Medicines & Healthcare products Regulatory Agency

NIBSC
Confidence in Biological Medicines

Advanced Therapies – regulatory challenges

Christian K Schneider, MD
Director
National Institute for Biological Standards and Control (NIBSC)
Medicines & Healthcare products Regulatory Agency (MHRA)

Medicines & Healthcare products Regulatory Agency

CPRD

Clinical Practice Research Datalink

- NHS observational data and interventional research service
- Observational research studies: links between things like diet, or family history, and particular illnesses
- Clinical trials: UK/pan-EU

NIBSC

National Institute for Biological Standards and Control

- Standardization and control of biological medicines
- Over 90% of international biological standards
- UK's Official Medicines Control Laboratory for biological medicines
- Research
- Close relationship with WHO
- WHO collaborating center for polio, influenza and HIV

MHRA

Regulatory Centre

- Regulation of medicines: quality, safety, efficacy
- Medical devices: overseeing the UK Notified Bodies
- Operating post-marketing surveillance
- Regulating clinical trials

Advanced Therapies have gained momentum

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

1 April 2016
EMA/CHMP/230486/2016
Press office

Press release

New gene therapy for the treatment of children with ultra-rare immune disorder recommended for approval

Orphan-designated Strimvelis to offer treatment option for patients with ADA-SCID who have no suitable stem cell donor

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

24 June 2016
EMA/CHMP/429337/2016
Media and Public Relations

Press release

New cell-based therapy to support stem cell transplantation in patients with high-risk blood cancer

Orphan medicine Zalmoxis recommended by CAT and CHMP for marketing authorisation

Initial Evaluation of Marketing Authorisation Applications (MAA) for ATMP										
	2009	**2010**	**2011**	**2012**	**2013**	**2014**	**2015**	**2016**	**2017**	**Total**
Submitted MAAs	3	1	2	3	2	2	1	1	3	18
Positive draft Opinion	1	0	1[ii]	1[ii]	2	1	1	2	1	10*
Negative draft opinions	1[i]	0	1[ii]	0	0	0	2[iii]	0	0	4
Withdrawals	1	1[i]	0	0	2	0	0	0	0	4
Ongoing MAAs										4

*** Corresponding to 9 ATMPs**
[i] Same product (Cerepro)
[ii] Same product (Glybera)
[iii] CAT adopted two negative draft opinions for the same product (Heparesc)

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

19 May 2017
EMA/CHMP/315817/2017
Media and Public Relations

Press release

New advanced therapy to repair cartilage defects in the knee

Spherox recommended for marketing authorisation

http://www.ema.europa.eu/docs/en_GB/document_library/Committee_meeting_report/2017/10/WC500237198.pdf

…and setbacks

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

28 October 2017
EMA/713863/2017
EMEA/H/C/002145

Public statement

Glybera

Expiry of the marketing authorisation in the European Union

The marketing authorisation for Glybera (alipogene tiparvovec) expired on 28 October 2017 following the decision of the marketing authorisation holder, uniQure biopharma B.V., not to apply for a renewal of the marketing authorisation.

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

13 September 2016

Public statement

ChondroCelect

Withdrawal of the marketing authorisation in the European Union

On 29 July 2016, the European Commission withdrew the marketing authorisation for ChondroCelect (characterised viable autologous cartilage cells expanded ex vivo expressing specific marker proteins) in the European Union (EU), which will become effective as of 30 November 2016. The withdrawal was at the request of the marketing authorisation holder, TiGenix NV, which notified the European Commission of its decision to permanently discontinue the marketing of the product for commercial reasons.

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

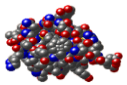
21 February 2013
EMA/52320/2013
EMEA/H/C/002657

Questions and answers

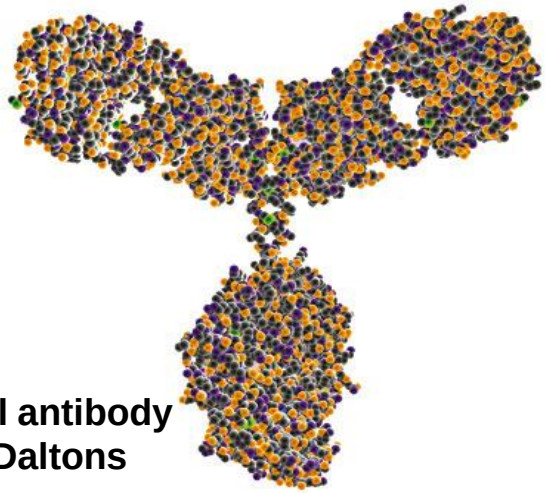
Withdrawal of the marketing authorisation application for Hyalograft C autograft (characterised viable autologous chondrocytes expanded in vitro, seeded and cultured on a hyaluronan-based scaffold)

