1st EMEA Workshop on Advanced Therapy Medicinal Products

EMEA
Innovation Task Force
EXPERIENCE

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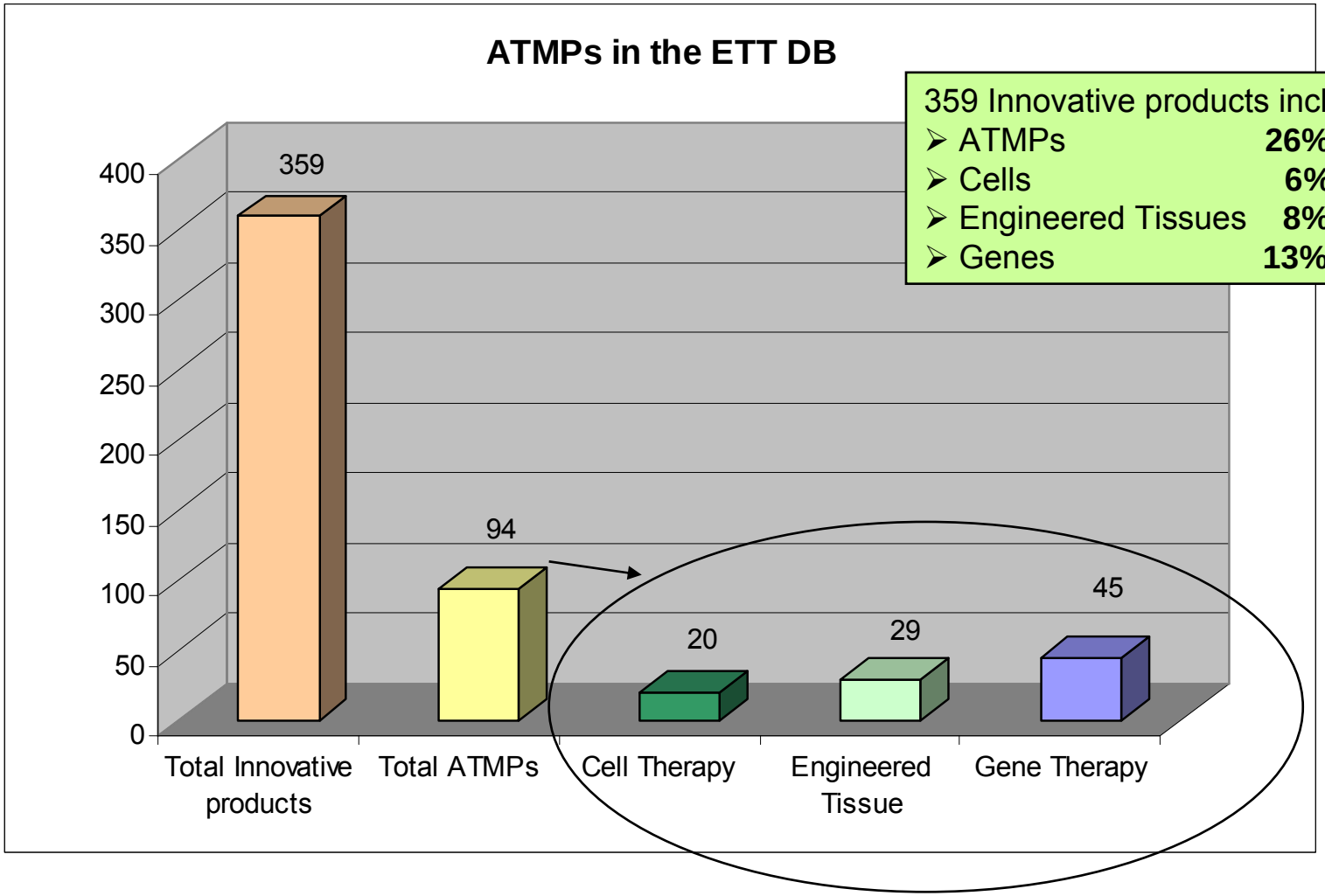
Innovation at the EMEA: the Innovation Task Force (ITF)

EMEA multidisciplinary group (2001-2008)

- ***"Soft landing zone"*** to establish early dialogue **(Briefing Meetings)** with sponsors of Emerging Therapies and Technologies (including advanced therapies).
- Involvement of Committees and Working parties to stimulate **awareness and learning in emerging therapies and technologies (thus including cells and nucleic acids based products).**
- Provide Regulatory **advice on eligibility** for access to EMEA pre-authorisation procedures **as medicinal products**, in conjunction with CHMP.
- Provide EMEA with a long term tracking of innovative products reaching the EMEA: **Emerging Therapies and Technologies (ETT) DB**.
- **NEW ! Contribute** to CAT EMEA scientific recommendation on the **classification of Advanced Therapy Medicinal Products.**

Emerging Therapies and Technologies (2001-2008) :
Experience at EMEA: focus on ATMPs(1/2)



ITF Briefing Meetings

► To establish early dialogue with stakeholders **(briefing meetings)** in order to **identify** scientific, legal and regulatory issues of Emerging Therapies and Technologies (including advanced therapies), in conjunction as appropriate with relevant EMEA Committees and Working Parties.

► also to **complement** and **reinforce** existing formal regulatory procedures (e.g. designation of orphan medicinal products, CHMP scientific advice etc).

