EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EU Support to ATMP Developers

CAT workshop on Cell-based Cancer Immunotherapy Products

Presented by Patrick Celis
CAT Secretariat

Overview

- Support to ATMP development by European Commission (DG RTD)

- Support by European Medicines Agency and National Competent Authorities (NCAs) for Medicines

Funding instruments within Horizon 2020

H2020 pillars

- Excellent Science
- Industrial Leadership
- Societal Challenges

Public-private partnership with big pharma

imi innovative medicines initiative

Collaborative projects

Public-public partnerships with EU Member States & beyond

AAL PROGRAMME

JPND research

jpiamr

SME Instrument

Grants for small businesses

Blue sky research

erc European Research Council

InnoFin EU Finance for Innovators

Loans for small and big R&I companies

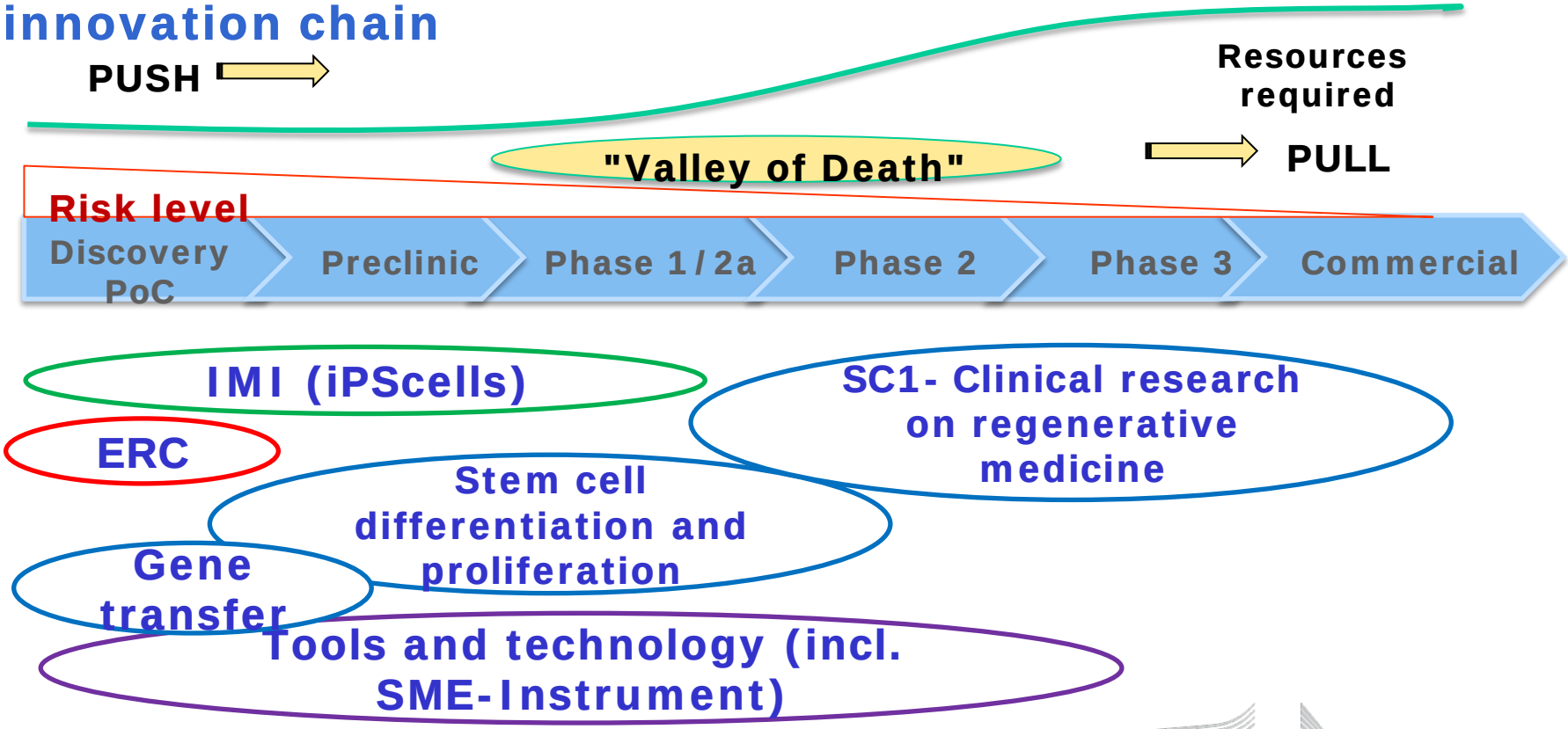
ESFRI European Strategy Forum on Research Infrastructures