Biologicals are complex

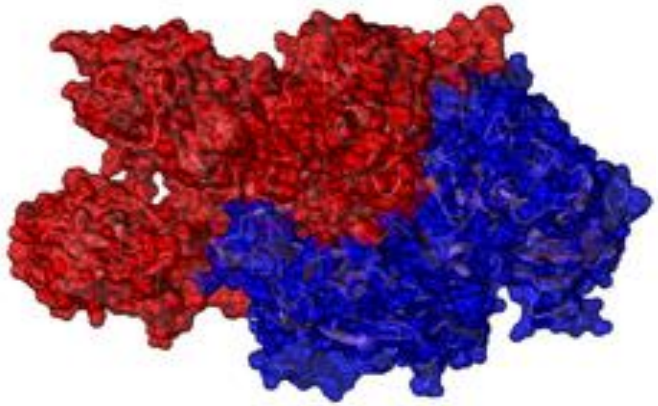
Aspirin
180 Daltons



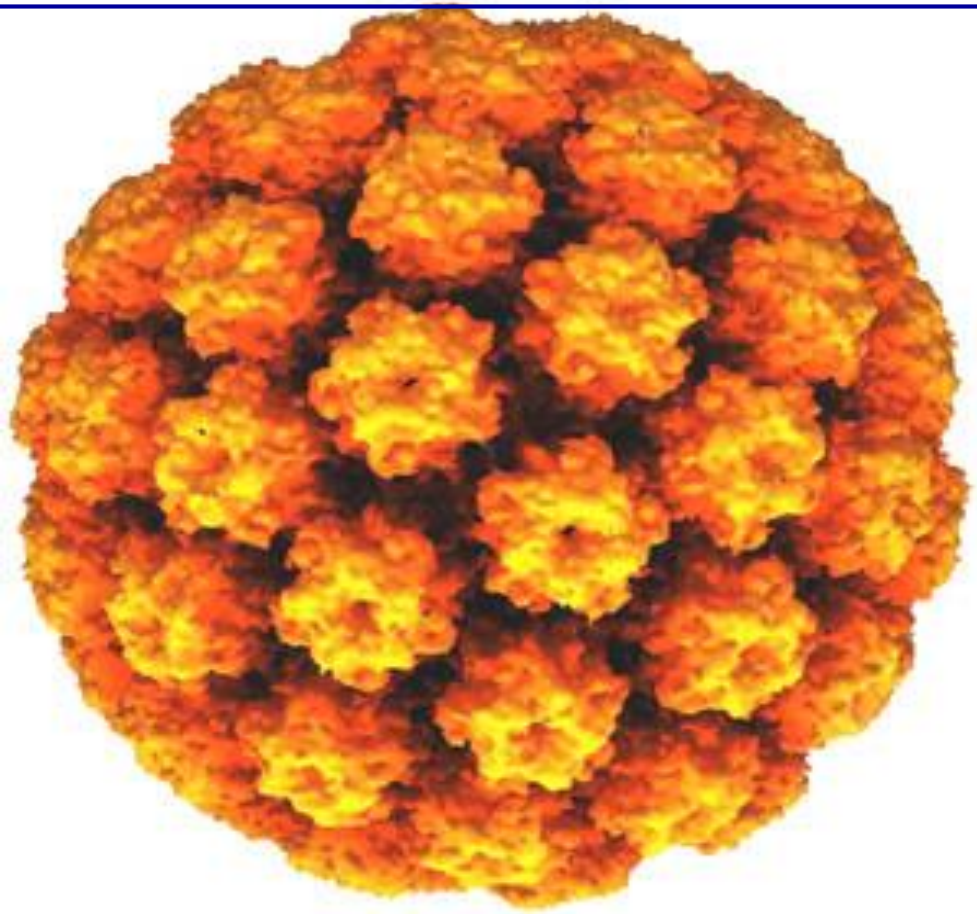
Insulin
5 700 Daltons



Monoclonal antibody
150 000 Daltons



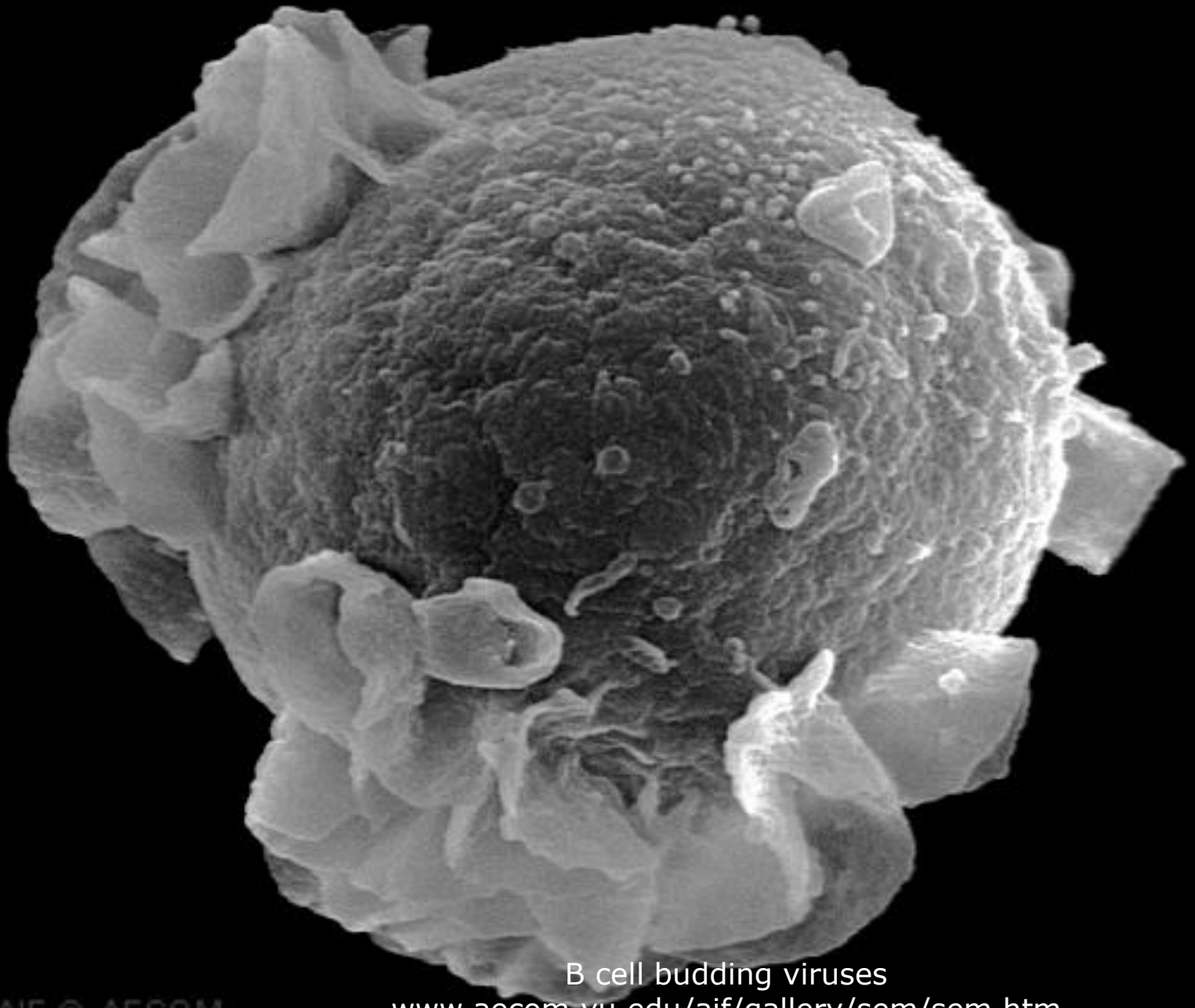
Coagulation Factor VIII
280 000 Daltons



Virus-like particle

Complexity of Advanced Therapies

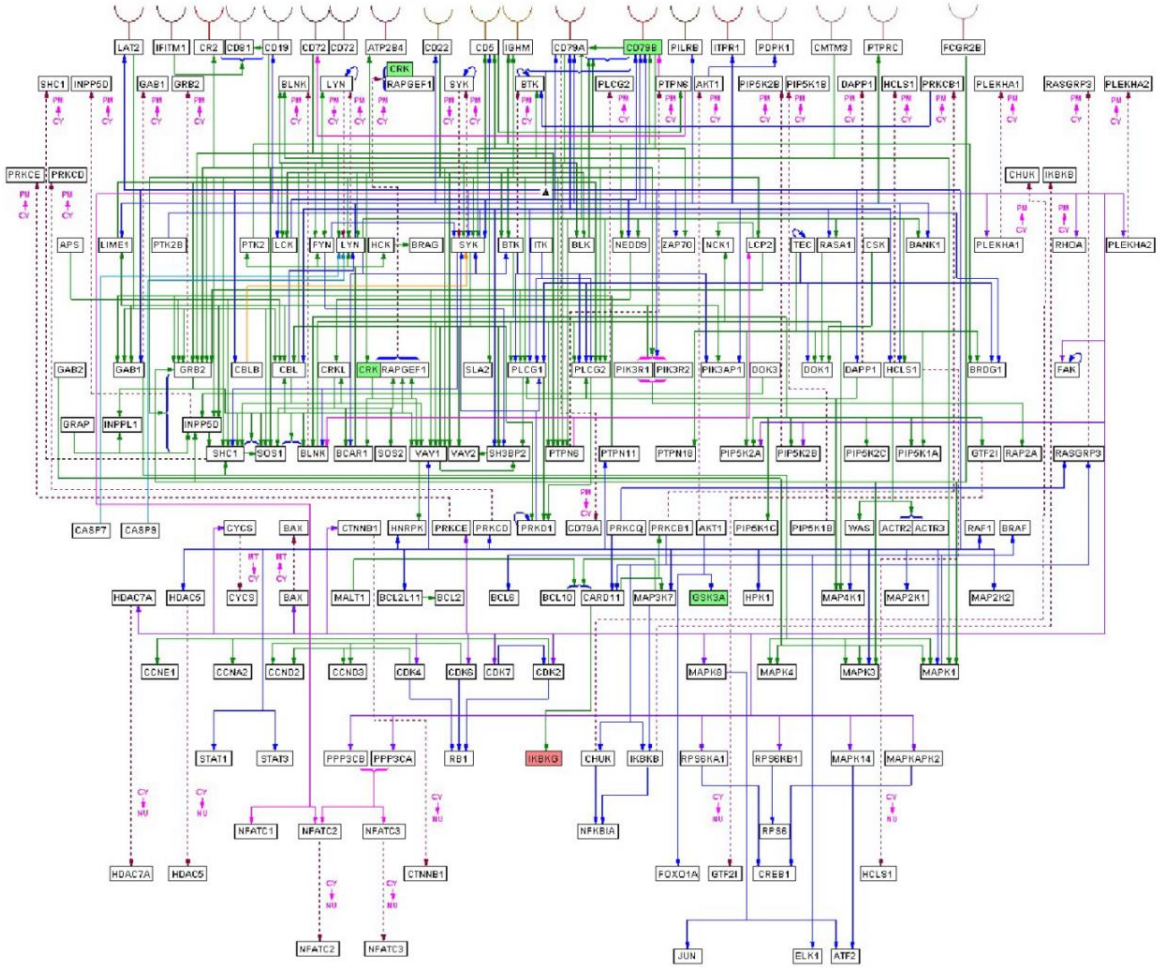
monoclonal antibody



B cell budding viruses
www.aecom.yu.edu/aif/gallery/sem/sem.htm