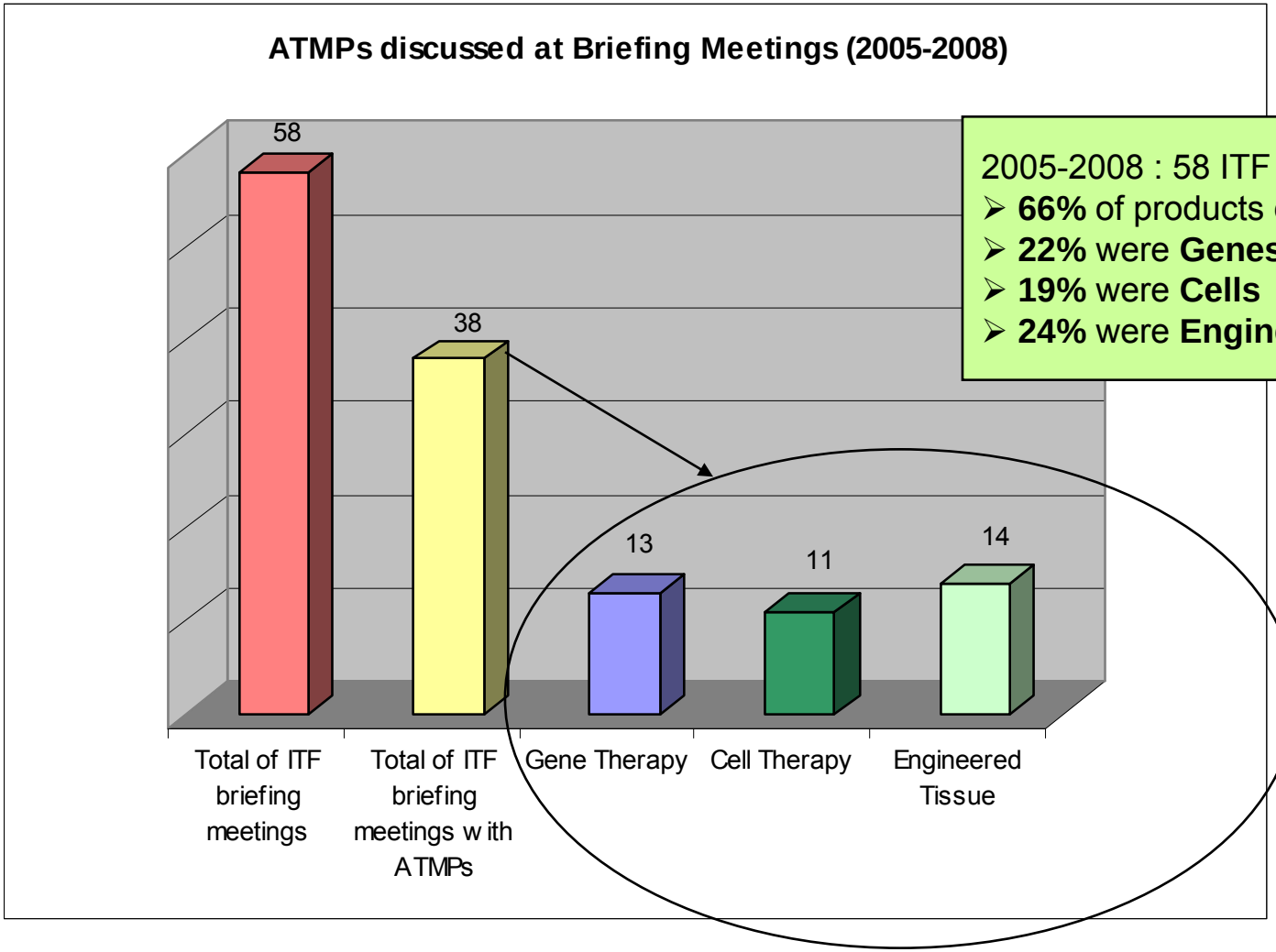
Issues discussed:

Profile of the product/technology

Key scientific and regulatory areas:

- **Development strategy/program: quality, safety, efficacy, manufacturing, ERA, Risk Management Plan (as applicable): questions on issues that need addressing**
- **Guidance towards relevant related guidelines, services (e.g. SMEs office) or scientific procedures (e.g. Scientific Advice, Orphan drug designation) to support the strategy of the company**
- **Identified areas for further reflection on the regulatory issues discussed**

