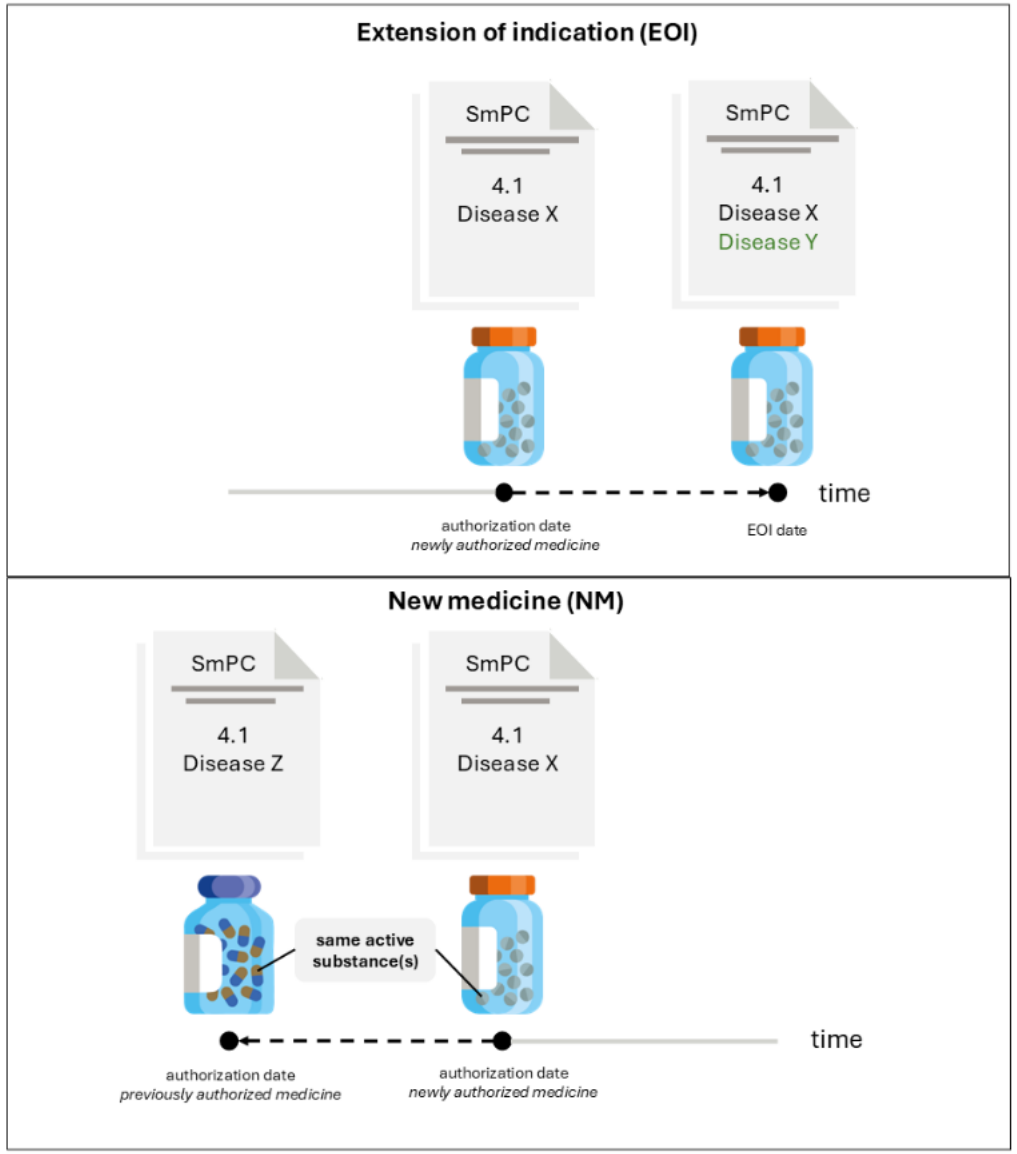
