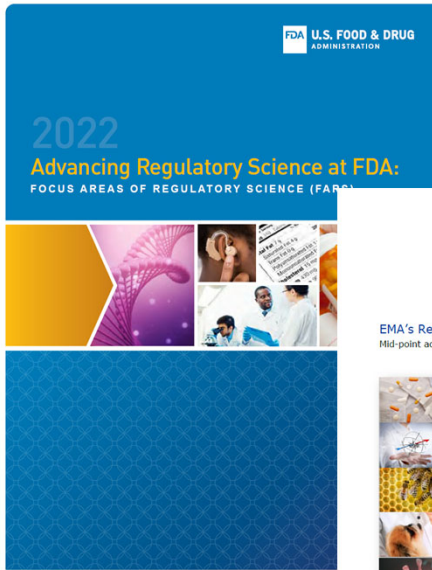


Evidence-based authorisation of the future / Regulatory science

Dr Eveline Trachsel

Head of Medicinal Product Authorisation and Vigilance Sector

What will evidence-based authorisation look like in the future?



Focus areas in regulatory science

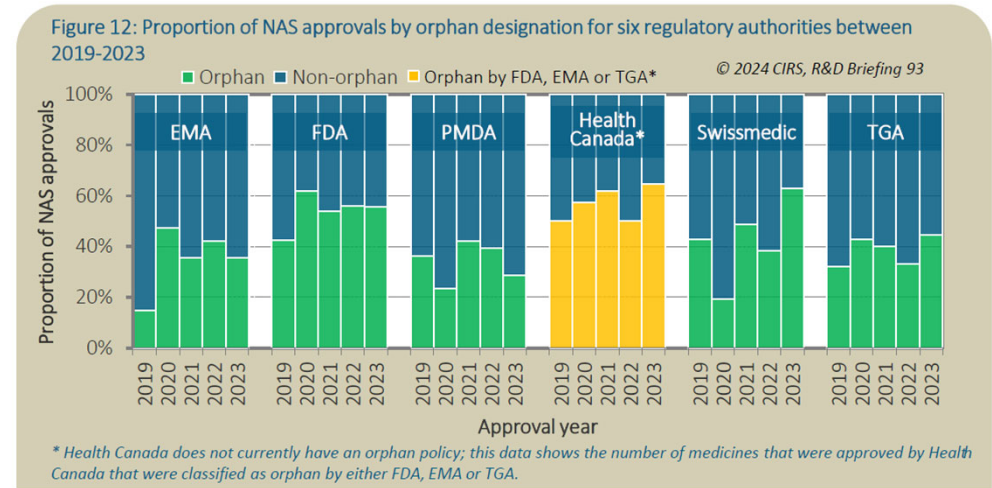
Balance between innovation and patient safety

- Orphanisation / real-world evidence
- Digitalisation / artificial intelligence
- Globalisation / international coordination
- Health equity / involving patients