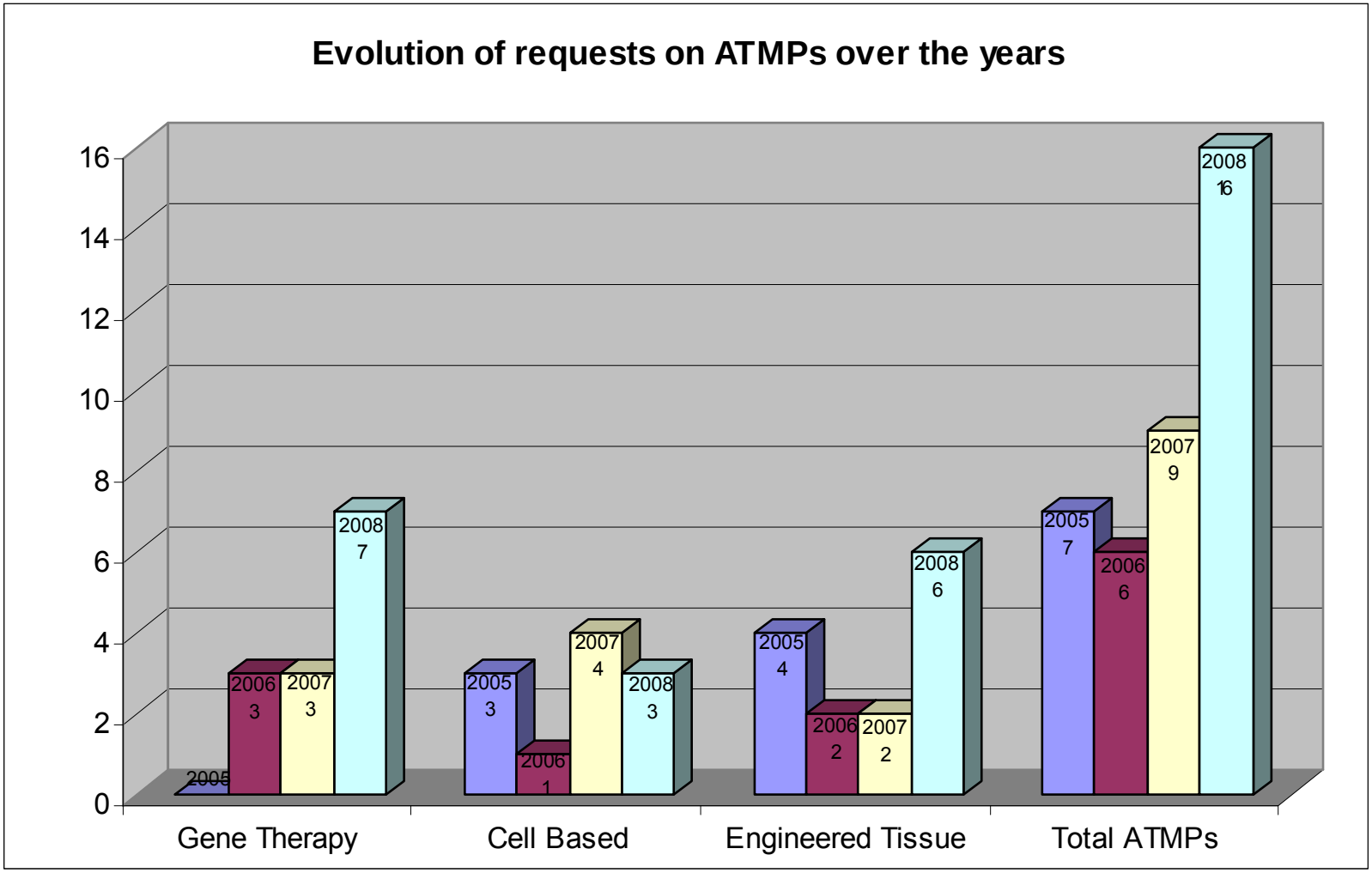
Briefing Meetings
Focus on: ATMPs



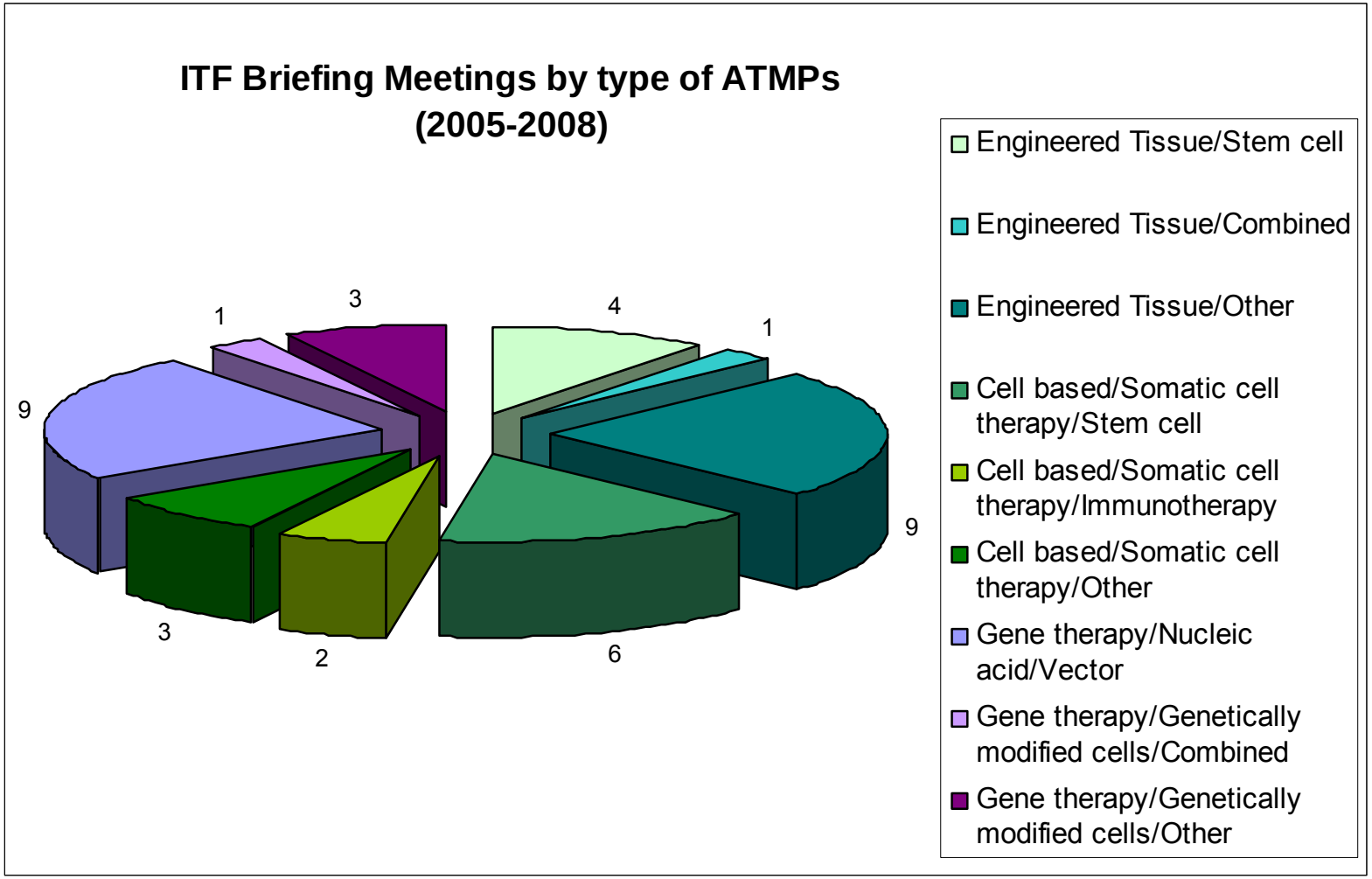
2005-2008 : 58 ITF briefing meetings

- **66%** of products discussed were **ATMPs**
- **22%** were **Genes**
- **19%** were **Cells**
- **24%** were **Engineered Tissues**