InnoLife eit Health

Knowledge triangle: Higher education, business, R&I

European Commission

ATMP development: targeting different points of the innovation chain



How much?- Regenerative Medicine research

- ✓ **2007-2013 (FP7)**

  Gene therapy / Gene transfer

  > € 240 million (collaborative projects, ERC, MSC grants)

  Cell Therapy

  > € 460 million (collaborative projects)

- ✓ **2014-2020 (Horizon 2020**, to date):

  Gene therapy / Gene transfer

  € 30.5 million for 5 collaborative projects

  Cell Therapy

  € 114 million for 20 collaborative projects

More info at RTD: C. Kessler, D. Gancberg, J. Sautter

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When?- Opportunities in 2017

Two topics open in forthcoming calls for proposals…

1. Clinical research on regenerative medicine

- SC1-PM11-2016-2017
- Clinical research –any stage;proof of regulatory engagement
- Collaborative research – min 3 EU/assoc countries
- Indicative budget: €30 million (2017)
- ~ €4-6 million per project – depending on work
- Deadline: 11 April 2017

2. Cell technologies in medical applications

- SMEInst-05-2016-2017 "Supporting innovative SMEs in the healthcare biotechnology sector"
- SME Instrument – 1 SME only (can subcontract)
- Phase 1 – Feasibility study (€50,000 lump sum)
- Phase 2 – Research/Innovation (~€1-5 million depending on work)
- Indicative budget:
  - 2016: €35 million
  - 2017: €80 million
- Multiple deadlines

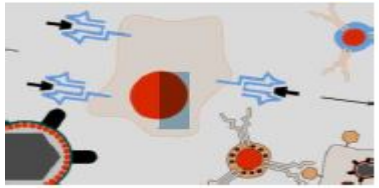
IMI interest in Advanced Therapies

1. **Consultative workshop on advanced therapies** IMI Stakeholder Forum, Brussels, 29 September 2016

   http://www.imi.europa.eu/events/2016/06/24/imi-stakeholder-forum-2016

2. **ADAPT SMART** Accelerated Development of Appropriate Patient Therapies - a Sustainable, Multi-stakeholder Approach from Research to Treatment-outcomes

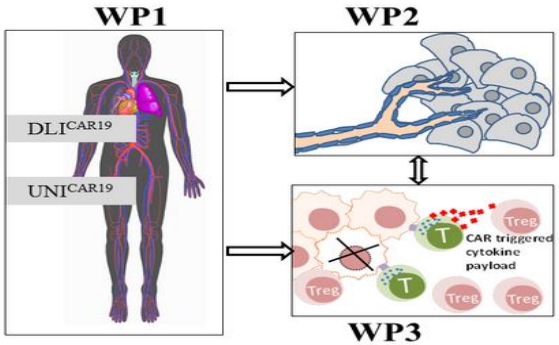
   http://adaptsmart.eu/



ATECT: Advanced T-cell Engineering for Cancer Therapy

SEVENTH FRAMEWORK PROGRAMME

- CAR T cells: TALEN-mediated gene editing strategies alongside genetic modification with integrating vectors for treatment of CD19+ refractory lymphoma.
- Strategy for creation of universal engineered T-cells.
- Engineer CAR T-cells to be resistant to the hostile microenvironment.
- Utilise endothelial cues of neo-angiogenesis to direct CAR T-cell migration and activity.
- Performance of a phase I clinical trial.



Philogen
innovating targeting

NETHERLANDS CANCER INSTITUTE
ANTONI VAN LEEUWENHOEK

UCL

University of Zurich UZH

cellectis

https://atect-fp7.org/

Coordinator: Dr. Martin Pule (UCL)

HORIZON 2020

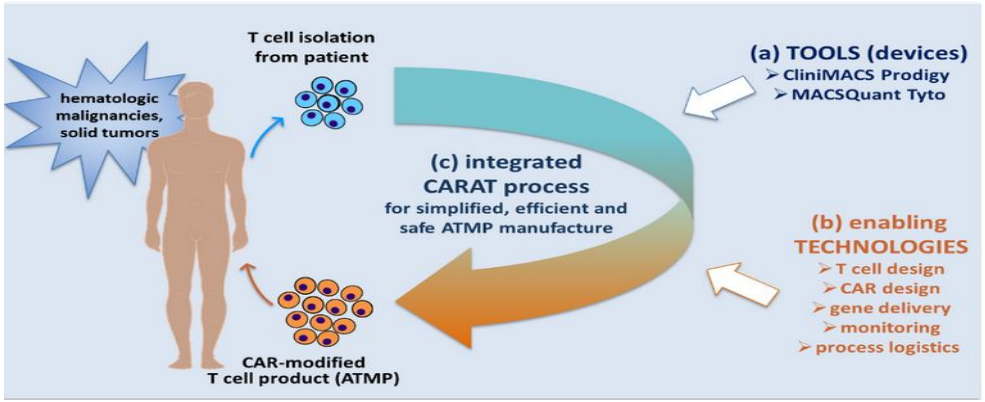
European Commission

CARAT: Chimeric Antigen Receptors (CARs) for Advanced Therapies

HORIZON 2020

Goal: Development of a platform that will enable the **automated, safe,** and **cost efficient manufacture of more effective CAR-modified T-cells** for personalized treatment of cancer patients.

