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Hospital Exemptions: IMB policy and experience to date

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Overview of Presentation:

- Description of Hospital exemption scheme
- IMB Requirements
- Experience to date

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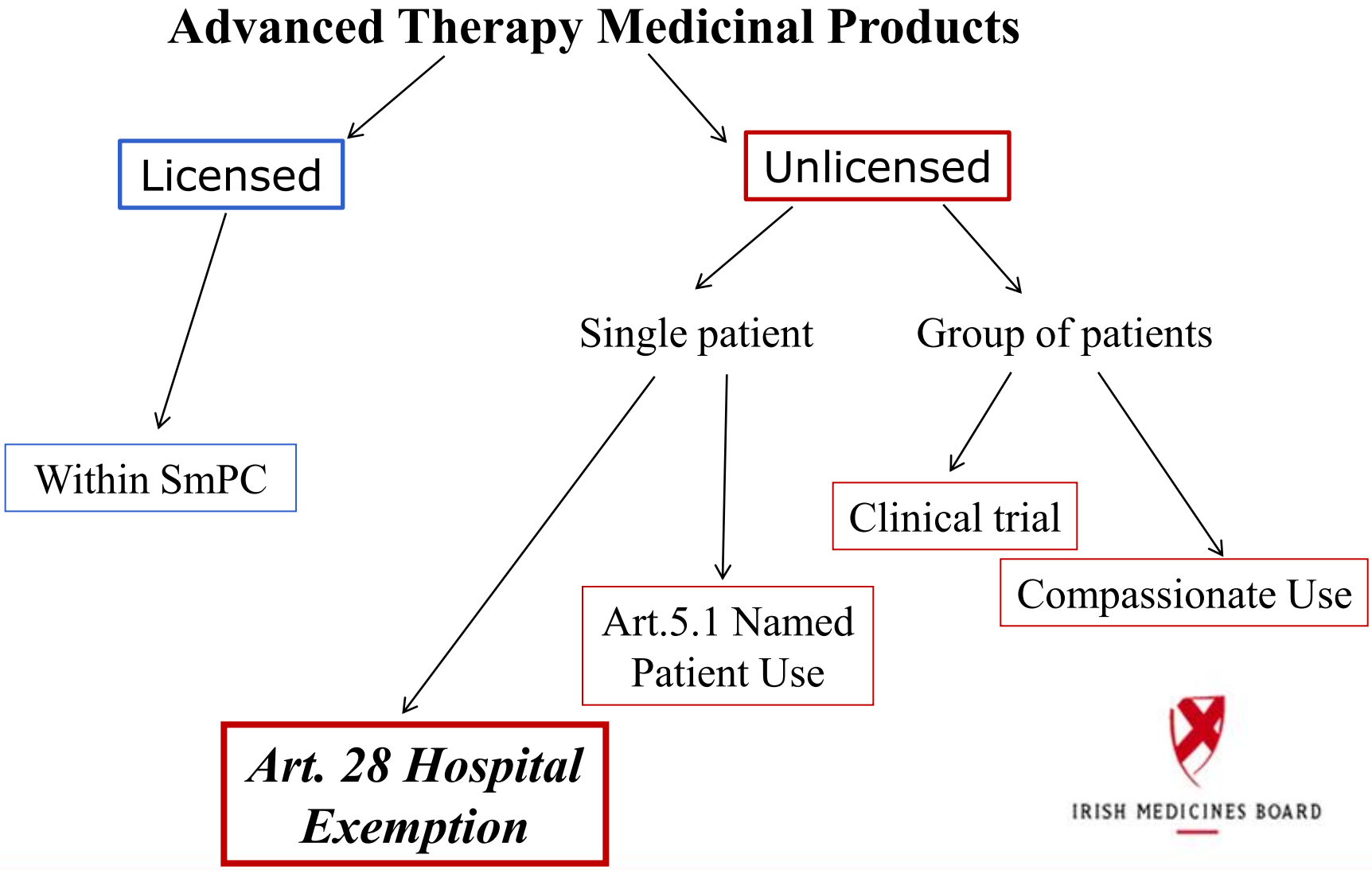
Regulatory Framework