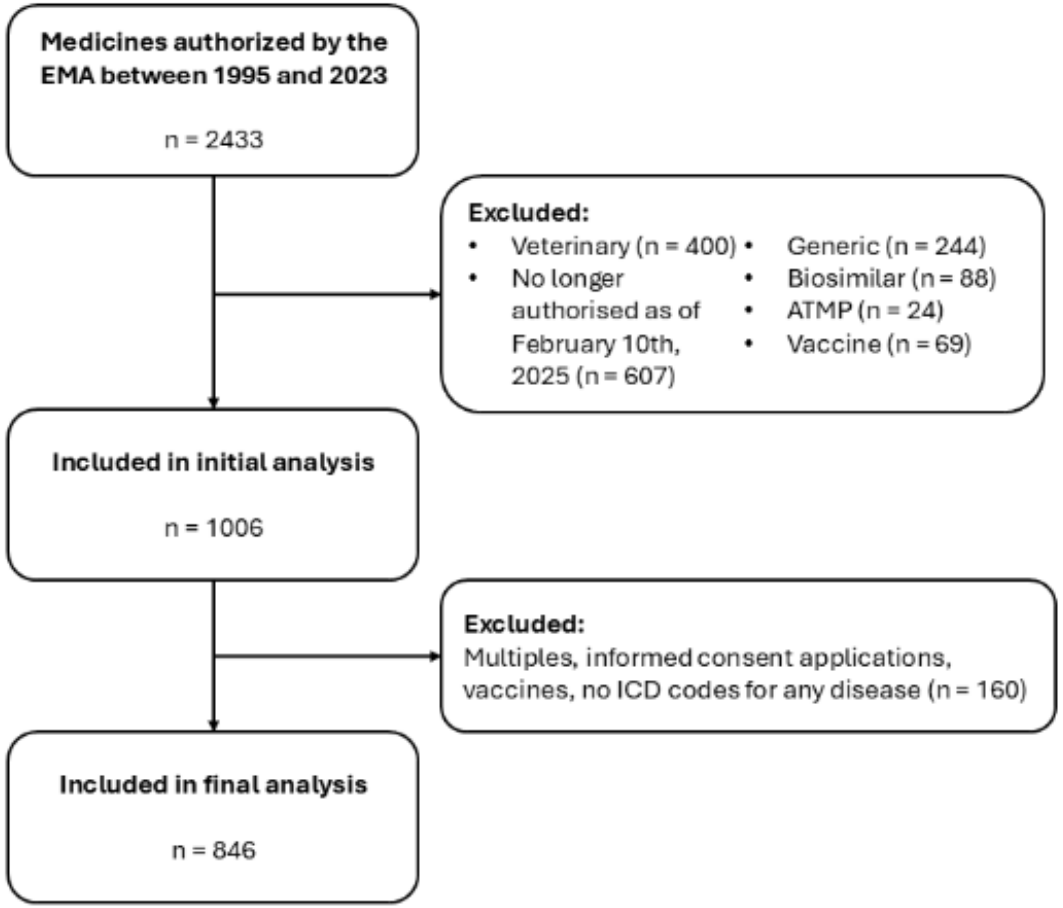
Addition of new uses



