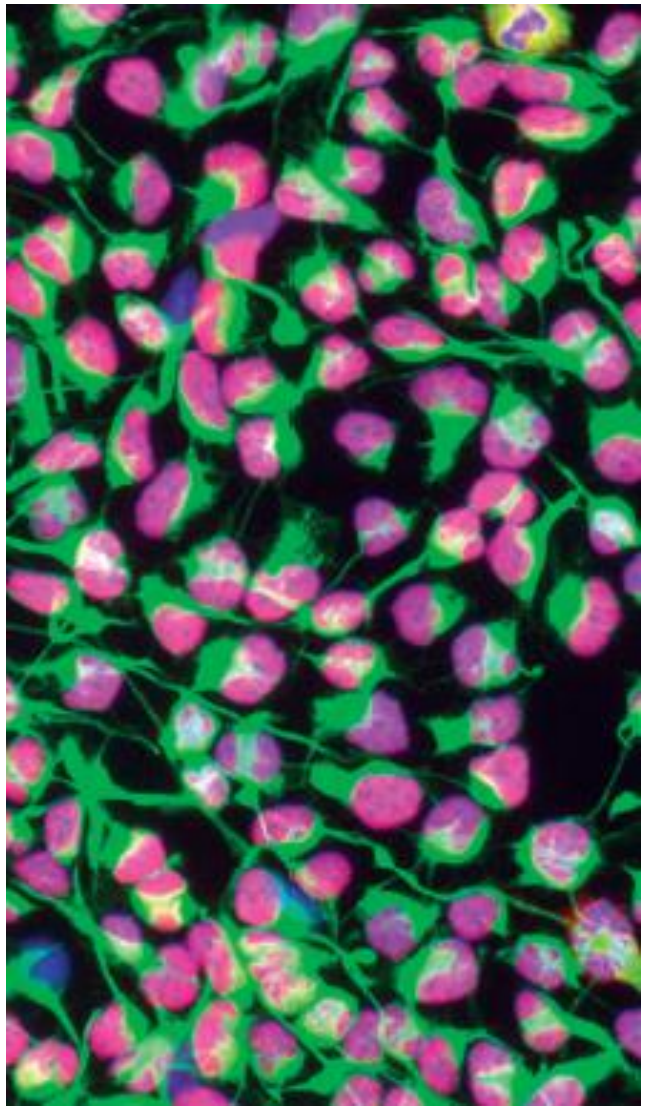


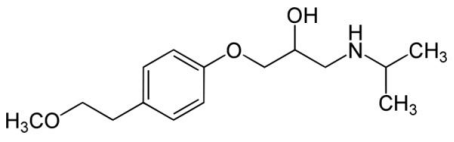
Regulating ATMPs as medicinal products

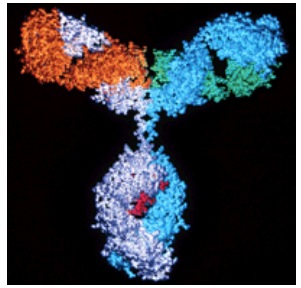
RSNN-EMA workshop, 17-11-2020

Delphi Coppens, PhD

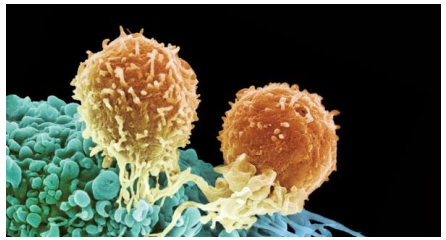
Dutch Cancer Society

Advanced Therapy Medicinal Products

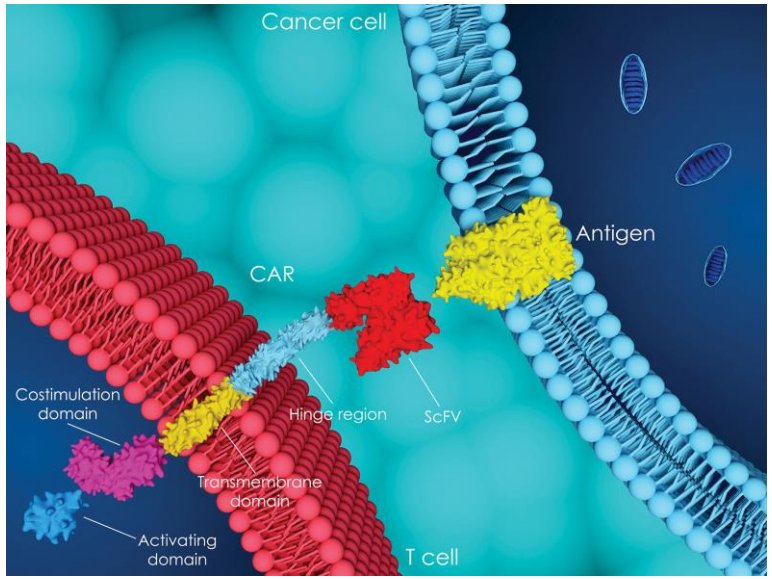




Alfred Pasieka/Science Photo Library



Scott Tysick/Getty Images;
Steve Gschmeissner/Science Source

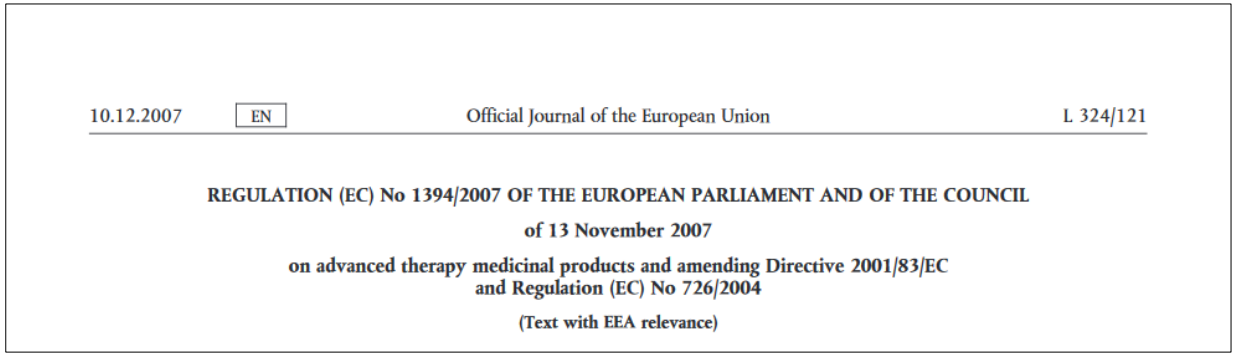


Samantha Zaroff/Genetic Engineering & Biotechnology News

Strategies for regulatory change

1. Stretch existing boundaries	**2. Implement new regulations**
❑Regulations aimed at marketing authorization (MA) ❑Aimed at commercial development and market entry of new ATMPs as medicinal products	❑Regulations that exempt ATMPs from MA ❑Aimed to facilitate treatment in clinical practice