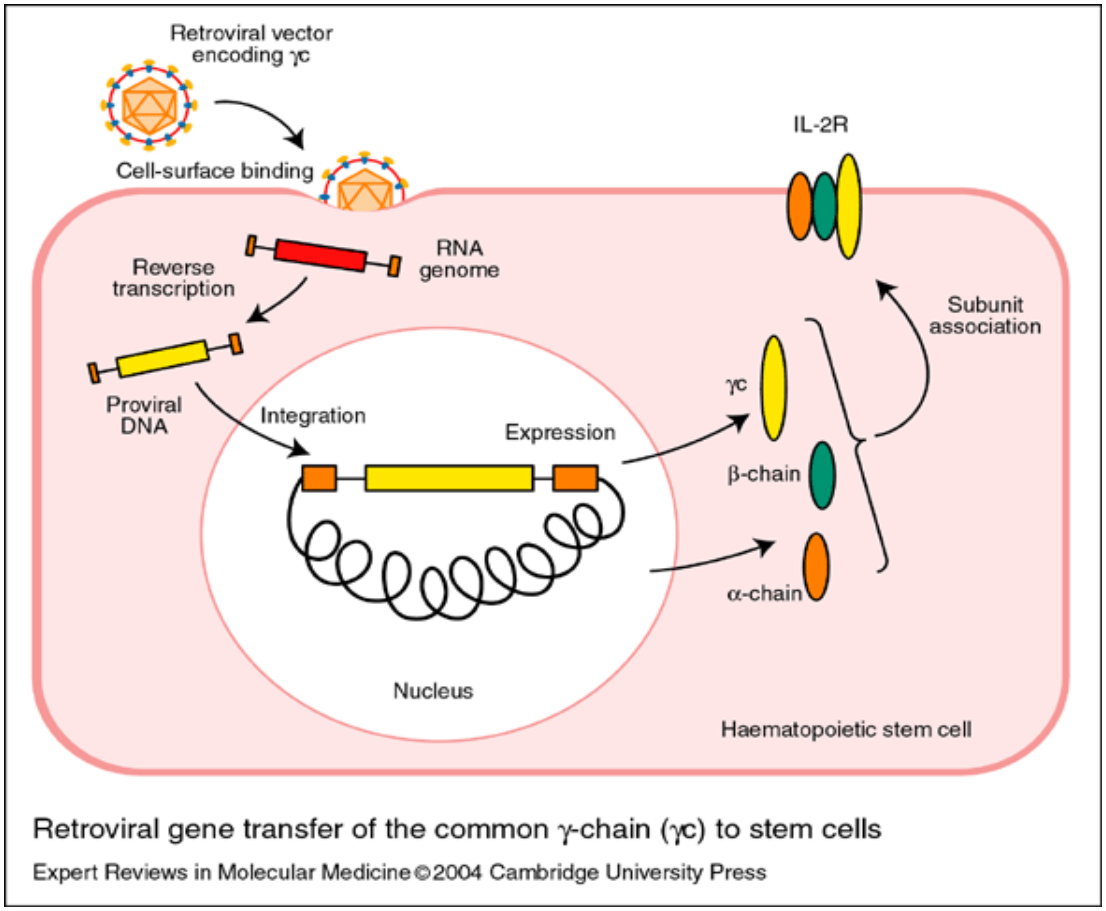
Complexity of signalling

Overlap and location of positive and negative modulators of NFk-B signalling identified in a cell-based screen within the T-cell receptor signaling pathway



Halsey et al, Genome Biology 2007

Gene transfer medicinal products



Retroviral gene transfer of the common γ-chain (γc) to stem cells

Vector-related issues clearly to be distinguished from effects mediated by expression of the gene = added complexity