How frequently are new diseases added to the indication?

- Data cut-off

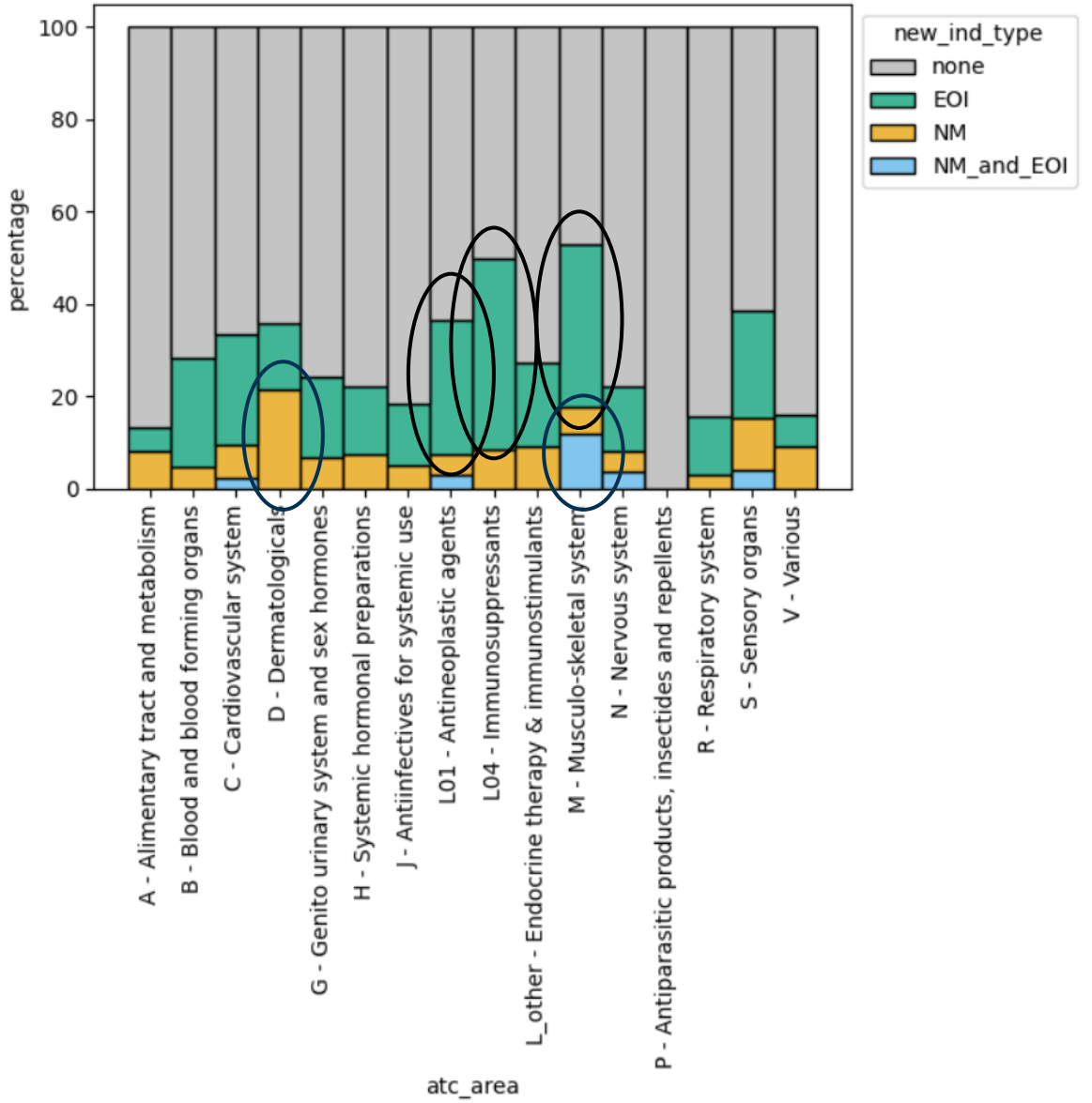
  31 December 2024



Muizelaar et al. 2026, in preparation

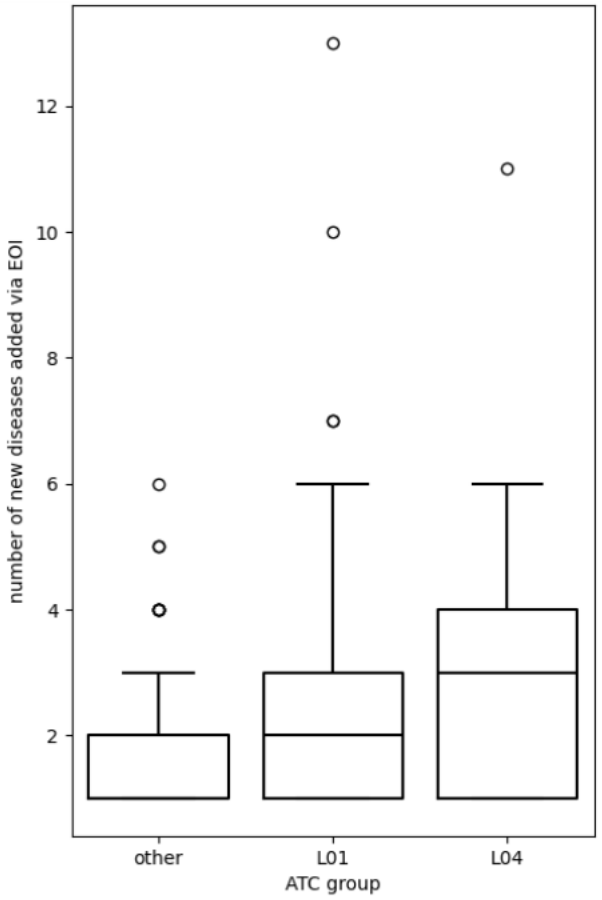
>30% of medicines with a new disease

- 846 medicines included
- 379 new diseases added to 184 medicines (EOI) – **22%**
- 67 medicines (NM) – **7.9%**
- 12 medicines both NM and EOI – **1.4%**