Briefing Meetings
Focus on: ATMPs

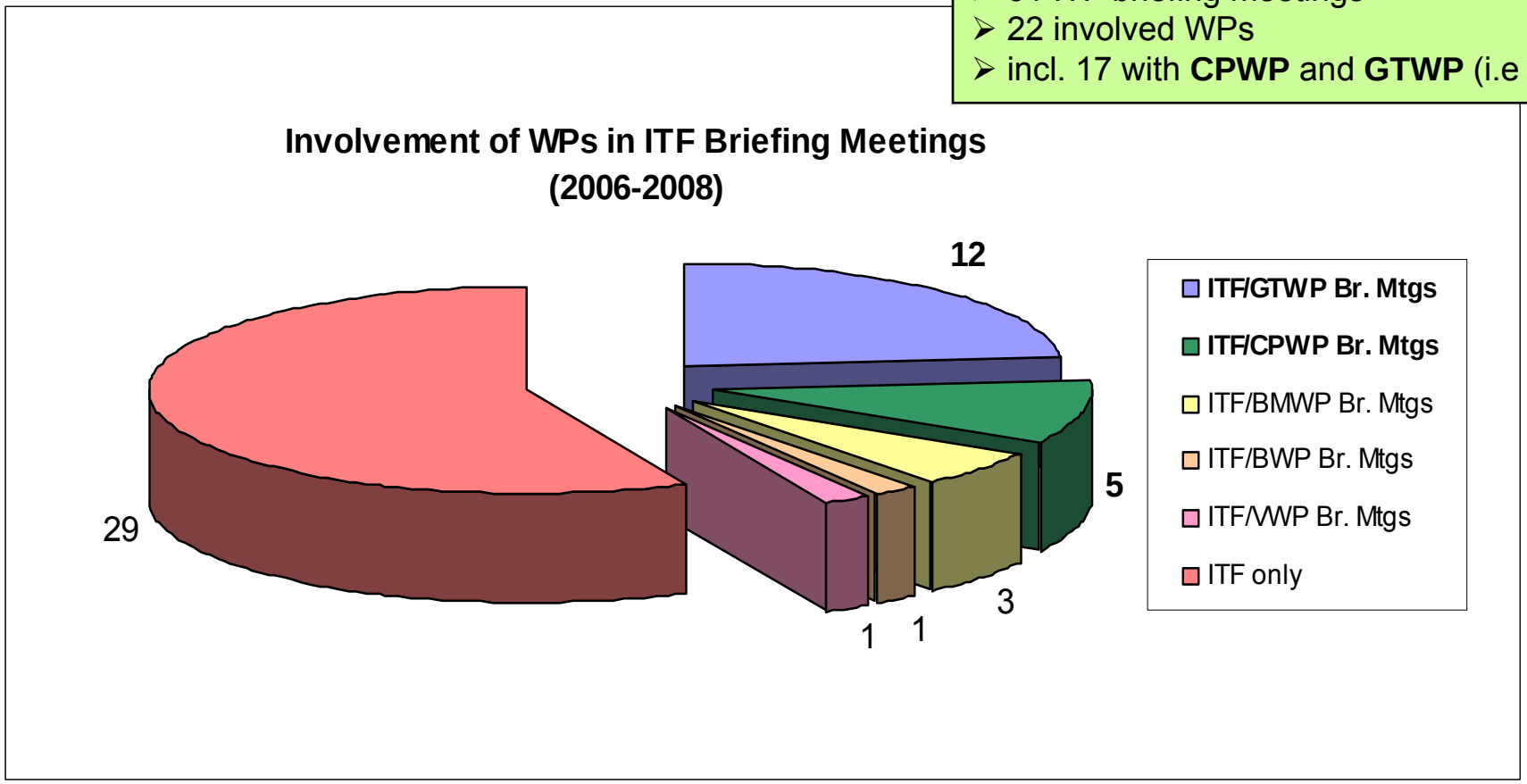


Briefing Meetings
Focus on type of ATMPs

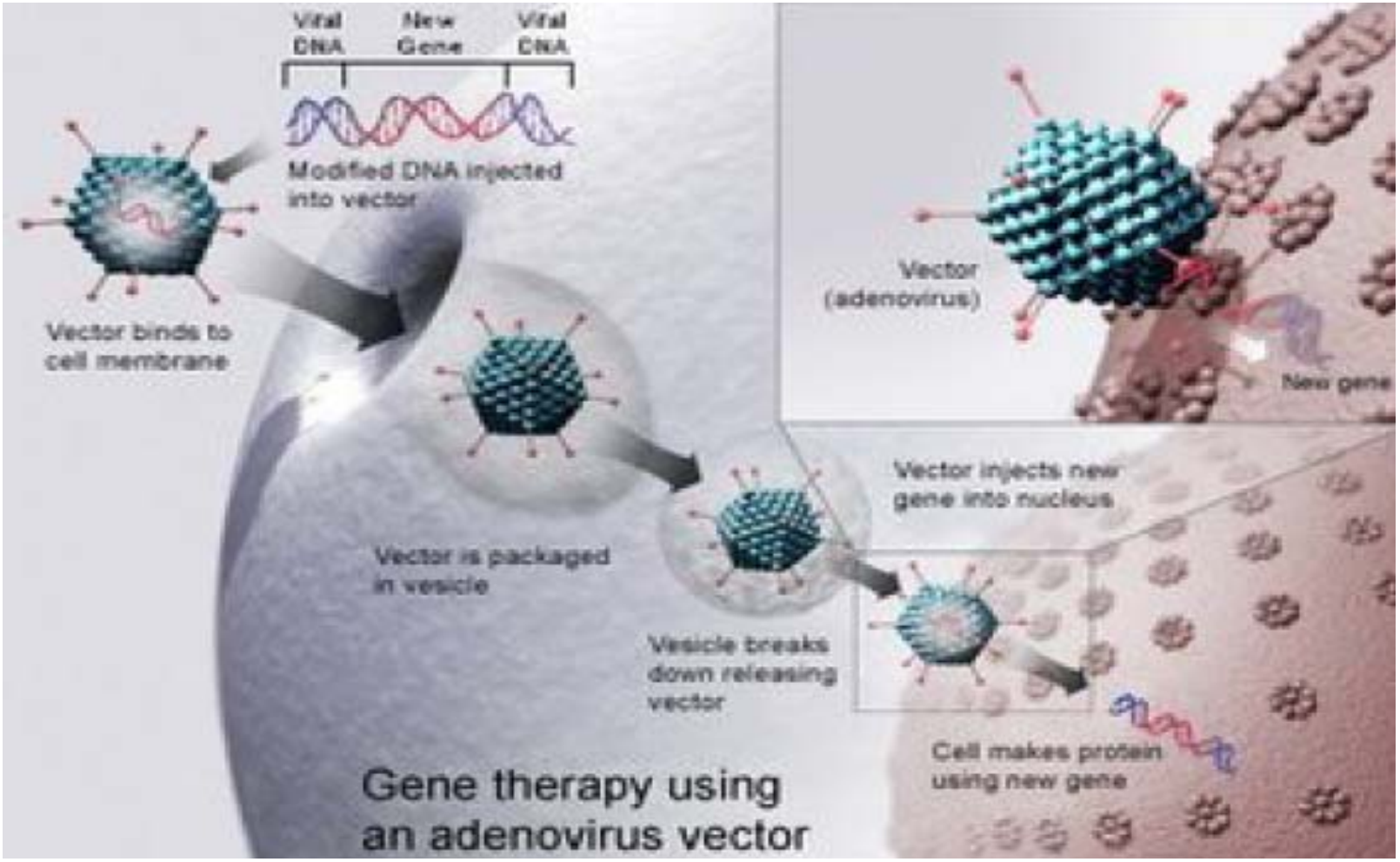


Briefing Meetings
Sharing experience with Working Parties

2006-2008
- 51 ITF briefing meetings
- 22 involved WPs
- incl. 17 with **CPWP** and **GTWP** (i.e **33%**)



Eligibility as a Medicinal Product
Focus on: Gene therapy



Gene Therapy Eligibility

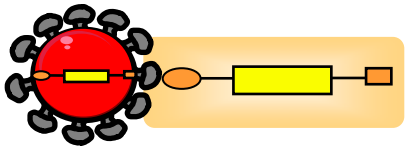
Ex-vivo gene therapy

cell line

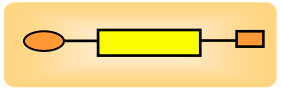
cell

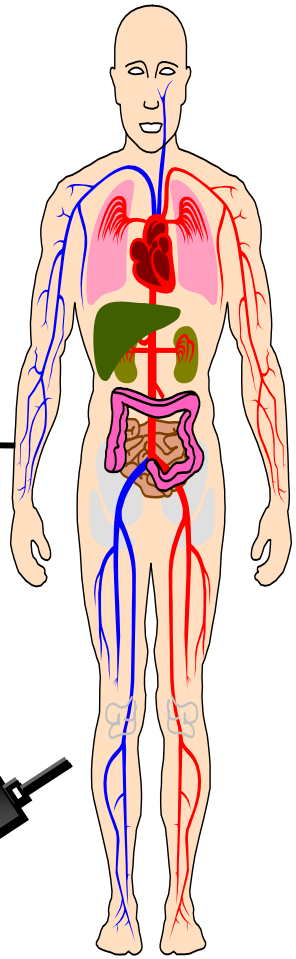
1) Isolation of the target cells

2) Gene transfer



3) Re-Infusion of the **genetically modified cells**

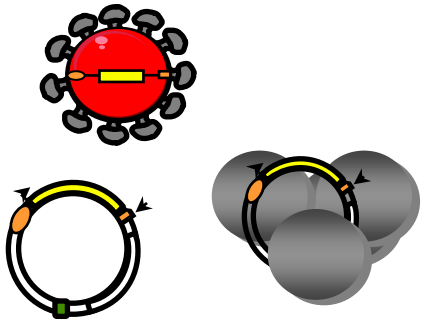