Clinical challenges with gene transfer

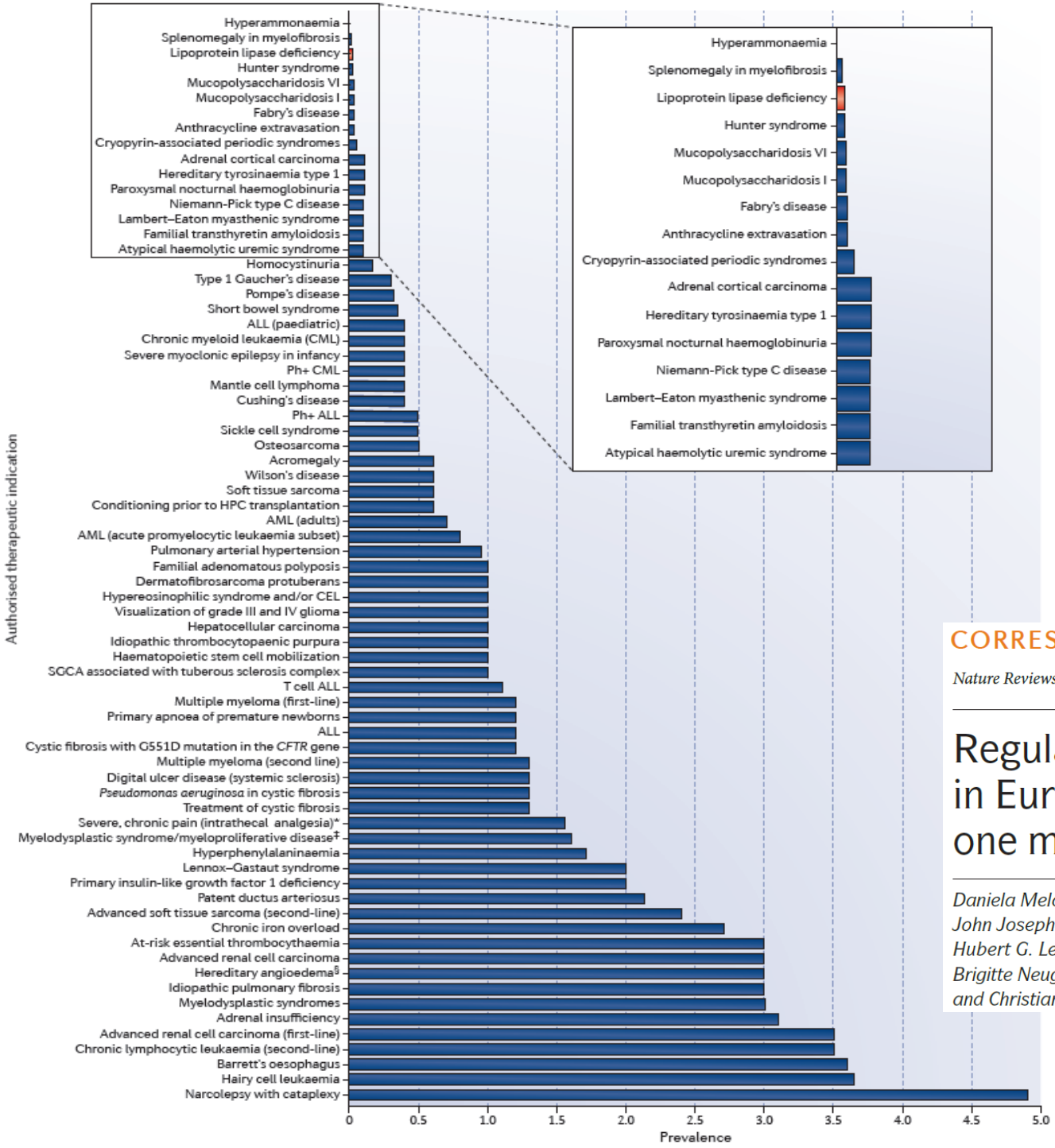
- „How to target only the target“

  Example:

  Gene therapy medicinal products which substitute for an organ or tissue-specific gene defect, but with multilocular occurrence (skin, muscle, bone,…)

  - **How to administer locally to ensure desired local distribution?**
  - **Impact on patient when administered multilocally**
    (more than 20 injections per patient etc.)

- **How to control the clinical trial?**
- **How to blind the trial?**
- **How to measure clinical outcome?**



Need for a wider perspective

„Ultra-orphan drugs“:
Could be defined as prevalence of the disease of equal to or less than 0.1 in 100,000.

CORRESPONDENCE

Nature Reviews Drug Discovery | AOP, published online 19 August 2013; doi:10.1038/nrd3835-c1

Regulatory evaluation of Glybera in Europe — two committees, one mission

Daniela Melchiorri, Luca Pani, Paolo Gasparini, Giulio Cossu, Janis Ancans, John Joseph Borg, Catherine Drai, Piotr Fiedor, Egbert Flory, Ian Hudson, Hubert G. Leufkens, Jan Müller-Berghaus, Gopalan Narayanan, Brigitte Neugebauer, Juris Pokrotnieks, Jean-Louis Robert, Tomas Salmonson, and Christian K. Schneider

Pivotal data for ultra-orphan drugs

Authorised therapeutic indication	Prevalence (per 10,000)	Medicinal product	Date of Marketing Authorisation	Endpoints used	pivotal study duration	pivotal study(ies) size (patients)	Disease characteristics
Hyperammonaemia	0,001	**Carbaglu®**	24-01-2003	Biochemical and clinical course, incl. growth and survival	N/A	20	progressive disease (metabolical)
Splenomegaly in myelofibrosis	0,01	**Jakavi®**	23-08-2012	Number of patients with ≥ 35% spleen volume reduction at Week 24	24 weeks	219	progressive disease (haematological)
LPLD (lipoprotein lipase deficiency)	0,02	**Glybera®**	25-10-2012	reduction in fasting plasma triglyceride levels; additonal enpoints included chylomicron-related endpoints and reduction in frequency and/or severity of clinical signs and symptoms related to LPL deficiency including pancreatitis	N/A (variable)	14 and 5	fluctuating clinical course (metabolical)
Hunter syndrome	0,02	**Elaprase®**	08-01-2007	6-minute walk test, % predicted FVC (baseline to week 53)	12 months	96	progressive disease (organ impairment)
Mucopolysaccharidosis VI	0,024	**Naglazyme®**	24-01-2006	12-minute walk test over time (week 6, 12, 18, 24)	24 weeks	39	progressive disease (organ impairment)
Mucopolysaccharidosis I	0,025	**Aldurazyme®**	10-06-2003	6-minute walk test, % predicted FVC (baseline to week 26)	26 weeks	45	progressive disease (organ impairment)
Fabry disease	0,027	**Fabrazyme®**	03/08/2001 (expired 07/08/2011)	Reduction of GL-3 accumulation from the capillary endothelium of the kidney to score 0 at week 20	20 weeks	58	progressive disease (organ impairment)
Fabry disease	0,027	**Replagal®**	03-08-2001	brief pain inventory BPI (study TKT003) and cardiac Gb3 levels as determined from cardiac biopsy samples (study TKT005)	24 weeks	26 and 15	progressive disease (organ impairment)
Anthracycline extravasation	0,03	**Savene®**	28-07-2006	proportion of patients undergoing surgical intervention	<28 days	23 and 57	acute condition
Cryopyrin-Associated Periodic Syndromes	0,05	**Ilaris®**	23-10-2009	proportion of patients with disease flare in part II (randomized withdrawal)	48 weeks (three parts)	35	active inflammatory clinical course
Cryopyrin-Associated Periodic Syndromes	0,05	**Rilonacept Regeneron®**	23-10-2009	mean change from baseline to endpoint in the mean key symptom score (both parts of study)	48 weeks	47	active inflammatory clinical course
Adrenal cortical carcinoma	0,1	**Lysodren®**	28-04-2004	Bibliographical evidence (220 articles) with various endpoints including survival, remission time, tumour size reduction	N/A (variable)	N/A (ca. 500 patients overall)	progressive disease (cancer)
Hereditary tyrosinemia type 1	0,1	**Orfadin®**	21-02-2005	Data from a compassionate use programme including survival, survival without transplantation, death due to liver failure, transplantation due to liver failure and hepatocellular carcinoma.	N/A (variable)	ca. 207	progressive disease (metabolical)
Paroxysmal nocturnal haemoglobinuria	0,1	**Soliris®**	20-06-2007	haemoglobin stabilization and units of PRBCs transfused during the treatment phase	26 weeks	88	active disease requiring regular intervention
Niemann-Pick type C disease	0,1	**Zavesca®**	26-01-2009	mean change from baseline to Month 12 for Horizontal saccadic eye movements	12 months	29	progressive disease (neurological)
Lambert-Eaton myasthenic syndrome	0,1	**Firdapse®**	23-12-2009	Bibliographical evidence with two main studies; endpoint measurements included various neurological scores including measurements of neurological disability score, muscle strength, electrophysiological measurements, or the quantitative myasthenia gravis score	15 days and 6 days	12 and 26	progressive disease (neurological)
Familial transthyretin amyloidosis	0,1	**Vyndaqel®**	16-11-2011	Co-primary endpoint: (1) improvement or stabilization in the Neurologic Impairment Score–Lower Limb score; (2) Change from Baseline in the Total Quality of Life Norfolk QOL-DN score	18 months	128	progressive disease (neurological)
Atypical haemolytic uremic syndrome (aHUS)	0,1	**Soliris®**	24-11-2011	Platelet change from baseline and haematologic normalisation (study 1), and TMA Event-Free status and haematologic normalization from baseline (study 2)	26 weeks	17 and 20	progressive disease (organ impairment)