1. Regulatory change – ATMP Regulation for MA



10.12.2007 EN Official Journal of the European Union L 324/121

REGULATION (EC) No 1394/2007 OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL

of 13 November 2007

on advanced therapy medicinal products and amending Directive 2001/83/EC and Regulation (EC) No 726/2004

(Text with EEA relevance)

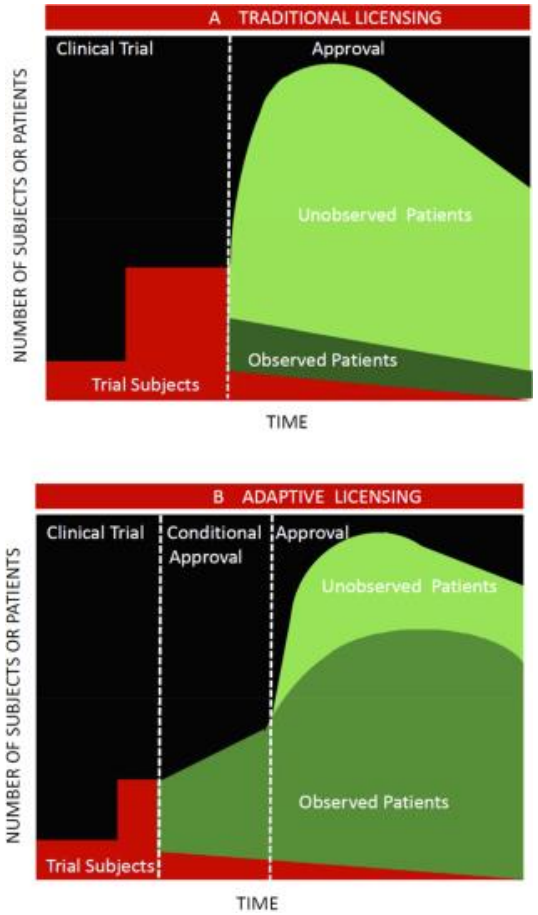
- Committee for Advanced Therapies (CAT)
  - Support CHMP
  - Scientific advice
- Amended Directive 2001/83/EC
  - High-level requirements for ATMP authorization & Guidelines
  - Risk-based approach
  - Hospital Exemption (HE)
- Incentives to stimulate commercial development
  - Fee reductions for scientific advice
  - ATMP classification procedure
  - Certification procedure for quality and preclinical data (SMEs)