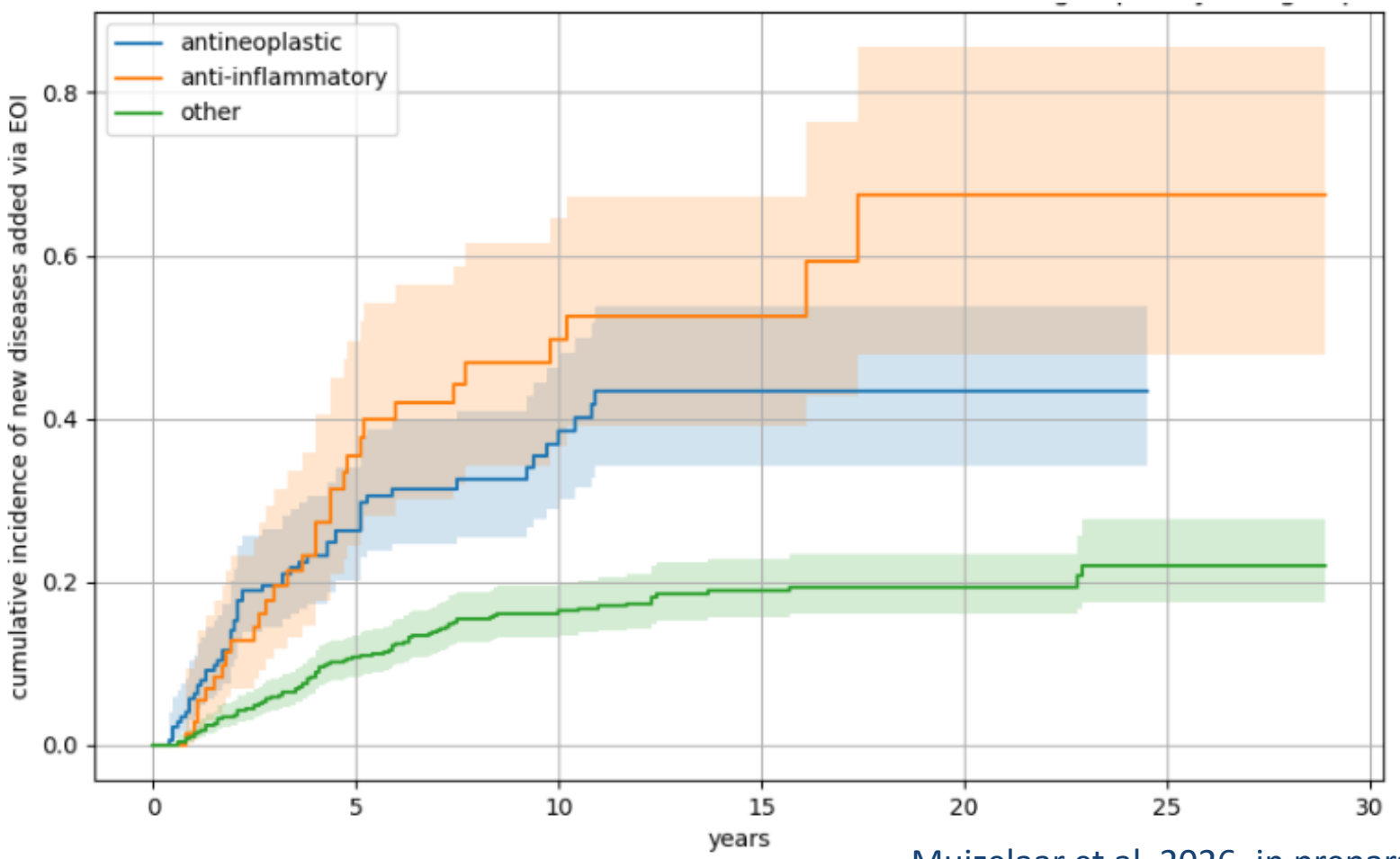


Muizelaar et al. 2026, in preparation

Majority new diseases added first 10 years after