Melchiorri D et al: Regulatory evaluation of Glybera in Europe - two committees, one mission. Nat Rev Drug Discov. 2013 Sep;12(9):719.

Classification

Article 17:

"Any applicant developing a product based on genes, cells or tissues may request a scientific recommendation of the Agency with a view to determining whether the referred product falls, on scientific grounds, within the definition of an advanced therapy medicinal product.(…)"

Biological Gene Delivery Vehicles: Beyond Viral Vectors

Yiqi Seow[1] and Matthew J Wood[1]

[1]*Department of Physiology, Anatomy and Genetics, University of Oxford, Oxford, UK*

Molecular Therapy, 17, 767-777, 2009.

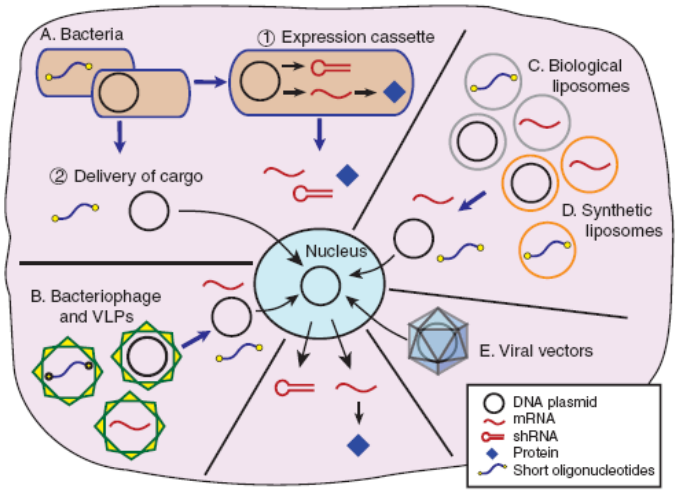
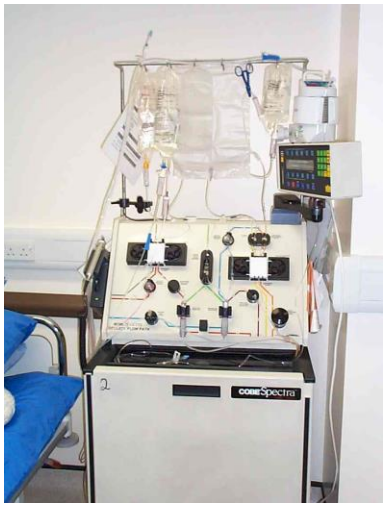


Figure 3 Release of cargo intracellularly by delivery vehicles. (A) Bacteria can deliver genetic cargoes in two distinct fashion after endocytosis and endosomal release. First, short oligonucleotides and DNA plasmids can be released directly into the host cells through the lysis of the bacteria. Alternatively, intracellular bacteria can produce and excrete therapeutic RNAs and proteins. (B–D) Bacteriophage, VLPs and both types of liposomes are capable of delivering mRNAs, short oligonucleotides and DNA plasmids. (E) Viral vectors are typically only capable of delivering DNA or RNA vectors that ultimately end up in the nucleus as DNA templates for transcription of mRNAs. shRNA, short hairpin RNA; VLP, virus-like particle.

How to classify borderline cases?

„Bedside-application“ of autologous (stem-cell containing) cell preparations



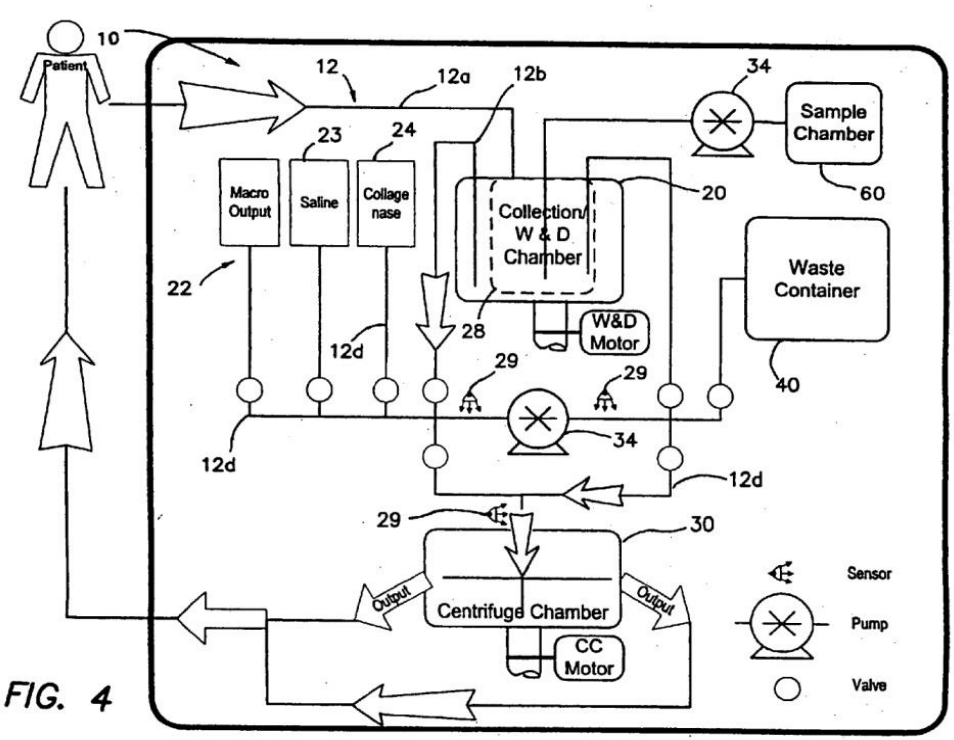
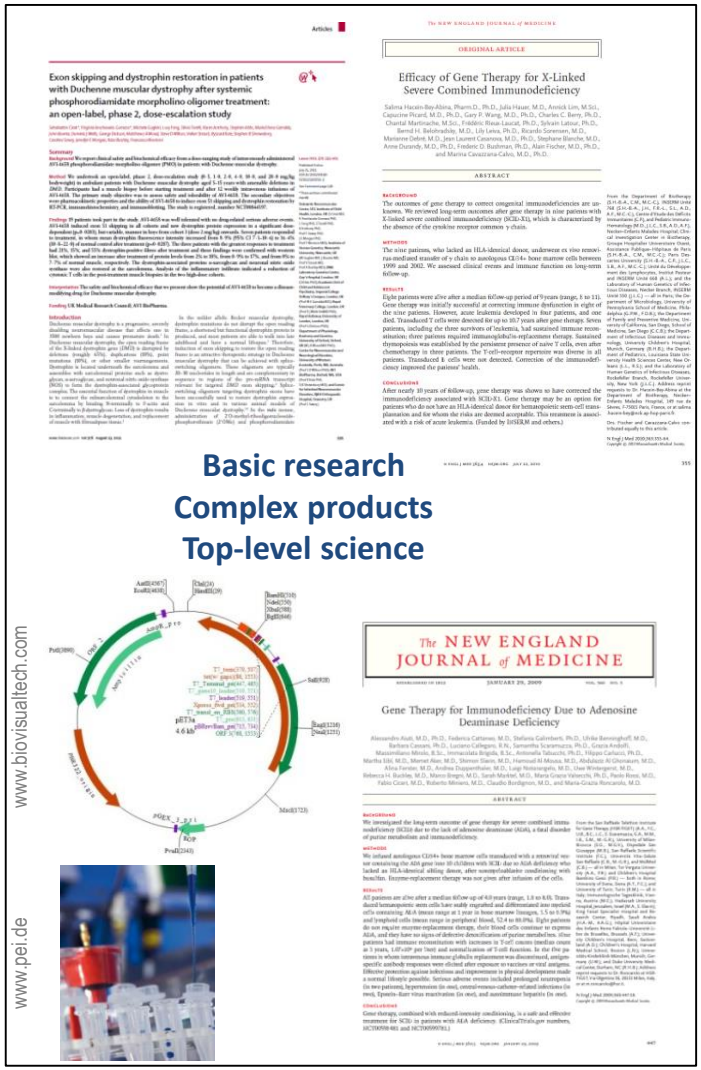