1. Regulatory decision-making for MA

		Considerations for approval				Outcomes	Post-marketing study obligations		
	Product	Study design	Clinical benefit	Safety profile	Unmet medical need	Restricted labeling	Quality	Efficacy	Safety
US	Laviv	Δ	Δ	Δ	□	■			□
	Gintuit	Δ	■ ■	■	□	■	■	■	■
	MACI	■	■ ■	■	□	Δ	■	■	■
	Imlygic	□	Δ	■	■	■ ■			■
	Provenge	Δ	■ ■	Δ	Δ	■			□ Δ
	Kymriah	■	■ ■	□	Δ	□	Δ		Δ
	Yescarta	Δ	■	□	■	■			□ Δ
EU	ChondroCelect	□	■	■	□	■	Δ	Δ ■	Δ
	Imlygic	Δ	Δ	Δ	Δ	■		■	□ ■
	MACI	■	■	■	□	Δ		■	■
	Provenge	Δ	■ ■	Δ	■	■	■	■	□ Δ
	Strimvelis	Δ	■ ■	□	■	□		□ Δ	□ Δ
	Spherox	Δ	■	■	□	■	■	■	■
	Holoclar	Δ	■ ■	□	■	Δ	■	□ ■	□ ■
	Zalmoxis	Δ	Δ	■	■	□		□ Δ ■	□ Δ ■
	Glybera	Δ	□	□	■	■	■	■	□ ■
JP	Temcell	□	Δ	□	■	■		Δ	Δ
	Heartsheet	□	□	□	■	■	■	■	■