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Hospital Exemption – Article 28

- Any ATMP which is prepared
  - Non-routine basis
  - According to specific quality standards
  - Used within the same Member State
  - In a hospital
  - Under the exclusive professional responsibility of a medicinal practitioner
- In order to comply with an individual medicinal prescription for a custom – made product for an individual patient

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Purpose of the Hospital Exemption

- Therapy of single patients
- Degree of flexibility for SMEs & academic sector
- To foster early stage product development
- Authorisation for products not suited for a centralised Marketing Authorisation Application (MAA)

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Hospital Exemption Requirements

- Manufacture of ATMPs under the hospital exemption must be authorised by the Member States (IMB)

- Requirements
  - Manufacturing
  - Traceability
  - Safety monitoring
  - Quality standards

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IMB Guidance Document

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GUIDE TO HOSPITAL-BASED ADVANCED THERAPY MEDICINAL PRODUCTS

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Manufacturing Requirements

- Donation and procurement of tissue and cells and donor testing
  - European Communities (Quality and Safety of Human Tissues and Cells) 2006 (S.I. No. 158 of 2006)
  - European Communities (Quality and Safety of Human Blood and Blood Components) Regulations, 2005 (S.I. No. 360 of 2005)
- Good Manufacturing Practice for the processing of the tissues and cells after procurement
  - Medicinal Products (Control of Manufacture) Regulations 2007, as amended (S.I. 539 of 2007, as amended)

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Quality Standards

Example of Quality Documents required

RISK ASSESSMENT OF KNOWN RISK FACTORS

manufacturer's analysis certificate

Suitability of starting materials of human/animal origin

Compatibility of non-cell-based components of combination products

DESCRIPTION OF THE PRODUCTION PROCESS

Donor testing confirmation

Description of the key process controls during production

Characterisation tests – product quality & batch consistency

validation of aseptic processes

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Quality Standards

- Specification for active substance & end product
  - Identity
  - Dose determination
  - Cell viability
  - Freedom from adventitious agents (viruses, mycoplasma, bacteria, fungi)
  - Potential impurities
  - Cell based medicinal products: Evaluation of tumorigenicity of cells grown for an extended period of time
  - Gene therapy products: viruses capable of replication and the percentage of infective viruses relative to the entire virus population

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Traceability

- System in place to ensure traceability from starting materials through manufacturing, packaging, storage, transport & delivery.
    - From donor to tissue establishment *(DG Sanco Directive 2004/23/EC)*
    - From receipt of cells/tissue in pharmaceutical facility to the delivery of cell-based product at the hospital *(GMP)*
    - From receipt in hospital to adminstration to patients & patient follow-up, if required
- Data to be kept for 30 years.
- In the event of bankruptcy, data to be transferred to the IMB.

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Safety Monitoring Requirements

- Pharmacovigilance
  - Standards equivalent to ATMPs with centralised Marketing Authorisations
  - appropriate recording and reporting of adverse reactions and events

- Risk Management Plan - case by case basis
  - need considered at the time of manufacturer's authorisation is sought
  - IMB request if safety concern raised

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Annual Report of Activity

- The number of ATMPs manufactured
- The number of patients treated with each ATMP
- The name of the physician prescribing the ATMP and who is responsible for care of that patient
- Any serious adverse incidents in the preparation of the ATMP
- Any adverse reactions caused by the ATMP

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Public consultation process

Public consultation process on hospital-based advanced therapy medicinal products

04 July 2012

Categories: Human Medicines-Guidance

The IMB is undertaking a public consultation on hospital-based advanced therapy medicinal products. Details of the consultation are given in the consultation information note and in the draft Guide to hospital-based advanced therapy medicinal products.

Please submit any comments that you may have by 17 September 2012 to consultation@imb.ie.

We look forward to your contributions to the process.