FIG. 4

What is the product?
What (who) determines classification?

The environment: The „academic gap“ and „small company gap“



Basic research
Complex products
Top-level science

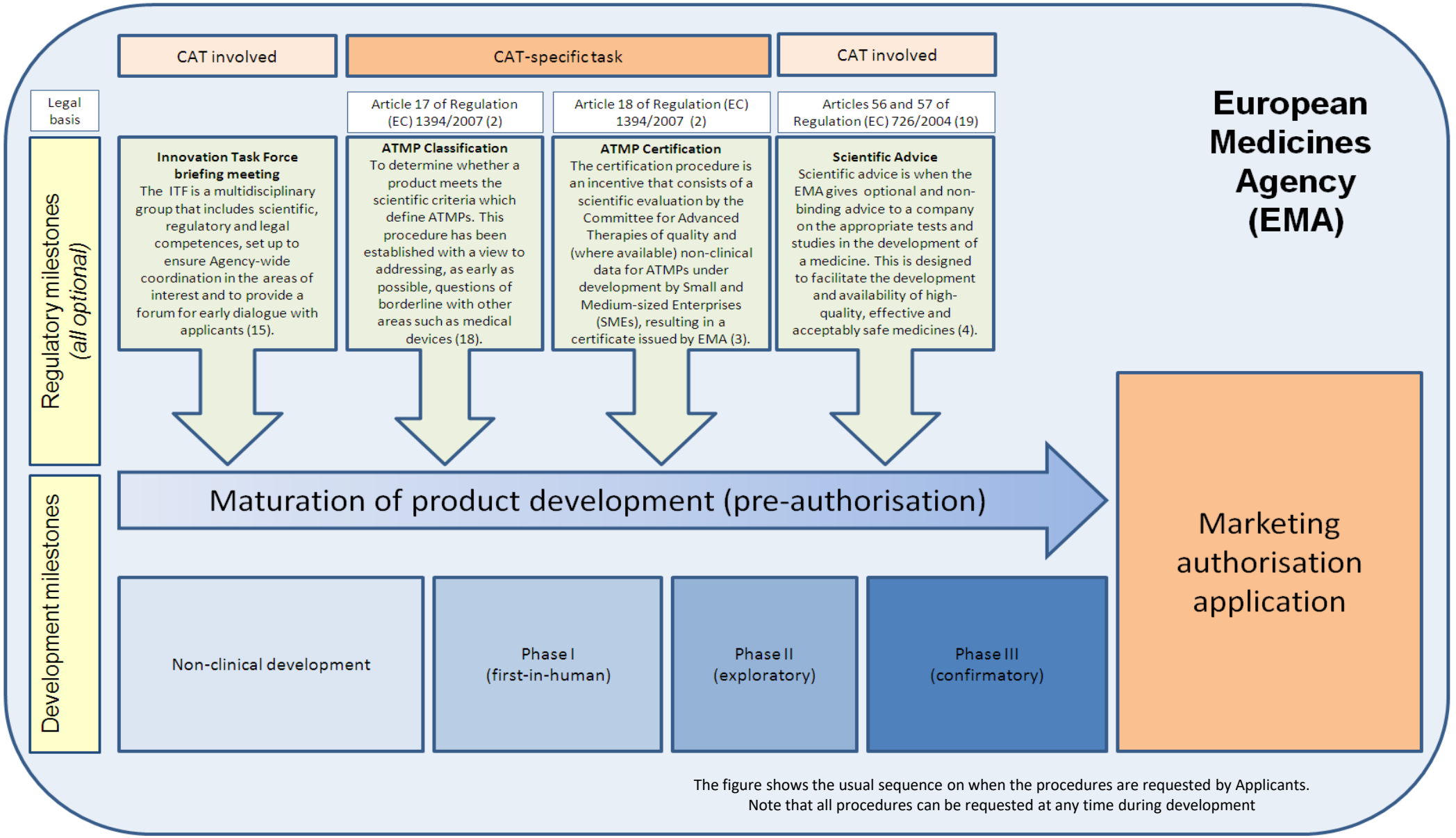
Regulation 1394/2007 („ATMP regulation“)

Directive 2009/120/EC amending Directive 2001/83/EC (”revised Annex I”)

Marketing authorisation

Translation into a medicinal product (”translational medicine”)

ATMP development and involvement of CAT



The figure shows the usual sequence on when the procedures are requested by Applicants.
Note that all procedures can be requested at any time during development

Certification: What is it?