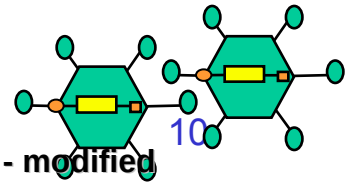


In-vivo gene therapy

Direct application:

- viral vector
- non-viral vector
- naked DNA
- replicating rec. micro-organism (adenovirus, salmonella)



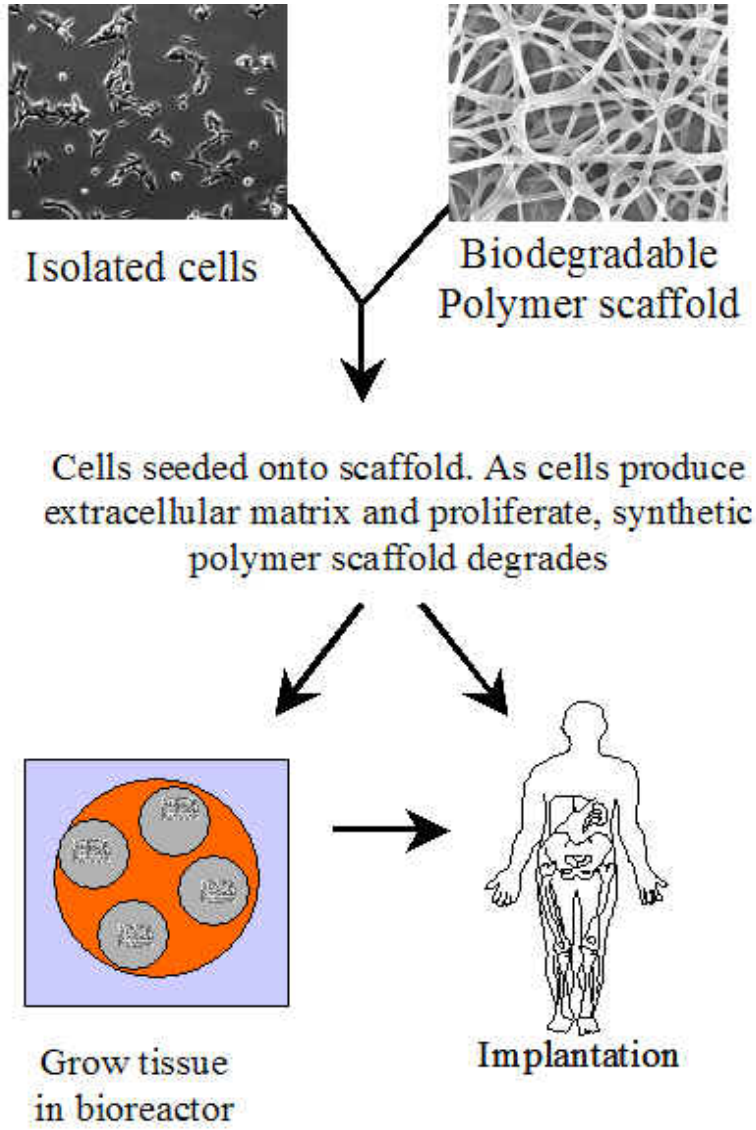


Source: Prof. K. Cichuteck - modified

XXX Product Profile

- "Engineered" oncolytic virus
  - designed to selectively replicate in and destroy cancer cells while leaving normal cells/tissues unharmed
- Viral gene deletions:
  - to restrict viral replication to dividing cells and reduce its toxicity / dissemination
- Armed with a gene able to activate cytotoxic pro-drugs
- Destruction of cancer cells by viral oncolysis combined with enhanced molecular chemotherapy