marketing authorisation



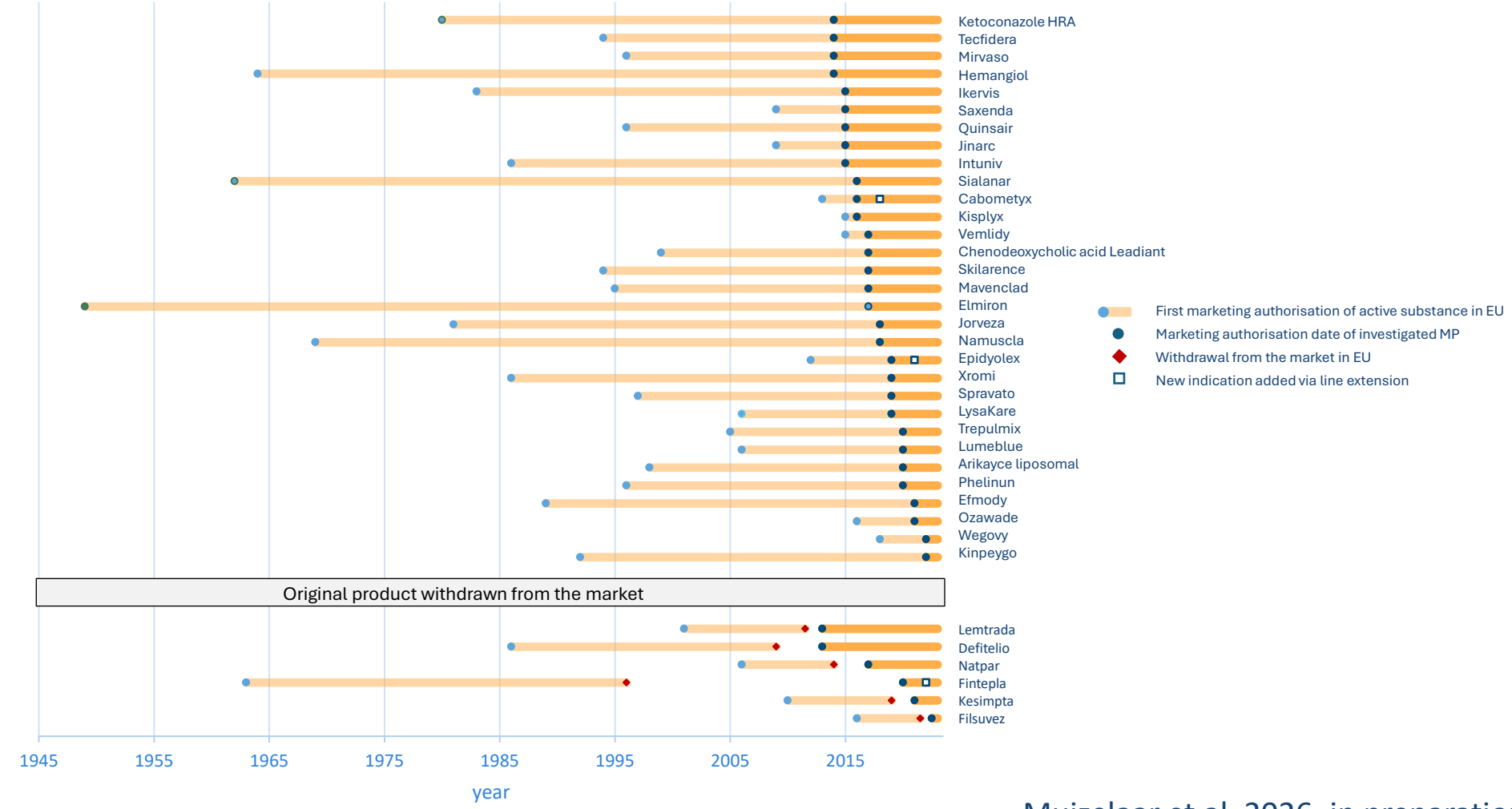
Cumulative incidence for new diseases added via an EOI