Start: 01 January 2016
EC contribution: € 6 million



http://carat-horizon2020.eu
Coordinator: Dr. Andrew Kaiser (Miltenyi)

HORIZON 2020

European Commission

Support by EMA and NCA

1. Early interactions with Regulators
   - EMA's Innovation Task Force and Innovation Offices in NCA
   - SME office
2. Advanced Therapy Medicinal Product (ATMP)-specific support by CAT:
   - Classification and Certification
3. Scientific Advice (by EMA and NCA)
4. Support for early access:
   - PRIME (Priority Medicines)

Support to ATMP developers - Interactions with EMA/CAT

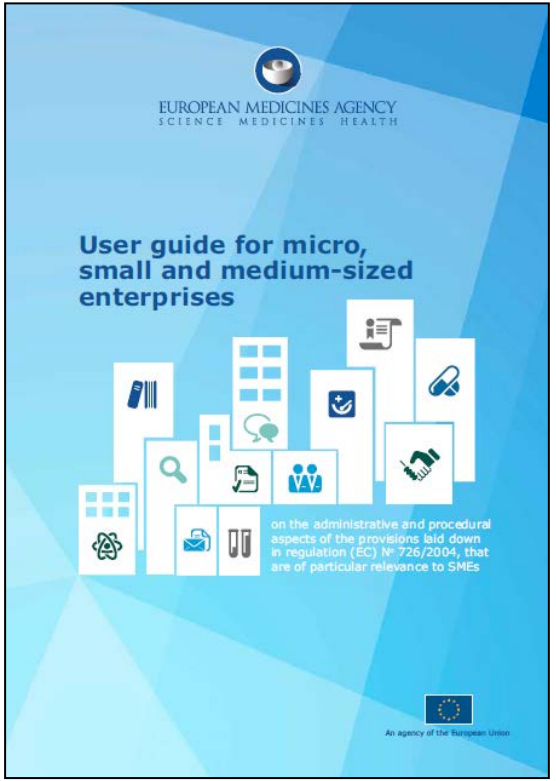
- Briefing meeting with **EMA Innovation Task Force**
  - Platform for early dialogue on scientific, regulatory and legal requirements
  - Informal, not binding
  - EMA staff + members for Committees & working parties
  - Since 2009: 82 meetings with ATMP developers; 57 with participation of CAT or WP members.

- **Innovation offices in NCAs** – direct interactions with national regulators to discuss early development questions

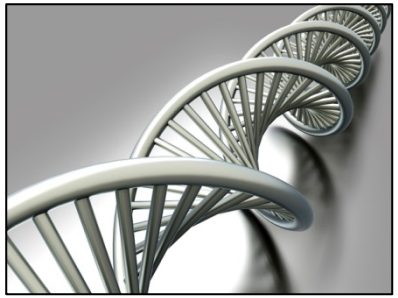
Picture: www.upwardsleader.com

Support to ATMP developers - Interactions with EMA/CAT

- **SME office**
  - Administrative / regulatory and financial support to SME companies
  - SME office will
    - facilitate communications with dedicated EMA staff to respond on practical / procedural issues
    - Organise workshops and training
  - SME user guide
  - sme@ema.europa.eu



ATMP-specific procedures



- **ATMP classification**
  - Incentive: early / regulatory certainty
  - Open to all applicants – free-of-charge
  - Scientific recommendation from CAT on the regulatory classification of their ATMP

- **ATMP certification**
  - Incentive: (early)-late / scientific certainty
  - Only for SMEs
  - Scientific evaluation by CAT of quality / manufacturing (CMC) data (Module 3) and non-clinical data (Module 4)