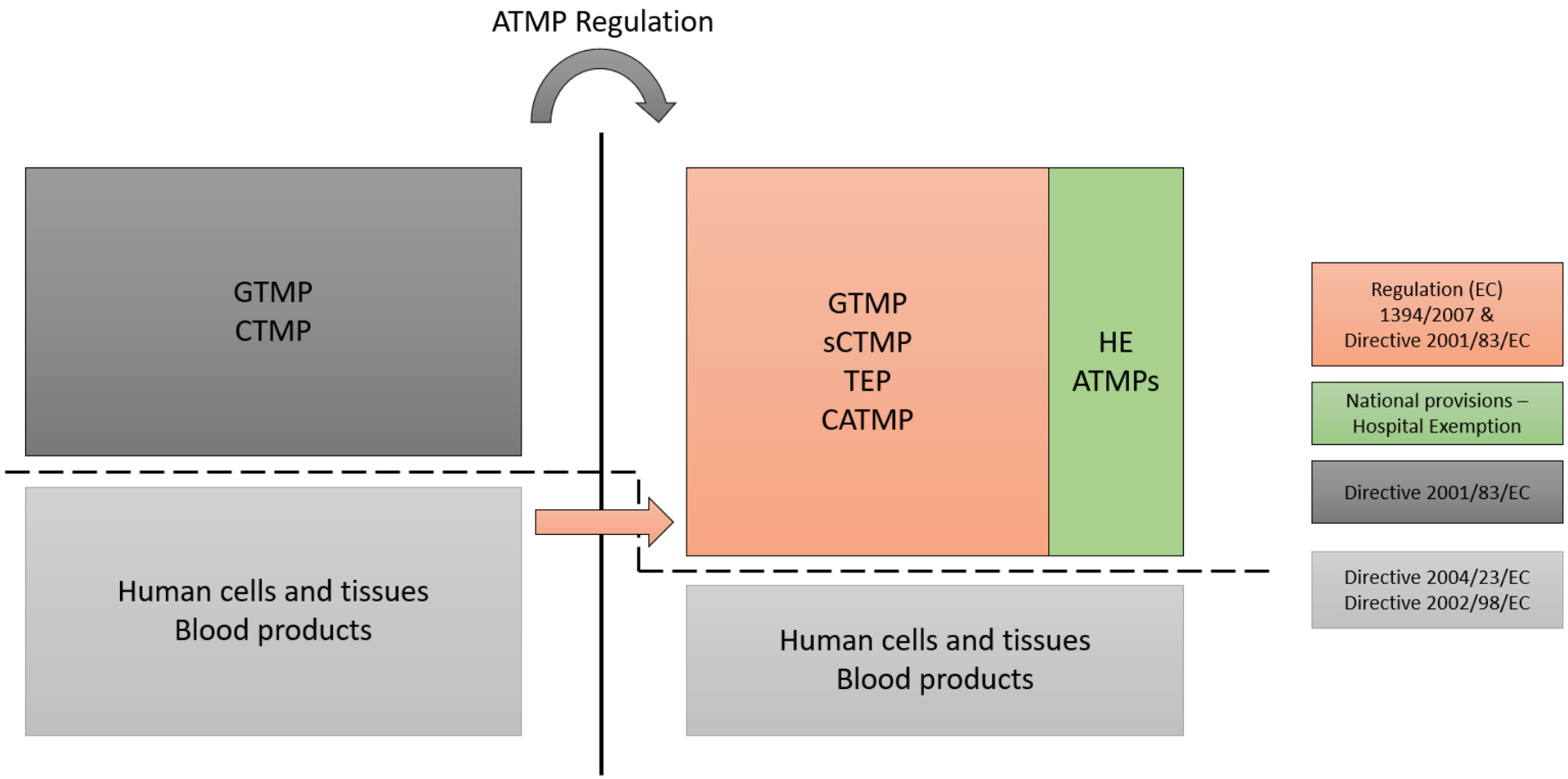
2008-2017

Coppens et al (2018) Cytotherapy

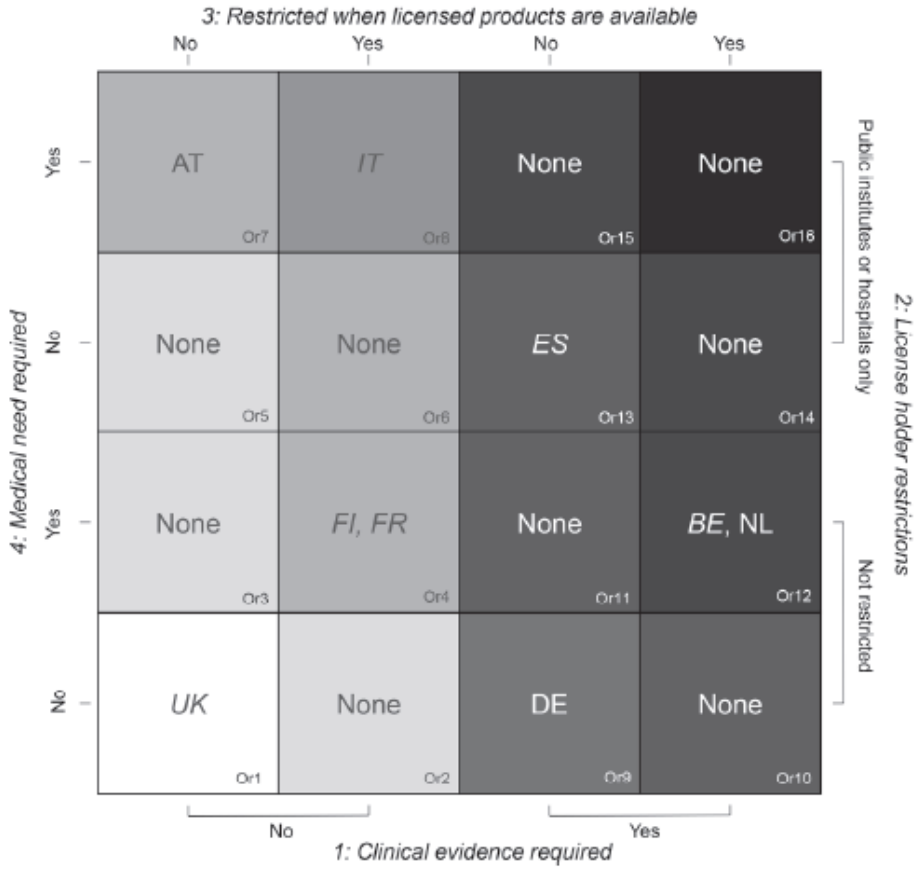


Oye et al (2016) CPT

2. Regulatory change - the Hospital Exemption



2. Regulatory change – use of the Hospital Exemption in clinical practice

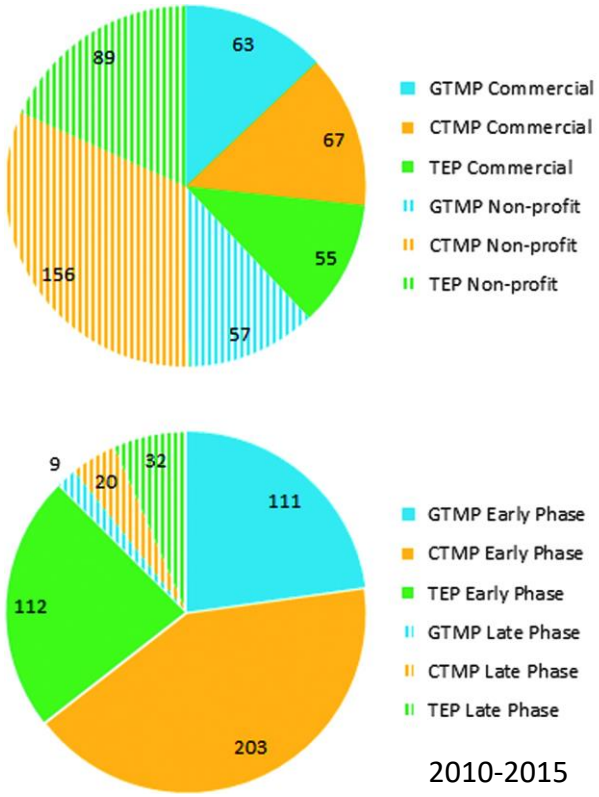


Coppens et al (2020) Regen Med

	Public facilities		**ATMPs (public)**	
	Recipients (n)	Respondents (n)/ Response rate (%)	HE (n) 2009-2017	NPU (n) 2015-2017
Belgium	7	3 (43%)	0	0
Finland	2	1 (50%)	1	0
France	1	1 (100%)	0	0
Germany	22	3 (14%)	3	0
Italy	5	3 (60%)	2	5