Consultation information note

Guide to hospital-based adanced therapy medicinal products

www.imb.ie

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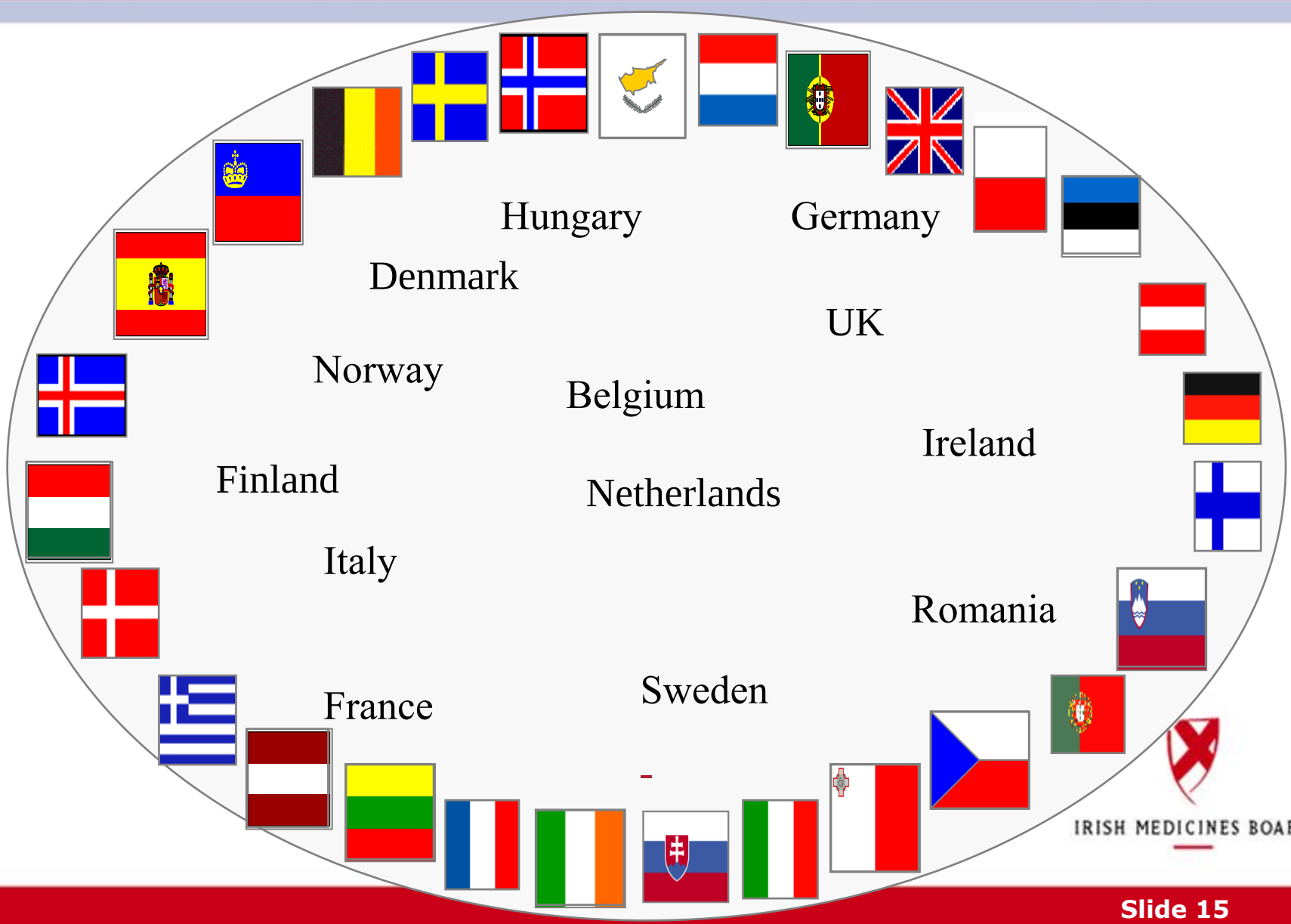
Experience to Date



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Thank you!

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