Tissue engineered products



Eligibility to the EMEA procedures as a Medicinal Product

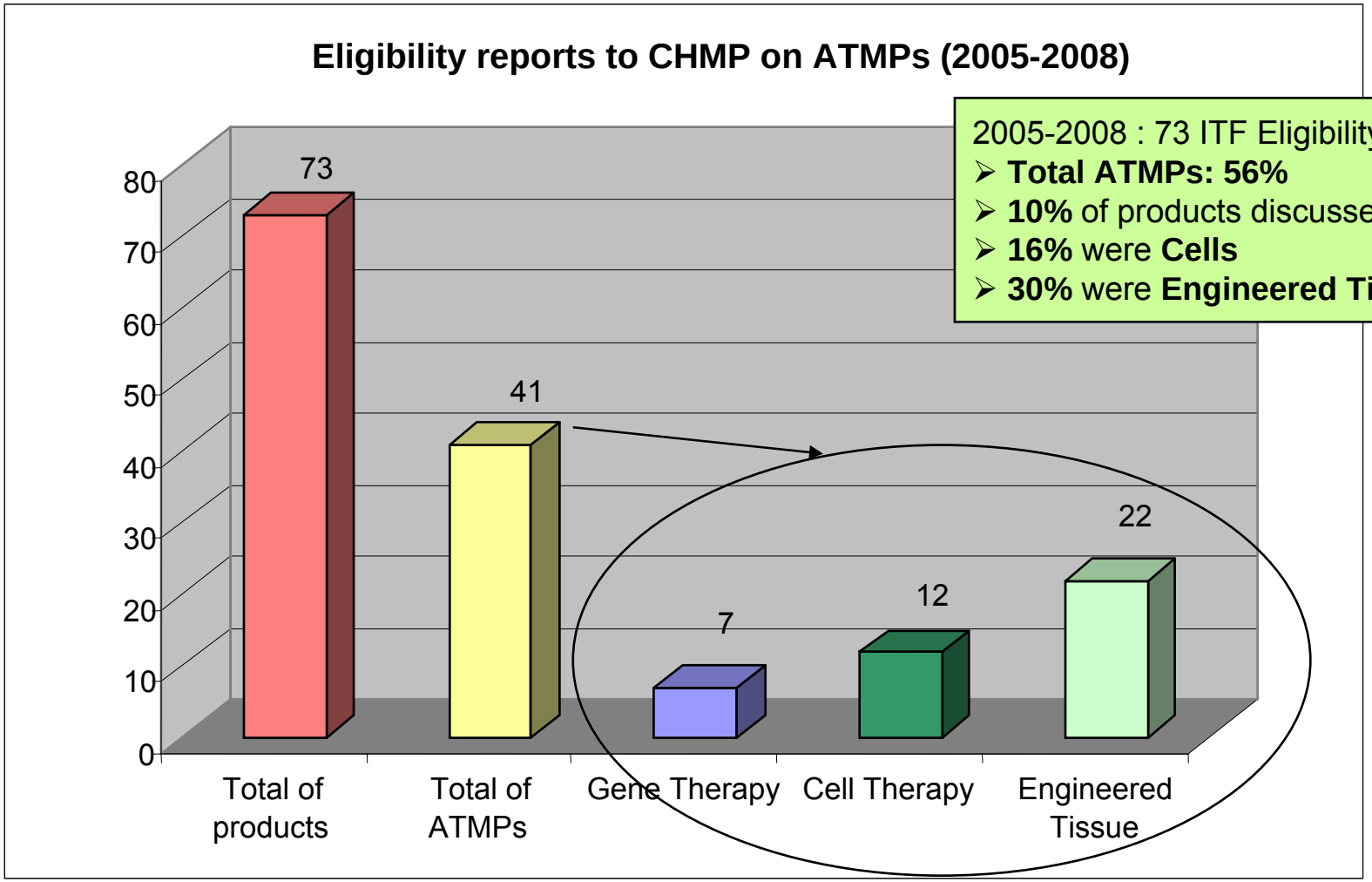
- **Frequent areas of uncertainty** :
  - Viral vector or viral vaccine?
  - Medicinal product or medical device?
  - Medicinal product or Transplant?
  - Manufacturing process or clinical cells processing?

Eligibility to the EMEA procedures as a Medicinal Product

- The ITF provides, in conjunction with the CHMP and the European Commission, advice to applicants on the **eligibility to EMEA procedures as a Medicinal Product** e.g. where there are uncertainties on whether the concerned therapeutic product(s) would qualify as Medicinal product(s).

- Understanding of the applicability of pharmaceutical framework for medicinal products, not officially about “category” of ATMP.