Netherlands	9	8 (89%)	6	2
United Kingdom	21	8 (38%)	0	4
Total	67	27 (40%)	12	11

Coppens et al (2020) Cytotherapy

Way forward



Boráň et al (2017) Hum Gene Ther Clin Dev

❑ Optimized clinical developments
- Improve transfer from pre-competitive to competitive environment
- Early scientific advice
- Knowledge dissemination
- Public-private engagements

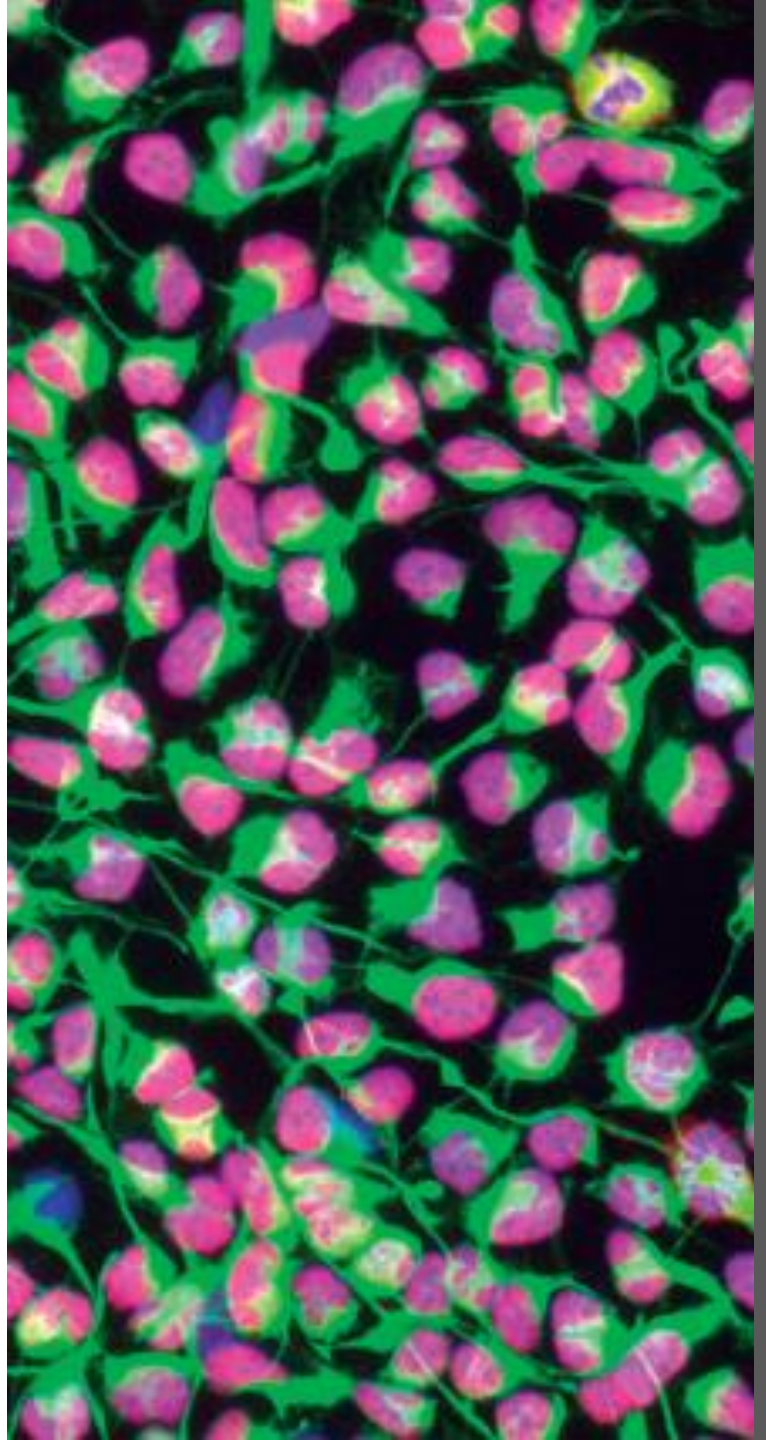
❑ EU-wide public registries
- Insight into available ATMPs (clinical trials & HE)
- Continuous long-term surveillance and data collection
- Use MA and HE pathways in a complementary fashion

