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COLLEGE TER BEOORDELING VAN GENEESMIDDELEN

Compassionate Use Programme (CUP)

GOEDE MEDICIJNEN GOED GEBRUIKT

Introduction speaker

Cees Tuinenburg: Regulatory Project Leader Medicines Evaluation Board.

- Biomedical Technology (M Sc)
- Regulatory Project Leader MEB since 2007
- Chair of Commitee Compassionate Use at MEB
- Area's of interest: oncology, companion diagnostics, pulmonology, medicinal gasses, CUP

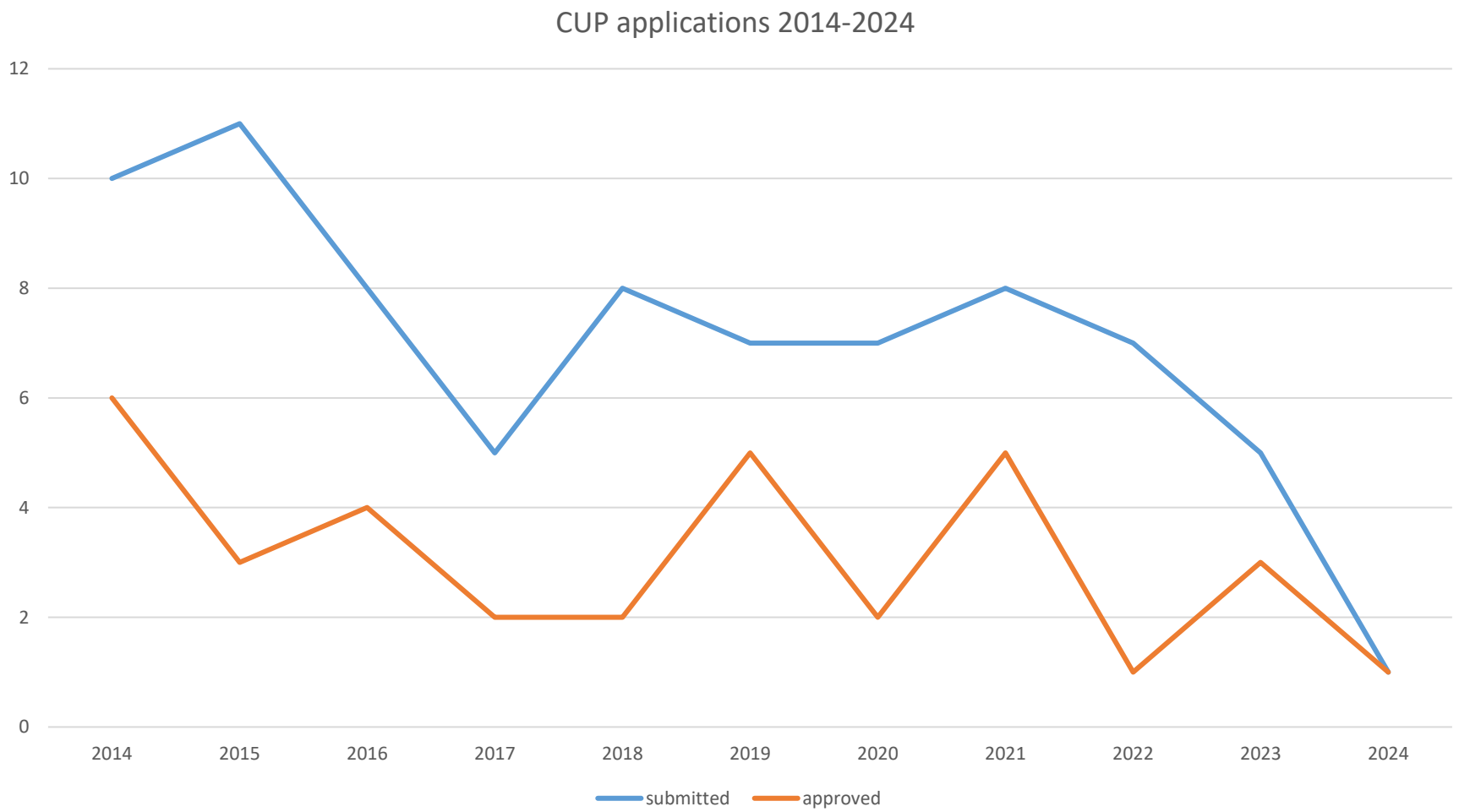
Disclosure of interests of Cees Tuinenburg	
(Potential) Conflict of interest	None
Potentially relevant company relationships in connection with event	None
Sponsorship or research funding Fee or other (financial) payment Shareholder Other relationship, i.e. …	None

Named Patient ≠ CUP

Compassionate Use Program (CUP) vs. Named Patient Use (NPU) in the Netherlands

	CUP	NPU
EU Legislation	Article 83 of Regulation (EC) No 726/2004	Article 5(1) of Directive 2001/83/EC
NL Legislation	Article 3.18 of the Dutch Medicines Act Regulations	Article 3.17 of the Dutch Medicines Act Regulations
Purpose	To provide access to unauthorized medicines to patients with life-threatening conditions with no registered alternatives	To provide access to unauthorized medicines to patients with no registered alternatives and an unmet medical need
Initiator	Pharmaceutical company	Physician, pharmacy, wholesaler, or pharmaceutical company
Eligibility	Group of patients (cohort)	Individual patient
Regulatory body	MEB	IGJ
Benefit-risk assessment	Assessed by the MEB	No benefit-risk assessment by the IGJ
Duration	Valid until marketing authorization	Valid for 1 year; an extension is possible

Stats


CUP applications 2014-2024
12
10
8
6
4
2
0
2014
2015
2016
2017
2018
2019
2020
2021
2022
2023
2024
submitted
approved

Study of Aimee Timmerman

- What motivates pharmaceutical companies to apply for **Compassionate Use Programs (CUPs)** ?

  - Factors influencing the decision to apply for a CUP have to do with **regulatory, medical, operational, and financial factors.**
  - Most significant barriers were **regulatory uncertainties** (like what happens after the medicine is approved) and **operational challenges** (such as prior experience and supply logistics).

Legal framework

MEB 49:

In the Netherlands the MEB is the competent authority for approval of CUP.

The legal framework originates from

- Article 83 of Regulation (EC) No. 726/2004,
- read in conjunction with Section 40(3), preamble and f of the Dutch Medicines Act
- and Section 3.18 of the Medicine Law Regulation.

Criteria

1. The medicinal product must be intended for treatment of a group of patients (cohort) suffering from a chronic disease, a disease that is detrimental to their health or a life-threatening disease.
2. This disease cannot be treated satisfactorily using an authorised medicinal product.*
3. An application for a marketing authorisation for the medicinal product has been submitted, or clinical tests to support such an application are still ongoing

* With regard to the second criterion: there should be sufficient evidence that the Benefit/Risk balance of the medicinal product in the CUP is positive

Why set up a Compassionate Use Programme?

- Earlier access to the medicinal product
- Immediate availability for patiënt and HCP
- The MEB has assessed the Benefit/Risk Balance

MEB 49: what's new?

- Published policy document based on current legislation
- A decision of the MEB to grant a CUP has no end date.