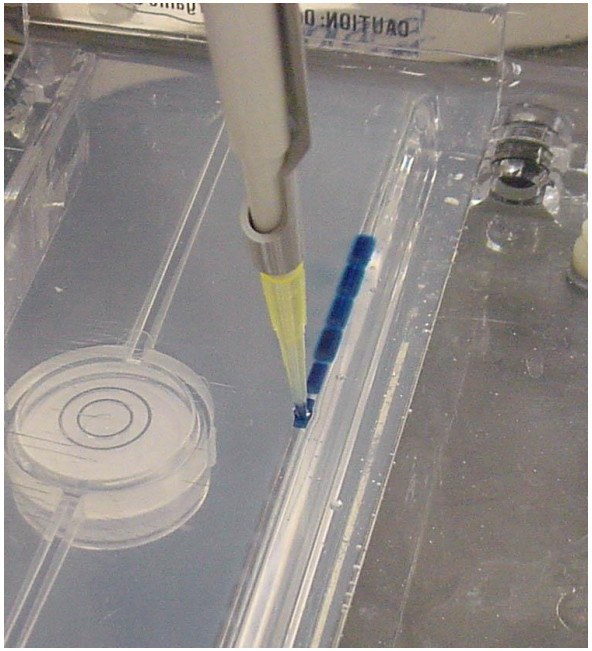
Art. 18: ” Small and medium-sized enterprises developing an advanced therapy medicinal product may submit to the Agency all relevant quality and, where available, non-clinical data required in accordance with modules 3 and 4 of Annex I to Directive 2001/83/EC, for scientific evaluation and certification.”

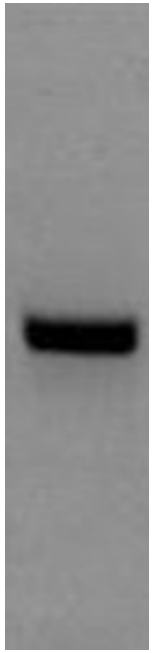
Early stage development vs. Annex I: Is the Annex I written from a Marketing Authorisation perspective?

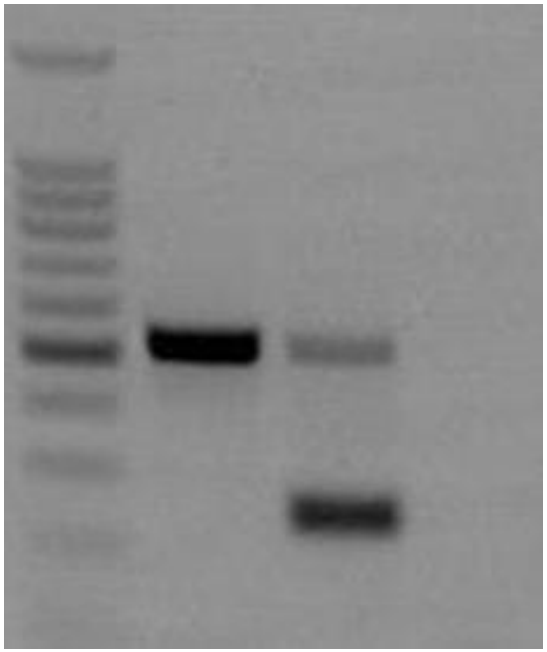
EMA procedureal guidance EMA/CAT/418458/2008/corr.: Not binding for future MA; not a Scientific Advice; not binding for National Agencies for Clinical Trial Authorisations.

Certification

- What is a certification?
  (a personal interpretation)







http://delliss.people.cofc.edu/virtuallabbook/LoadingGel/GelLoadB.jpg

Art. 28: the so-called “hospital exemption”

- Additional exclusion under very specific conditions e.g.:
  - Non-routine basis of production [what is this?]
  - Specific quality standards
  - Used in same MS in hospital (manufacturing authorized by Comp. Authority of MS)
  - Custom-made product for individual patient
  - Under the exclusive professional responsibility of a practitioner
  - National rules on the use of cells on ethical grounds
- An alternative Marketing Authorisation procedure?
- Creation of a second market?

Art. 28: the so-called “hospital exemption”

frontiers in PHARMACOLOGY

PERSPECTIVE ARTICLE
published: 14 February 2012
doi: 10.3389/fphar.2012.00012

Advanced therapy medicinal products and exemptions to the Regulation 1394/2007: how confident can we be? An exploratory analysis

Philippe Van Wilder *

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The market authorization procedure for medicinal products for human use is relying on their demonstrated efficacy, safety, and pharmaceutical quality. This applies to all med-

”Obviously differences in development track … resources, which may result in substantial pr… the applicant submitting a centrally authoriz… alternatives are on the market.”
(van Wilder)

INTRODUCTION

The market authorization (M… ucts for human use is relying on their demonstrated efficacy, safety, and pharmaceutical quality (The European Parliament and the Council of the European Union, 2001). This applies to all medicinal products whether of chemical (e.g., blood pressure lowering diuretic) or biological (e.g., anti-inflammatory monoclonal antibody) origin. Modern biotechnology medicinal products obtain market approval through the centralized procedure as detailed in the EC Regulation 726/2004 (The European Parliament and the Council of the European Union, 2004).

Since 2008, a “lex specialis” – Regulation (EC) No 1394/2007 (The European Parliament and the Council of the European Union, 2007) – applies to advanced therapy medicinal products (ATMPs); these ATMPs are pharmaceuticals with high complexity (The Committee for Advanced Therapies (CAT) and the CAT Scientific Secretariat, 2010) linked to their development, manufacturing, or administration process.

The Regulation highlights the following:

- It provides an explicit ATMP definition: ATMPs are gene therapy, somatic cell therapy, or tissue-engineered medicinal products.
- An ATMP must comply with the existing MA requirements (quality, safety, and efficacy) and the post-marketing pharmacovigilance rules. For MA, the centralized procedure is mandatory: it aims to pool Community expertise and ensure a high level of scientific evaluation and facilitate access to market.

responsibilities are:

- The mandatory evaluation of MA applications by providing opinions to the Committee for Medicinal Products for Human Use (CHMP); the CHMP may adopt or refuse the CAT opinion.
- The optional scientific certification (art. 18) of quality and non-clinical data of a proposed ATMP-compound in development.
- The optional scientific recommendation on ATMP-classification (art. 17), prior to their clinical development.

The CAT (The Committee for Advanced Therapies (CAT) and the CAT Scientific Secretariat, 2010) is a multidisciplinary scientific expert committee: it also focuses on the scientific developments in the field. There is no doubt about the huge scientific, regulatory, and ethical challenges triggered by these complex products and a specific expert committee for ATMPs is necessary to deal with these challenges (similar to the creation of the Committee on Orphan Medicinal Products for drugs used in rare diseases) and beneficial to all relevant public and private stakeholders.

- The Tissues and Cells Directive (2004/23/EC) applies to donation, procurement and testing of human tissues and cells.
- The Regulation defines the pre- and post-authorization requirements: GMP and GCP standards, product follow-up on efficacy and safety, risk management plan, and traceability.

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

13 September 2016

Public statement

ChondroCelect

Withdrawal of the marketing authorisation in the European Union

On 29 July 2016, the European Commission withdrew the marketing authorisation for ChondroCelect (characterised viable autologous cartilage cells expanded ex vivo expressing specific marker proteins) in the European Union (EU), which will become effective as of 30 November 2016. The withdrawal was at the request of the marketing authorisation holder, TiGenix NV, which notified the European Commission of its decision to permanently discontinue the marketing of the product for commercial reasons.