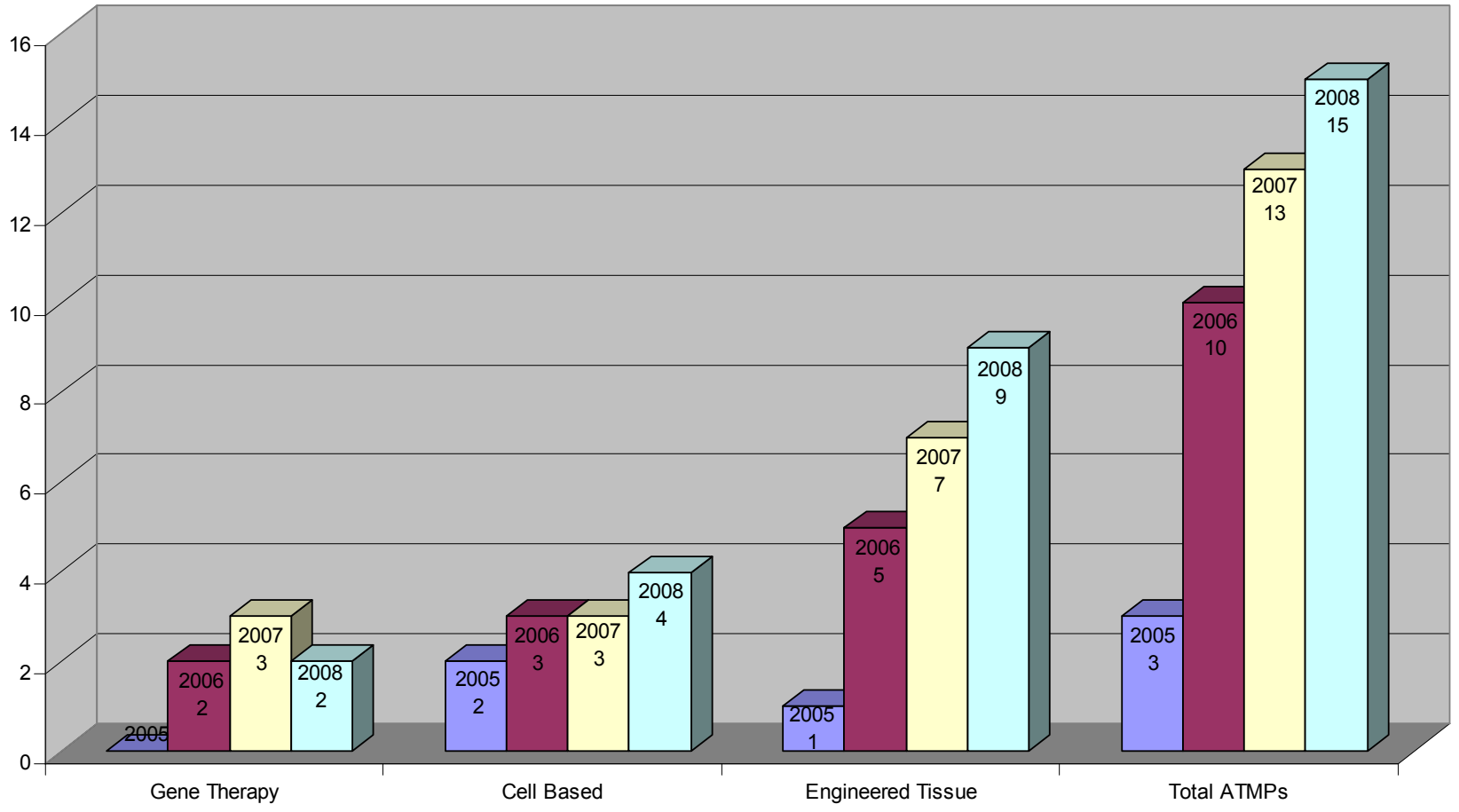
Eligibility as Medicinal Product Experience: focus on ATMPs



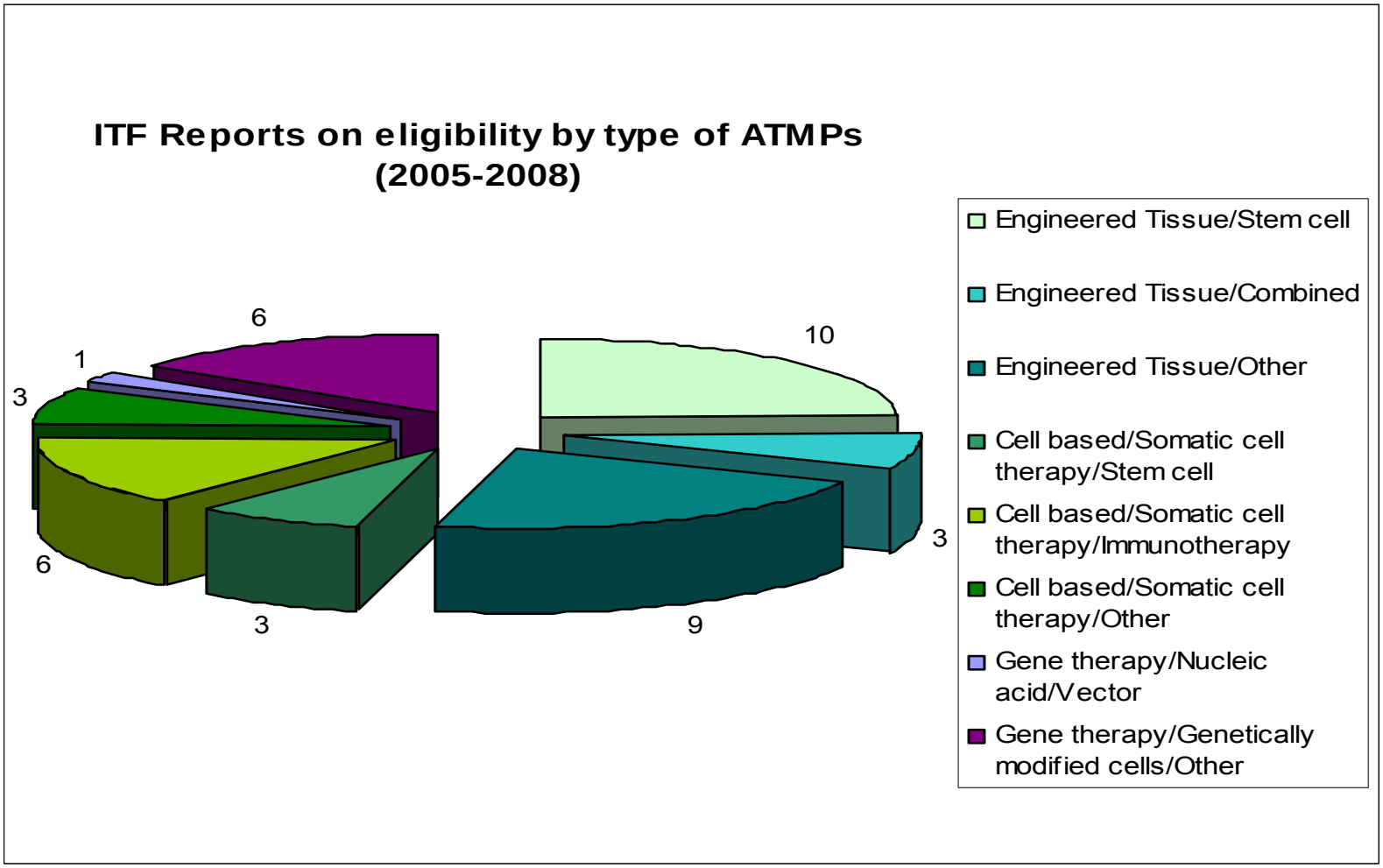
2005-2008 : 73 ITF Eligibility reports

- **Total ATMPs: 56%**
- **10%** of products discussed were **Genes**
- **16%** were **Cells**
- **30%** were **Engineered Tissues**