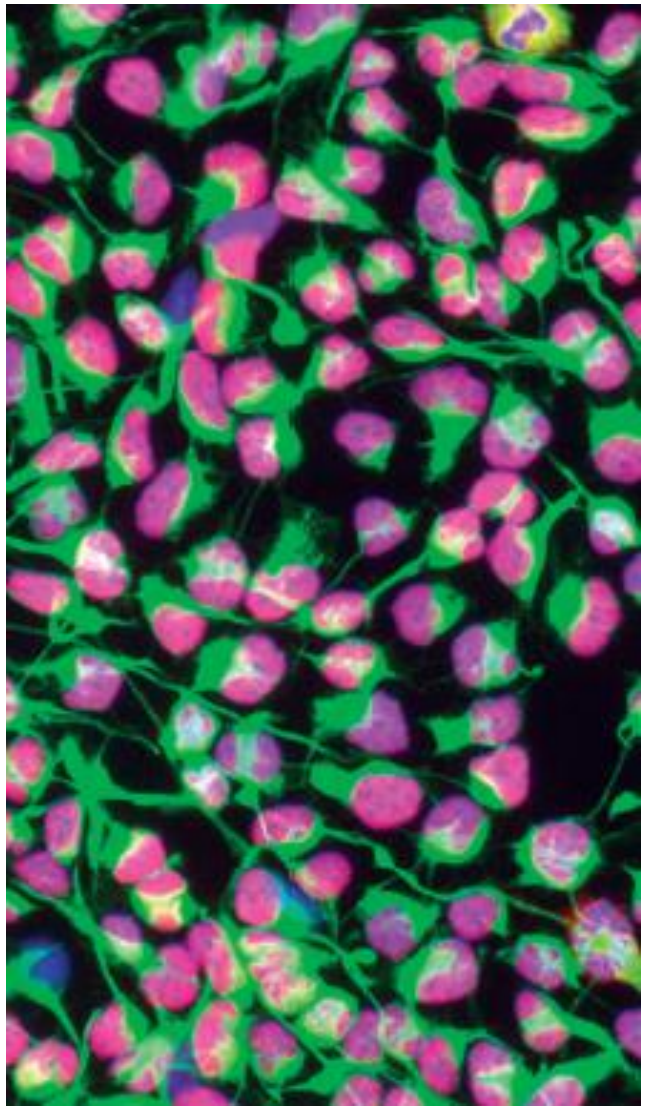
➢ More downstream hurdles:
- Global distribution and scaling up issues
- Reimbursement

Conclusion

- ❑Shift to early market access and enhance post-marketing surveillance & implementation of exemption pathways

- ❑More centralized knowledge dissemination and collaborative efforts could catalyse availability and benefit patients on a regional and global level
  - Regulators
  - Scientists, pharmacists & medical doctors
  - Industry
  - Coordinating bodies





Questions?

dcoppens@kwf.nl