Muizelaar et al. 2026, in preparation

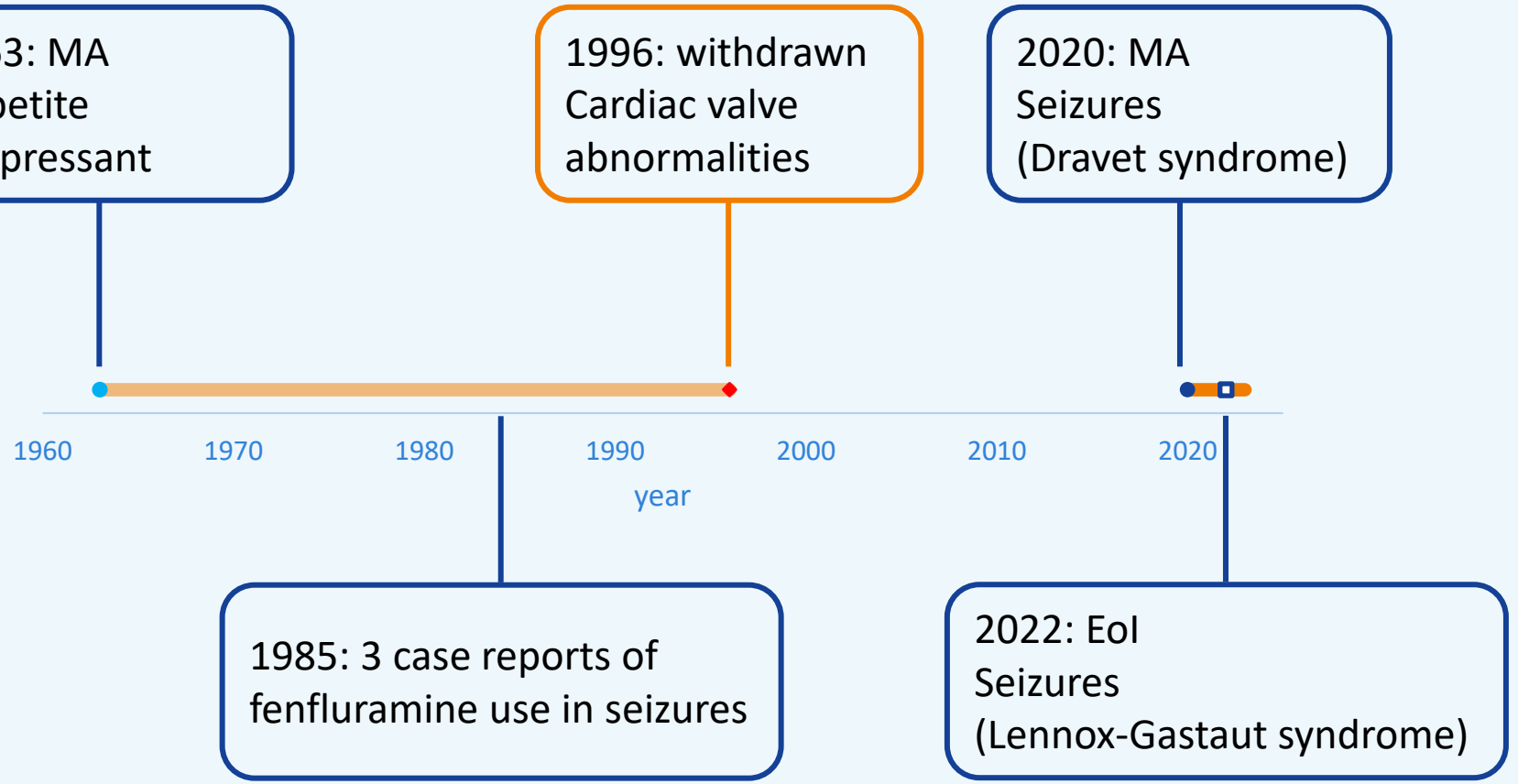
New medicinal product based on known active substance