Rare diseases and orphan drugs



ICMRA "Rare" Symposium 16/17 Sep. 2024



CIRS R&D briefing - New drug approvals by 6 authorities 2014-2023

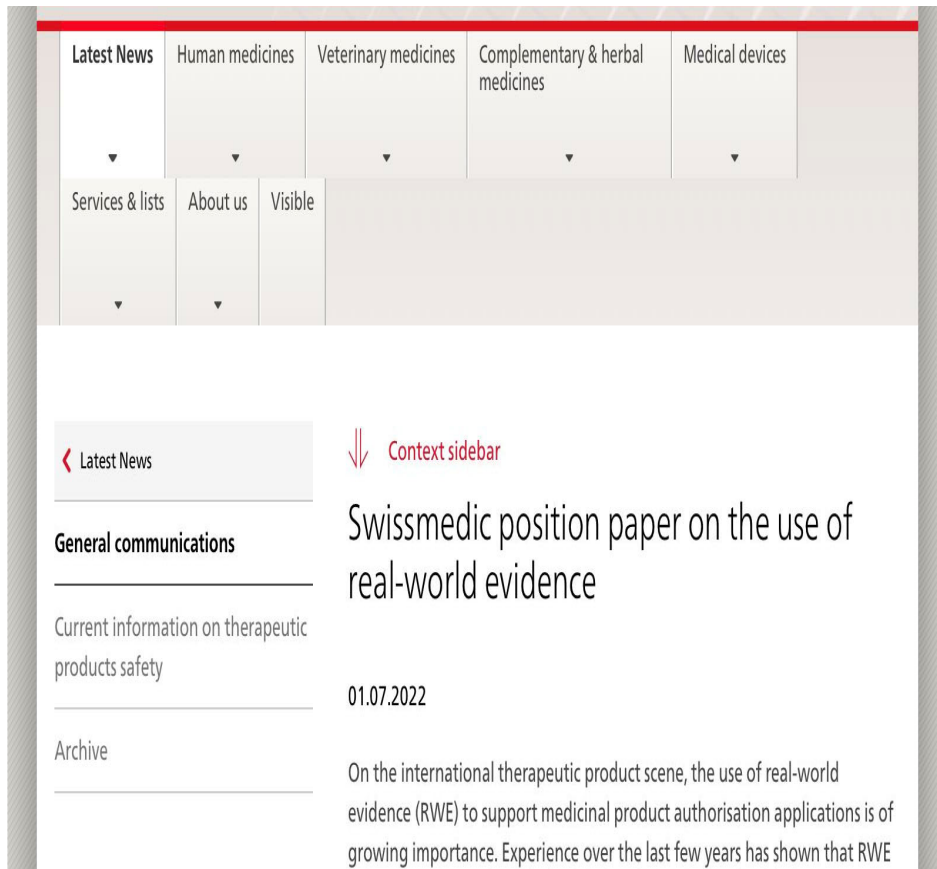
5.3 Application for recognition of ODS (Art. 4 – 7 TPLO)

5.3.1 Principle

A human medicinal product is granted the status of important medicinal product for rare diseases (Orphan Drug) on application if the applicant can demonstrate that the medicinal product meets the criteria in accordance with Art. 4 para. 1 letter a^{decies} TPA (Art. 4 TPLO).

The criterion for the rarity of the disease always applies to the disease in its entirety, including all stages of it, and not to an isolated stage in the course of the disease or to a sub-group defined by molecular genetic markers, unless the subgroup is so limited as a result of another medical condition that it is recognised and classified as a separate disease. A sub-group (e.g. Her-2 positive breast cancer) does not qualify as an independent, rare disease; neither, for example, does the restriction of an indication to second-line treatment.

The RWE position paper describes Swissmedic's stance



The screenshot shows the Swissmedic website's navigation menu with categories: Latest News, Human medicines, Veterinary medicines, Complementary & herbal medicines, Medical devices, Services & lists, About us, and Visible. Below the menu, there is a sidebar with 'Latest News' and 'Context sidebar' options. The main content area displays a news article titled 'Swissmedic position paper on the use of real-world evidence' dated 01.07.2022. The article text states: 'On the international therapeutic product scene, the use of real-world evidence (RWE) to support medicinal product authorisation applications is of growing importance. Experience over the last few years has shown that RWE'.

Due to the various uncertainties associated with the use of RWD/RWE, detailed descriptions and explanations of the methodology and statistics, predefined in a study protocol, are of particular importance. The following general aspects need to be addressed when planning RWE:

- Definition of the **research question(s) and objective(s)**, including rationale and appropriateness of outcome measures and preferably using the estimand framework (ICH E9(R1))
- Description and **justification of the research/study design** including a discussion of strengths and weaknesses
- Detailed information on the pertinent **RWD sources including data standards** applied, coding systems, traceability, quality check procedures and whether the data were collected prospectively or retrospectively
- Definition of the **study population using inclusion/exclusion criteria**, including a discussion on generalisability
- **Statistical Analysis Plan** including sample size considerations, detailed description of primary and secondary outcome measures, statistical methods, planned sensitivity and subgroup analyses
- Milestones/timelines such as approval/waiver by ethics committees, data capture (start/end date), data cut-off(s), database lock, planned reporting (interim/final)
- Discussion of anticipated limitations, challenges and potential biases
- Reporting of amendments and protocol deviations

In addition to the critical points listed above, compliance with national and international law and regulations, ICH guidelines, ethical, legal and regulatory standards needs to be ensured.

