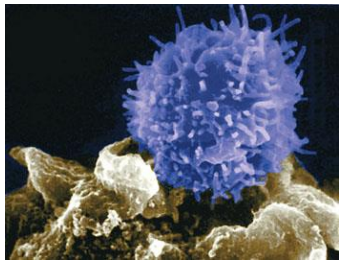
Lääkealan turvallisuus- ja kehittämiskeskus | Säkerhets- och utvecklingscentret för läkemedelsområdet | Finnish Medicines Agency

ATMP-valmisteet ja Fimean rooli ATMP-valvonnassa Suomessa ja EU:ssa

Heli Suila, FT
Erikoistutkija, BIO-jaosto
Farmaseuttis-biologinen-yksikkö
Lääkevalmisteiden arviointi
Lääkealan turvallisuus- ja kehittämiskeskus Fimea

Pitkälle kehitetyn terapian lääkkeet (Advanced Therapy Medicinal Products) (ATMPs)



Soluista lääkkeeksi

- Verensiirto
  (1600-luku)

- Elinsiirrot
  (1800-luvun loppu)

- Kantasolusiirteet
  (ensimmäinen HSCT 1939)

- Soluterapia

- Kudosmuokkaus

- Geneettisesti muokatut solut

Classification as medicines

Cells or tissues are medicines if they are considered ‘engineered’:

- the cells or tissues have been **subject to substantial manipulation**
- the cells or tissues are **not intended to be used for the same essential function** in the recipient as in the donor (non-homologous use)

Safety perspective:

- Cells may have different properties after treatment
- Cells used in different fysiologic purpose or in different environment from their origin

Advanced therapy medicinal products, ATMPs (Dir. 2001/83 & regulation 1394/2007)

- Gene therapy medicinal products (GTMPs)
  - contain genes that lead to a therapeutic, prophylactic or diagnostic effect. They work by **inserting 'recombinant' genes into the body**, usually to treat a variety of diseases, including genetic disorders, cancer or long-term diseases.
- Somatic cell therapy medicinal products (sCTMPs)
  - contain cells or tissues that have been manipulated to change their biological characteristics or cells or tissues intended to be used for **a function that is different to its original essential function** in the body. They can be used **to cure, diagnose or prevent diseases**.
- Tissue engineering products (TEPs)
  - contain cells or tissues that have been modified so they can be used to **repair, regenerate or replace** human tissue
- Combined advanced-therapy medicines:
  - these contain one or more **medical devices as an integral part of the medicine**. An example of this is cells embedded in a biodegradable matrix or scaffold.

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

21 May 2015
EMA/CAT/600280/2010 rev.1
Committee for Advanced Therapies (CAT)

Reflection paper on classification of advanced therapy medicinal products

Draft Agreed by CAT	June 2014
Adoption by CAT for release for consultation	20 June 2014
Start of public consultation	30 June 2014
End of consultation (deadline for comments)	31 October 2014
Draft Agreed by CAT	13 May 2015
Adoption by CAT	22 May 2015

Keywords	*ATMP classification, Gene therapy, Somatic cell therapy, Tissue engineered Products, Combined ATMPs*

Fimean rooli ATMP-valvonnassa Suomessa ja EU:ssa

ATMP-valmisteisiin liittyvät luvat ja valvonta Suomessa

• **GMO**	**Geenitekniikan lautakunta / Valvira**
• **Kansallinen valmistuslupa**	**Fimea**
• **Kliiniset lääketutkimukset**	**Fimea ja Eettinen komitea**
• **Tieteellinen neuvonta**	**Fimea / EMA**
• **Toimijoiden valvonta (GxP)**	**Fimea**
• **Keskitetty myyntilupa**	**Kansalliset virastot, EMA/ EU-Komissio**