Eligibility as Medicinal Product
ATMPs “emerging” over recent years



Eligibility as Medicinal Product Experience: focus by types of ATMPs

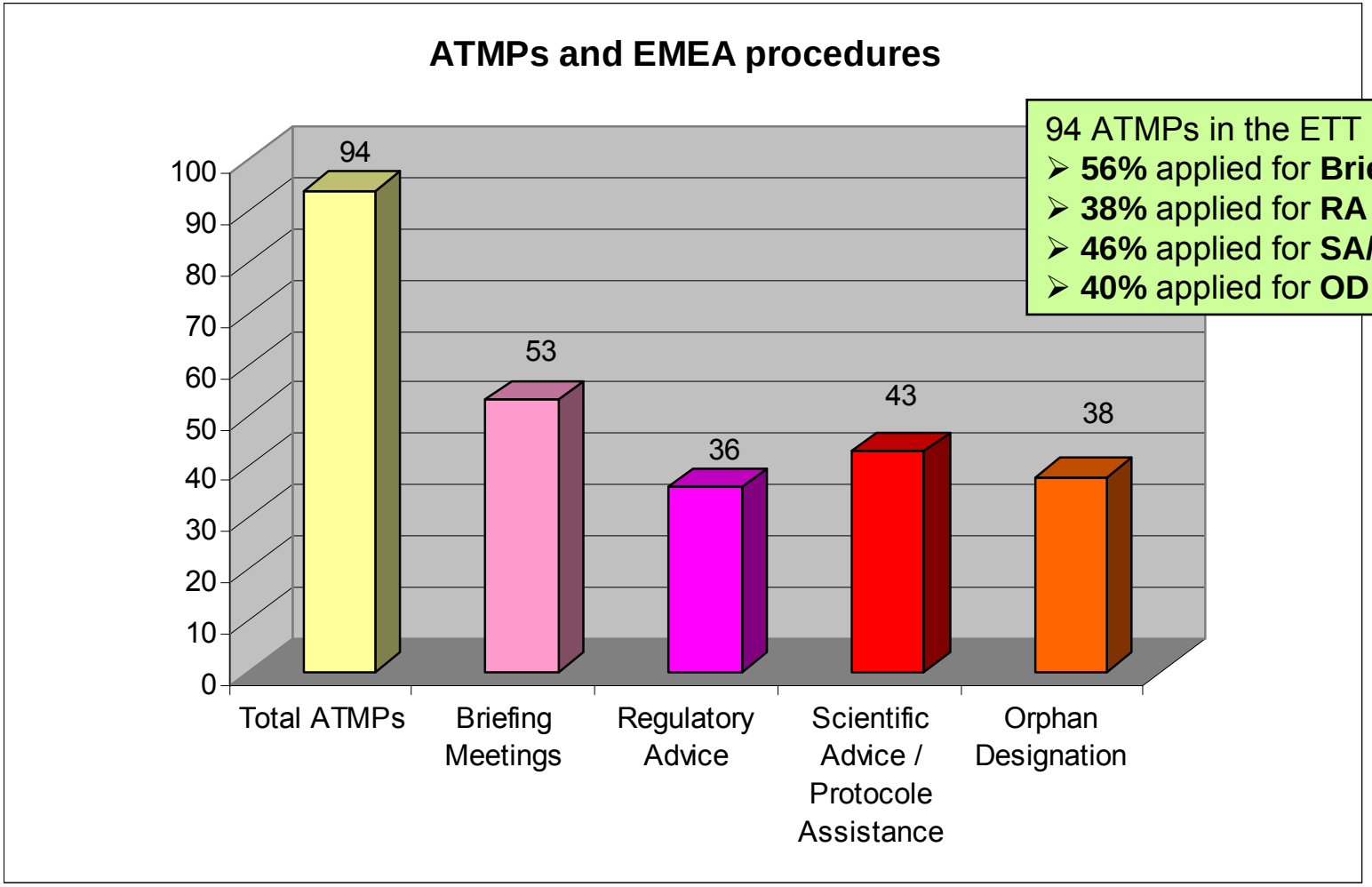


CAT Scientific Recommendation on Classification of ATMPs (ATMP Regulation) as of 2009

- **Scope**: to determine whether a medicinal product based on genes, cells or tissues meets the scientific criteria which define an advanced therapy medicinal products (including subcategories).

- **Similarities with eligibility advice**
  - Voluntary
  - Science based but with significant regulatory implications
  - Scientific Committee (CHMP for eligibility – CAT for classification) systematically involved
  - ITF as technical support
- **Differences**
  - Classification defined in the legislation as other new procedures (i.e. certification for SMEs and re-registration) specific for ATMPs and financial incentives
  - EC systematically consulted
  - Public disclosure (description of product, therapeutic area, classification)

Emerging Therapies and Technologies (2001-2008) : Experience at EMEA: focus on ATMPs(2/2)



94 ATMPs in the ETT
- **56%** applied for **Briefing Meeting**
- **38%** applied for **RA** (eligibility)
- **46%** applied for **SA/PA**
- **40%** applied for **OD**

Conclusions and way forwards

1. More than 90 ATMPs already seen at EMEA at all stages of development including MAA (6 CPs): Experience is there.

2. CAT as the highly specialized scientific resource in the EU system + tailored requirements and new procedures

3. Increased opportunities and competences for expert dialogues:
   - ITF Briefing Meetings
     - Preparation to eligibility or classification
     - Preparation to future regulatory procedures
     - Expertise from CAT and/or WPs

4. Benefits for both the EMEA and Stakeholders to learn early on challenges of ATMPs faced by sponsors.

Acknowledgements

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Thanks for your attention!