ChondroCelect was granted marketing authorisation in the EU on 5 October 2009 for repair of single symptomatic cartilaginous defects. The marketing authorisation was initially valid for a 5-year period. It was subsequently renewed for an additional 5-year period in 2014.

The European Public Assessment Report (EPAR) for ChondroCelect will be updated accordingly to reflect the fact that the marketing authorisation is no longer valid.

ATMPs are special:

Consequence:

Development and MA procedure may be difficult

Do we have to adapt our thinking to the products, not the products to our thinking?

Probably both: We have to adapt to the specificities of the products, but the developers will also have to adapt to the pharma framework.

How to regulate (minimally manipulated bone-marrow-derived) stem cells

Annex I to Directive 2001/83/EC: How to handle minimally manipulated ATMPs?

(e.g., bone marrow in non-homologous use)

- ”Starting materials”
- ”Validation”
- ”Identity”
- ”Purity”
- ”Potency”
- ”Mechanism of Action”

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

3 June 2016
EMA/345874/2016

Advanced therapy medicines: exploring solutions to foster development and expand patient access in Europe

Outcome of a multi-stakeholder meeting with experts and regulators held at EMA on Friday 27 May 2016

http://www.ema.europa.eu/docs/en_GB/document_library/Report/2016/06/WC500208080.pdf

European Commission

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

European Commission-DG Health and Food Safety and European Medicines Agency Action Plan on ATMPs

The term "advanced therapy medicinal products" ("ATMPs") is used to designate gene therapies, somatic cell therapies and tissue engineered products.

In the EU, these products are governed by Regulation 1394/2007 on advanced therapy medicinal products ("ATMP Regulation"). The cornerstone of the Regulation is that a marketing authorisation must be obtained prior to the marketing of ATMPs. The evaluation of these products is led by a specialised committee within the European Medicines Agency (EMA) i.e. by the Committee for Advanced Therapies ("CAT") who prepares a draft opinion before the Committee for Medicinal Products for Human Use (CHMP) adopts a final opinion and the authorisation is granted by the Commission. The ATMP Regulation also empowers Member States to permit the use of advanced therapies that have not been authorised by the Commission under certain conditions (so-called "hospital exemption").

The 2014 report on the application of ATMPs[1], concluded that the Regulation had protected patients from unsound treatments. However, it also recognised shortcomings and identified actions to help translate scientific progress into medicinal products available to patients. Such shortcomings were also discussed in a multi-stakeholder workshop organised by the EMA on 27 May 2016 and certain follow-up initiatives have already been taken, as also reflected in this action plan[2,3].

The European Commission services and the European Medicines Agency, in collaboration with the authorities of the Member States, have initiated a number of initiatives to improve the regulatory environment for ATMPs so as to facilitate the development and authorisation of these products in the EU for the benefit of patients. The actions presented in this document are wide-ranging and target challenges identified by various stakeholders at all stages of development, including manufacturing, early and later phases of development, marketing authorisation process and post-marketing setting.

[1] http://ec.europa.eu/health/human-use/advanced-therapies/developments/index_en.htm

[2] http://www.ema.europa.eu/docs/en_GB/document_library/Report/2016/06/WC500208080.pdf

[3] http://www.ema.europa.eu/docs/en_GB/document_library/Other/2017/02/WC500220952.pdf

1

http://www.ema.europa.eu/docs/en_GB/document_library/Other/2017/10/WC500237029.pdf

Standards for Advanced Therapies?

RESEARCH ARTICLES

Development of the First World Health Organization Lentiviral Vector Standard: Toward the Production Control and Standardization of Lentivirus-Based Gene Therapy Products

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Gene therapy is a rapidly evolving field. So far, there have been >2,400 gene therapy products in trials and four products on the market. A prerequisite for producing gene therapy products is e their quality and safety. This requires appropriately controlled and standardized production and procedures that result in consistent safety and efficacy. Assuring the quality and safety of lentiviru gene therapy products in particular presents a great challenge because they are cell-based m products that include viral and therapeutic proteins as well as modified cells. In addition to the cor refinement of a product, changes in production sites and manufacturing processes have become m more common, posing challenges to developers regarding reproducibility and comparability of This paper discusses the concept of developing a first World Health Organization International St suitable for the standardization of assays and enabling comparison of cross-trial and cross-manufa results for this important vector platform. The standard will be expected to optimize the develop gene therapy medicinal products, which is especially important, given the usually orphan natur diseases to be treated, naturally hampering reproducibility and comparability of results.

Keywords: LV production, WHO standard, integration analysis, genomic DNA, qPCR quantitat

HUMAN GENE THERAPY METHODS, VOLUME 28 NUMBER 4
2017 by Mary Ann Liebert, Inc.

DOI: 10.1089/hgtb.2017.0

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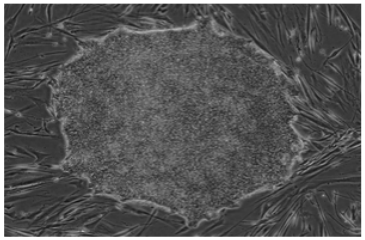
Press release

'Regulator ready' stem cell lines now available for clinical development

From: Medicines and Healthcare products Regulatory Agency
Published: 27 February 2017

The UK Stem Cell Bank (UKSCB) at the National Institute for Biological Standards and Control (NIBSC) is releasing its first stem cell lines suitable for development into novel cell-based medicines to researchers wishing to bring new and innovative therapies to clinical trial.



The stem cell lines are produced and quality-controlled under European regulation and are therefore suitable for use as starting materials in manufacturing therapies for clinical trials, saving researchers precious time and effort.

The UKSCB is a world leading not-for-profit pluripotent stem cell bank distributing stem cell lines qualified for use in clinical trials. Each cell line will be supplied with a certificate of analysis and we are in the process of compiling a starting materials dossier for each of our cell lines which will be available in the near future.

The UKSCB is a trusted supplier of stem cell lines with the highest quality and standards of due diligence and is a favoured partner in stem cell research.

Medicine Manufacturing Industry Partnership

abpi BIA Innovate UK Knowledge Transfer Network

Advanced Therapies Manufacturing Action Plan

Retaining and attracting advanced therapies manufacture in the UK

6. Develop a long-term regulatory strategy and plan for the MHRA to lead in global standards, supporting the scientific activities and international outreach of NIBSC

- Government to recognise the inherent challenge in the standardisation of complex ATMPs and the importance of standardisation in supporting the development of manufacturing processes
- The MHRA, NIBSC and the British Pharmacopoeia (BP) should lead a series of stakeholder engagement meetings with industry, SMEs and academic innovators to identify current gaps in advanced therapies standardisation and address different aspects of cell, gene and viral vector materials, as well as their manufacturing processes and products
- Government must enable and resource MHRA with NIBSC, to work through the challenges of standardising complex ATMP production with relevant parties and ensure that it is properly resourced with funding and expertise to take this critical work forward

NIBSC

Celebrating 40 years of excellence

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