Appropriate **consents and data anonymisation/de-identification techniques** are required to ensure compliance with data privacy requirements and must be confirmed to Swissmedic in writing.

Do medicinal products have different effects in women than men?

Médecine intégrant une approche genre et santé publique :



un plus pour toute la société
12 juin 2024, de 9h15 à 16h30, Berne

Keynote-Session II :

Les différences en matière de diagnostic, de soins et de thérapies

Différences de genre chez les proches aidants de personnes atteintes de démence

Annemarie Schumacher Dimech, responsable du programme CAS Palliative Care, Faculté des sciences de la santé et de médecine, Université de Lucerne (all)

Différences de genre dans le domaine des soins : la prestation de soins intensifs

Atanas Todorov, Scientifique médical, Gebhardlab (all)

Aspects spécifiques au genre dans l'approbation de médicaments

Christine Haenggeli, responsable Clinical Assessment, Secteur Autorisations de mise sur le marché et vigilance Médicaments (fr)

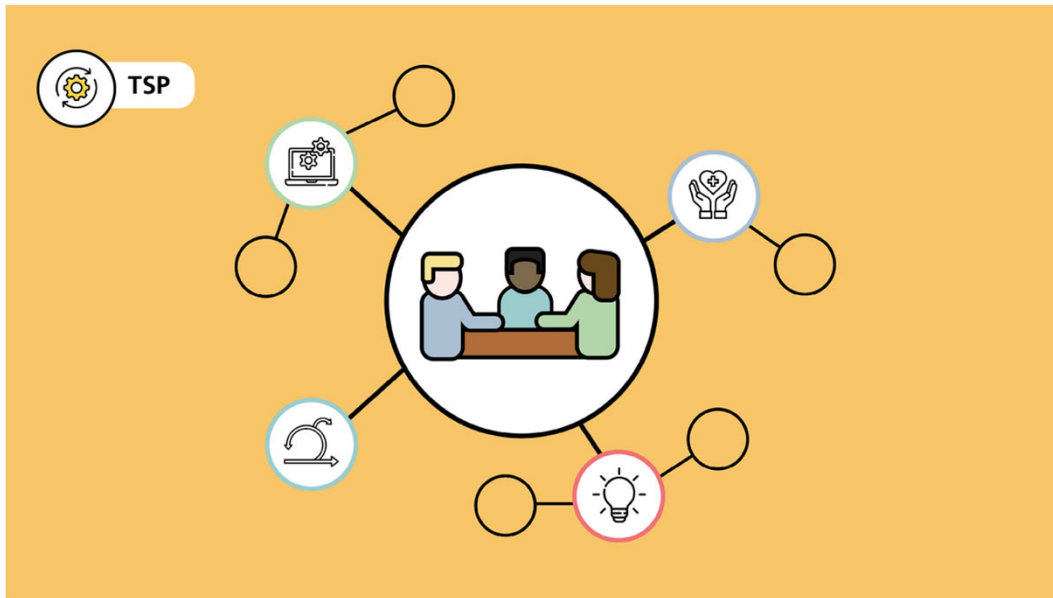


New member of the Swissmedic Expert Committee

Prof. Berna Özdemir

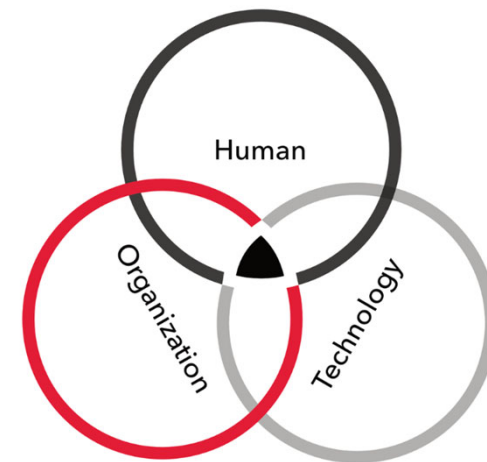
Oncologist and sex/gender specialist, Inselspital

Digitalisation and artificial intelligence



Presentation by Roger Rüegg

SWISSmedic 4.0



Presentation by Michael Renaudin