    → 'pre-assessment' of data already generated

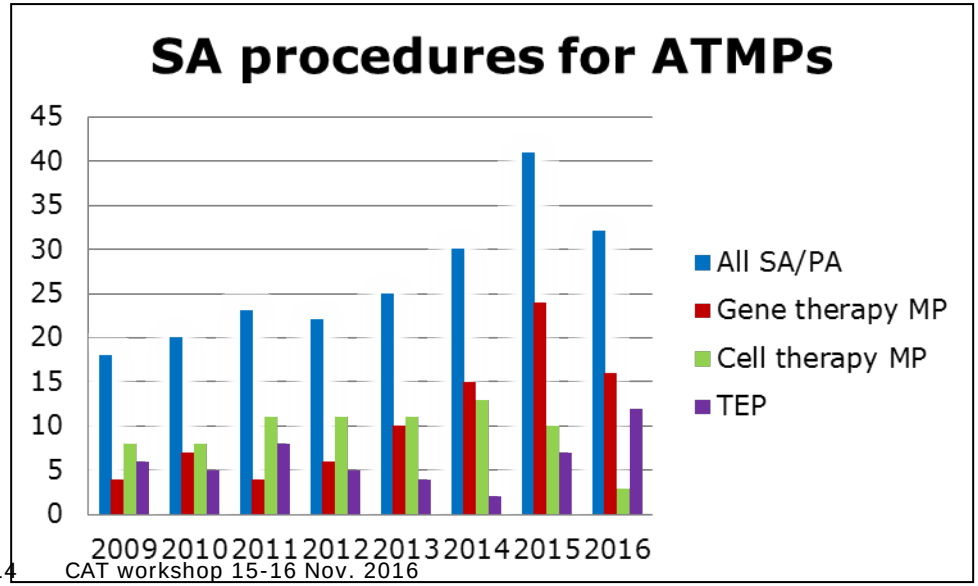
Picture www.raps.org

Scientific Advice

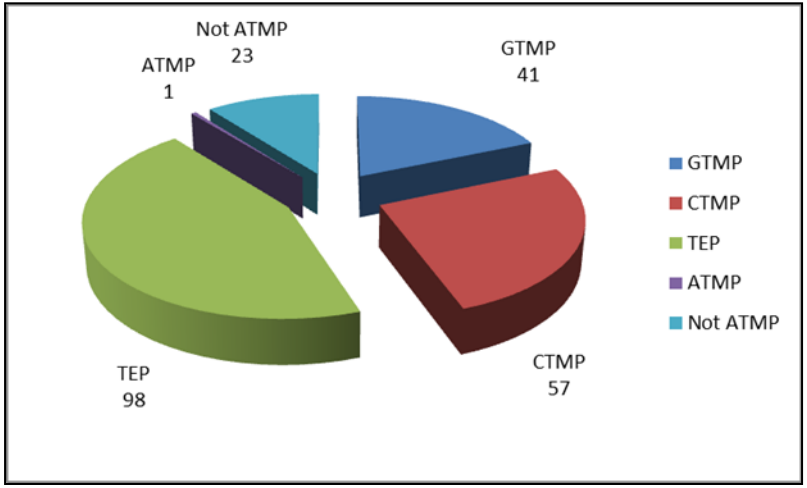
- Incentive: Early-late, scientific certainty
- Open to all applicants
  - Reduced fee for ATMPs
- Advice on future development steps (Q/NC/C)
  - Prospective, no assessment of data
- Advice on post-authorisation safety studies
- Possibility for
  - Joint advice from Regulators /Health Technology Assessment (HTA) bodies
  - Parallel scientific advice with FDA
- Scientific advice for ATMPs is given by the scientific advice working party of the CHMP in collaboration with the CAT

CAT Experience (Jan 2009 – August 2016)

- ATMP classifications (220)
- ATMP certification (7)
- ATMP scientific advice (205)



Finalised ATMP classifications



PRIME (Priority Medicines)

- To foster development of medicines with a **high public health potential**
  - Reinforced scientific and regulatory advice
  - Optimise development for robust data generation
  - Enable accelerated assessment

- Eligibility to PRIME:
  - Potential to address an **unmet medical need**
  - **Scientific justification** based on data / evidence from non-clinical and clinical development

Features of the PRIME scheme

Early access tool, supporting patient access to innovative medicines.

- **Written confirmation of PRIME eligibility** and potential for accelerated assessment;
- **Early CHMP/CAT Rapporteur appointment** during development;
- **Kick off meeting** with multidisciplinary expertise from EU network;
- **Enhanced scientific advice** at key development milestones/decision points;
- **EMA dedicated contact point**;
- **Fee incentives** for SMEs and academics on Scientific Advice requests.

Prime Eligibilities for ATMPs

- 21 applications received, 7 eligiblities agreed

- Presubmission meetings
- Interactions with Rapporteurs

- Scientific Advice (Q, NC, C)
- PRIME

- ATMP certification
- Scientific advice (Q, NC)

- Early interactions with Regulators
- ATMP Classification

- R&D support (EU, National)

Thank you for your attention

Further information on the EMA procedures:

www.ema.europa.eu > Human Regulatory

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