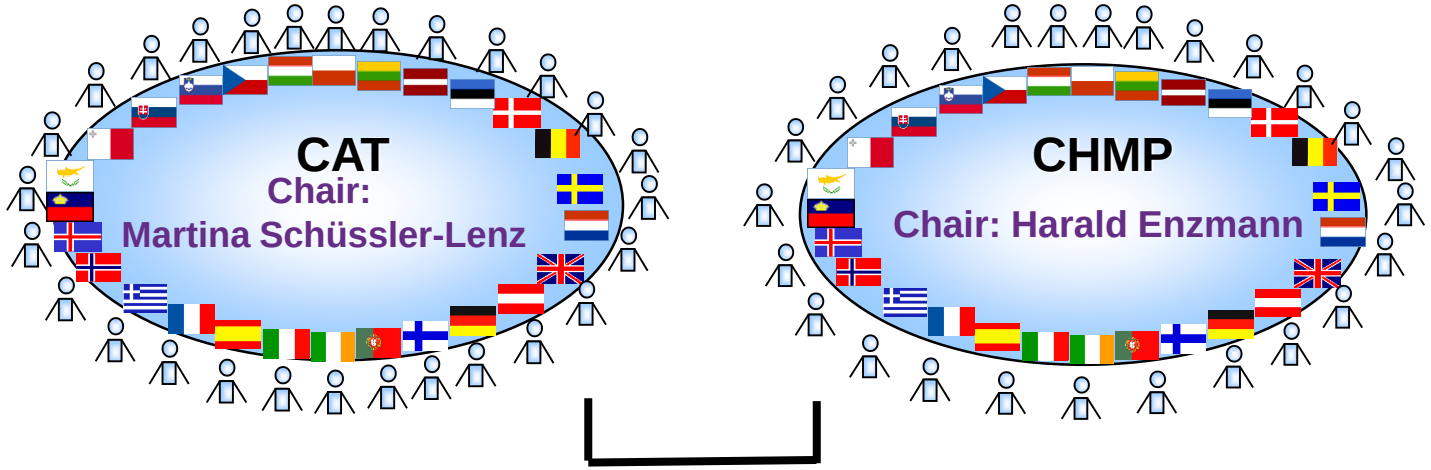
Lääkealan turvallisuus- ja kehittämiskeskus Fimea lyhyesti

- Sosiaali- ja terveysministeriön alainen lääkealan lupa- ja valvontaviranomainen.
- Fimea valvoo lääkkeitä, veri- ja kudostuotteita sekä kehittää lääkealaa.
- Henkilöstöä noin 250
- Henkilöstöämme työskentelee Kuopiossa, Helsingissä, Tampereella, Turussa ja Oulussa
- Myyntilupa-asiat, Tuotevirhetapaukset, Erityisluvat, Huumausaineiden vienti- ja tuontilupapäätökset
- Fimea on osa eurooppalaista lääkevalvonnan viranomaisverkostoa.

Fimea ja EU

- Euroopan lääkeviraston (EMA) työryhmät ja komiteat
- EU-komission työryhmät
- Hakeudumme aktiivisesti kansainvälisiin myyntilupa- ja valvontatehtäviin
- Neuvomme lupaavia lääkeinnovaatioita osana EU-innovaatioverkoston toimintaa (EU Innovation Network)