> time to new disease frequently longer than 10 years



Muizelaar et al. 2026, in preparation

Example case fenfluramine (Fintepla)



Example case fenfluramine (Fintepla)

2014: Protocol Assistance (SAWP)

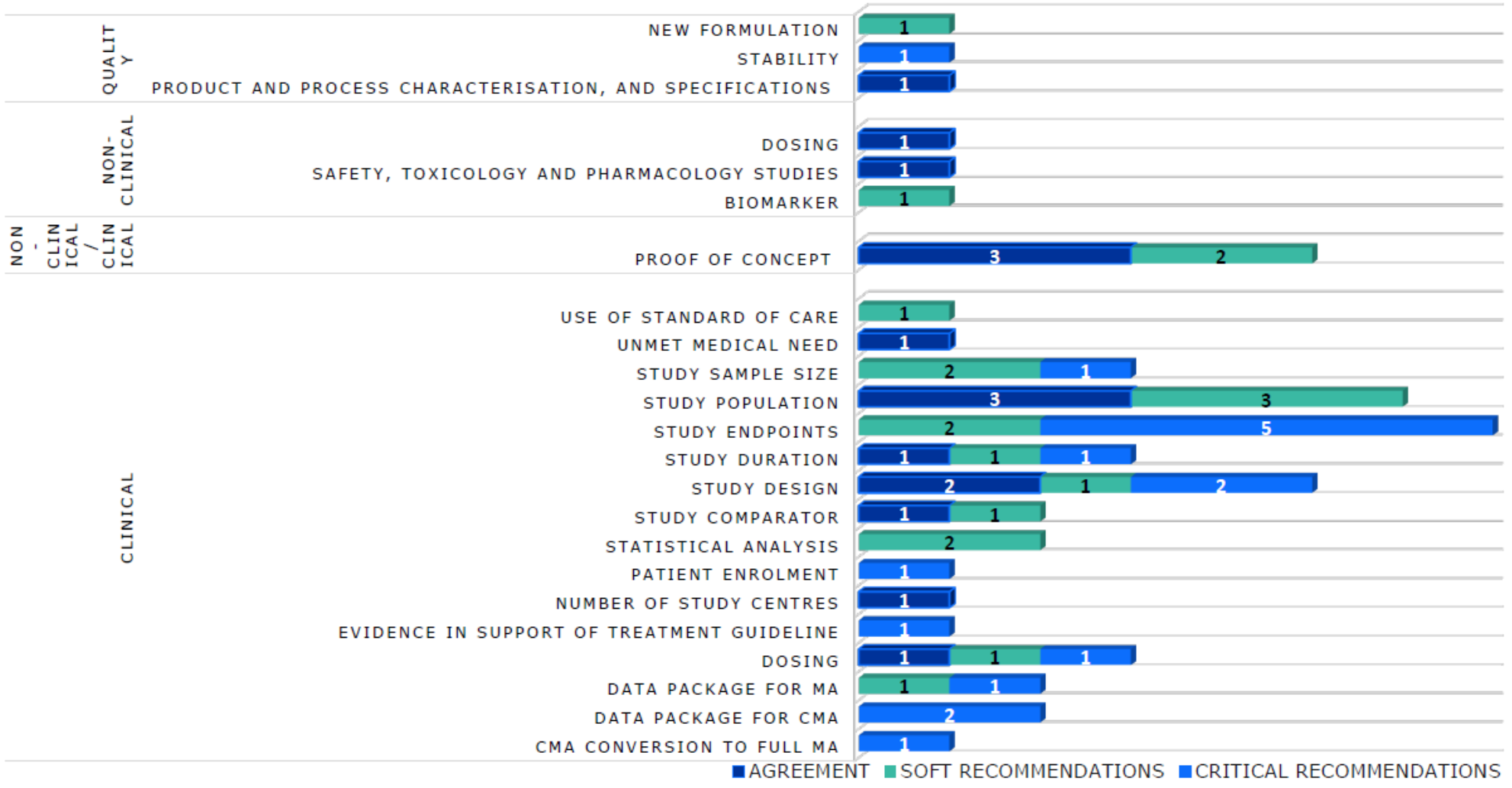
- Adequacy of the design of a planned placebo-controlled, double-blind clinical study, in particular to the proposed primary efficacy endpoint, the sample size, the duration of treatment, the stratified design based on age with a cut-off of 6 years of age and the proposed safety monitoring assessments.

• Appropriateness a proposed patient registry to collect safety and efficacy data in treated children post-authorisation including a special focus on cardiac safety monitoring as the basis for a future Risk Management Plan

• Adequacy of the plans for PopPK analyses to characterise the PK profile in subjects with Dravet Syndrome.

2020: MA
Seizures
(Dravet syndrome)

Frequency of agreement between champion's proposals and Scientific Advice outcome per topics



New Pharmaceutical Legislation

Article 48: Scientific opinion on data submitted from not-for-profit entities for repurposing of authorised medicinal products

A **not-for-profit entity** may submit to the Agency or to a competent authority of the Member State **substantive non-clinical or clinical evidence** for a **new therapeutic indication** that is expected to fulfil **an unmet medical need**.

The Agency may, at the request of a Member State, the Commission, or on its own initiative and on the basis of all available evidence, including any additional evidence that may be submitted by the marketing authorisation holders for the medicinal products concerned, make a scientific evaluation of the benefit-risk of the use of a medicinal product with a new therapeutic indication. The Agency shall draw up **guidance on the consultation process**.

The **opinion of the Agency shall be made publicly available** and the competent authorities of the Member States and the marketing authorisation holder shall be informed.

In cases where the opinion is favourable and the new therapeutic indication addresses an unmet medical need, on the request of the Agency, the marketing authorisation holders of the medicinal products concerned **shall submit a variation** to update the product information with the new therapeutic indication.

Scientific Advice sources – EMA and national

- Training for academic researchers and applicants on Scientific Advice

  EMA in collaboration with EATRIS -- training video and PDF resource

- EU-Innovation Network guidance on available scientific and regulatory support tools at national and European level for human medicinal products/technologies/methodologies

  https://www.ema.europa.eu/en/committees/working-parties-other-groups/eu-innovation-network-eu