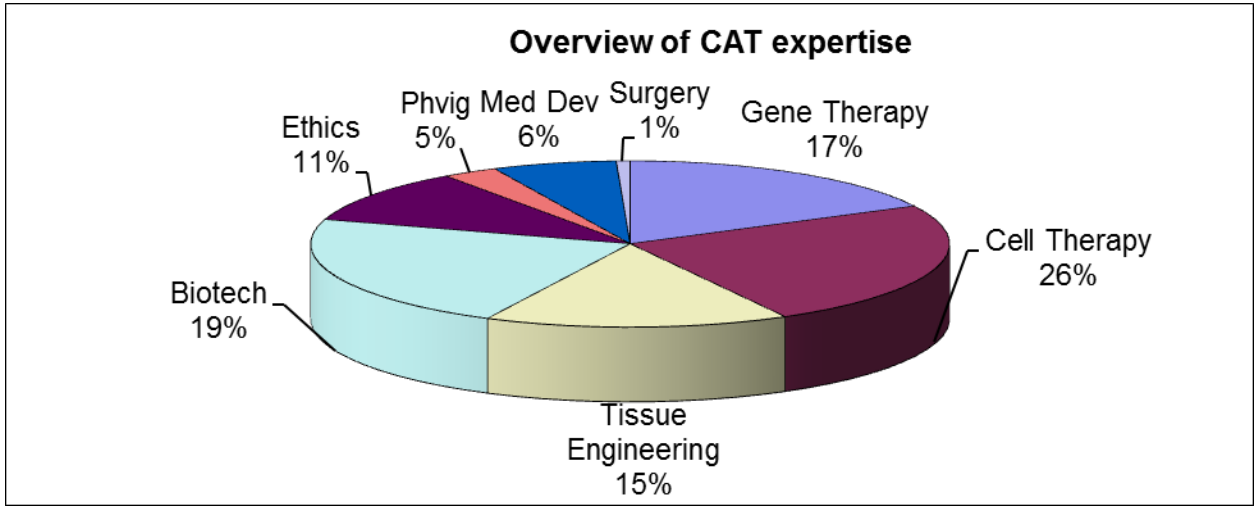
EMA Committees for ATMPs



5 „double members“

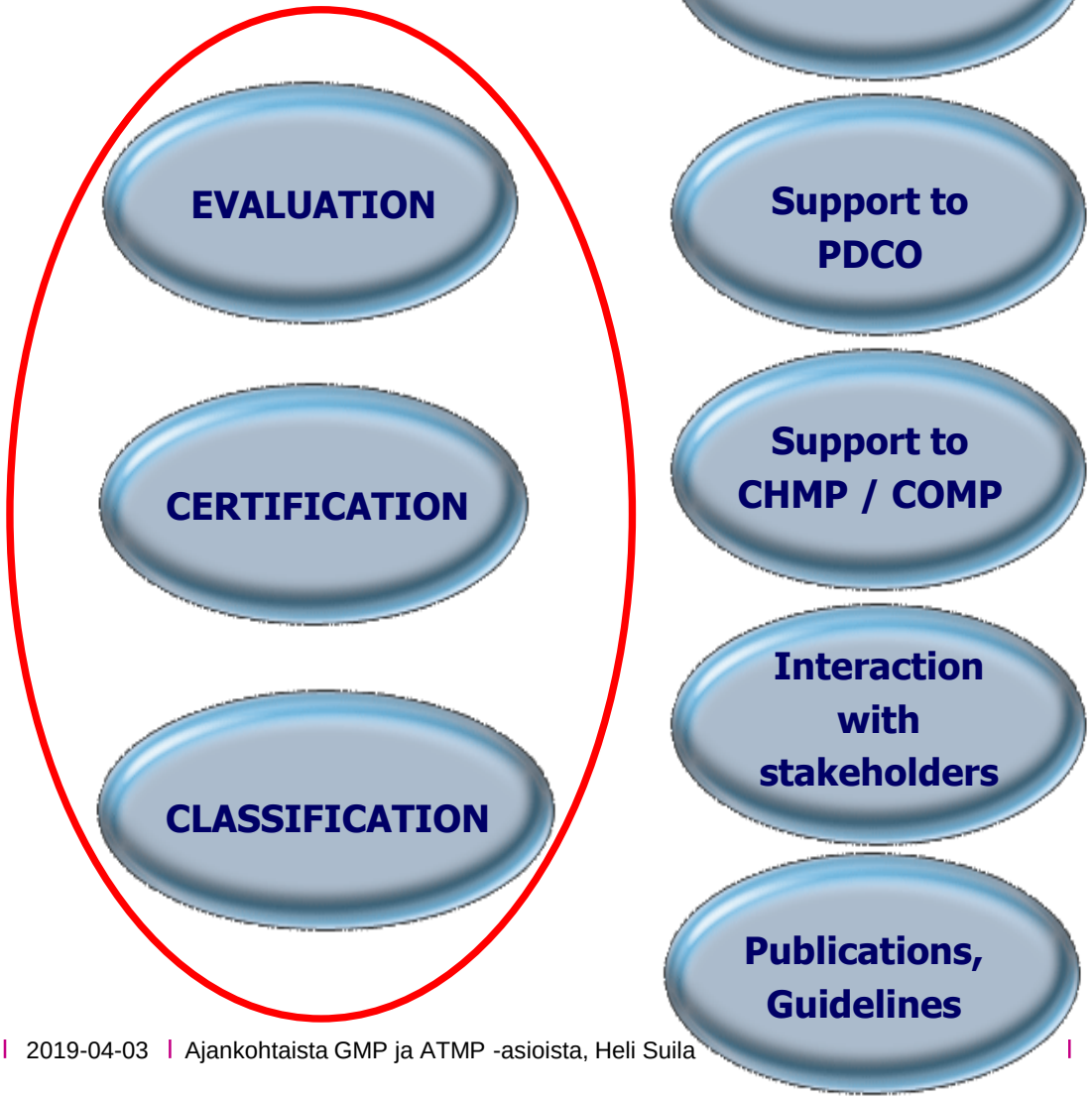
Total max. **68** experts (64/Oct. 2018):

- quality experts
- non-clinical experts
- clinical experts
- patient representatives
- other (scientists etc.)



Tasks of the CAT

TASKS DEFINED PER LEGISLATION



- DRAFT Guideline on quality, non-clinical and clinical aspects of medicinal products containing genetically modified cells
  (End of consultation / deadline for comments 31 July 2019)

- DRAFT Guideline on quality, non-clinical and clinical requirements for investigational advanced therapy medicinal products in clinical trials (End of consultation / deadline for comments 1 August 2019)

- Guideline on the quality, non-clinical and clinical aspects of gene therapy medicinal products (revised GL March 2018)