

Hospital Pharmacists taking the lead in Orphan Drugs

Marc Doms (presenter)
Helena Jenzer, Thomas De Rijdt (facilitator)



*EAHP Congress on partnerships and
technologies
Vienna March 2016*

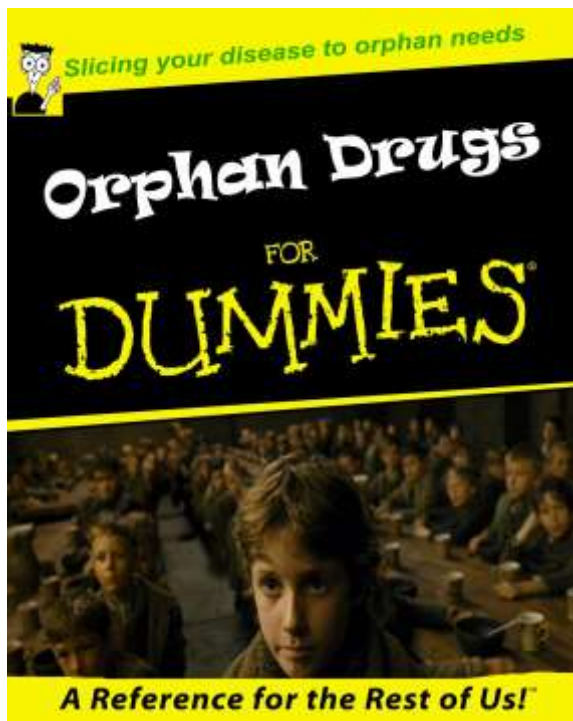
Nothing to



disclose

YES or NO Questions

1. Orphan drugs are a new class of pharmacological agents
2. Cost effectiveness and budget impact is always a problem
3. Orphan drugs are innovative medicinal products



Observationum Exempla Rara



Definition Orphan Drugs



“Medicinal products intended for the diagnosis, prevention or treatment of a life-threatening condition affecting no more than 5 in 10 000 persons in the EU”



EMA 2000

Chemical Classification System (WHO)

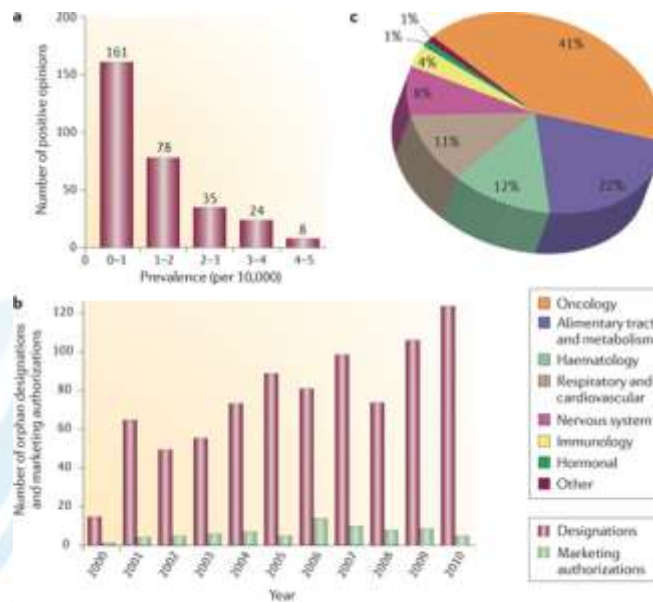
letter	code	Omschrijving hoofdgroep (eerste letter)
A	ATC code A	Spijverteringsstelsel en metabolisme
B	ATC code B	Bloed en bloedvormende organen
C	ATC code C	Cardiovasculair systeem
D	ATC code D	Dermatologica
G	ATC code G	Genito-urinaire systeem en geslachtshormonen
H	ATC code H	Systemische hormonale preparaten , met uitzondering van insuline en geslachtshormonen
J	ATC code J	Antinfectie middelen voor systemisch gebruik
L	ATC code L	antineoplasie en immunomodulerende stoffen
M	ATC code M	Spier- en skeletstelsel
N	ATC code N	Zenuwstelsel
P	ATC code P	Antiparasitische middelen , insecticiden en repellents
Q	ATC code Q	Veterinaire geneesmiddelen
R	ATC code R	Ademhalingsstelsel
S	ATC code S	Sensorische organen
V	Varia	



- Protocol assistance
- Market exclusivity (10 years)
- Financial incentives (fee reduction)
- Centralized EU-procedure
- National incentives (reimbursement)
- Research support
- etc...



- 119 authorised orphan drugs (1621 designated)
- 106 rare diseases
- 33 withdrawn or suspended
- 80 non-orphan status

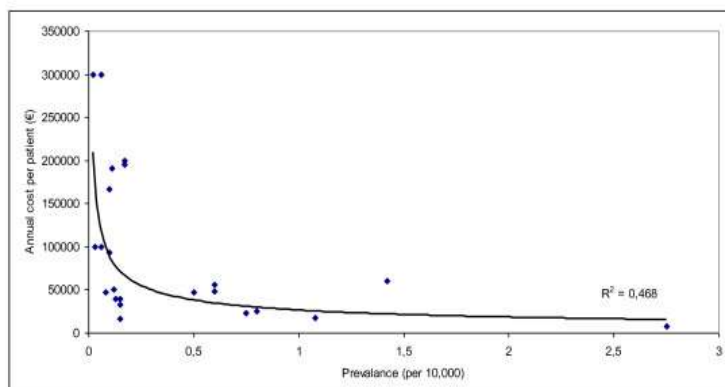


Route of administration



UZ Leuven | Herestraat 49 | 3000 Leuven | www.uzleuven.be | tel: +32 (0) 30 30 30 30 | UNIFORMITEIT HOSPITALS LEUVEN

Cost and Prevalence Orphan Medicines



Bron : prof. dr. Steven Simoens - Onderzoekscentrum voor Farmaceutische Zorg en Farmaco-economie

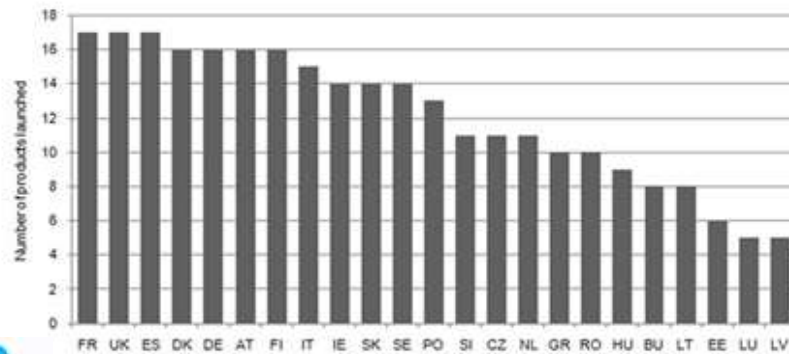
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Reimbursement in Europe



Total number of orphan drugs launched in 2001-2009 per country



Prevention



NO orphan autorisation

- Folic acid 0,4 mg / 4 mg

Orphan Autorisation

- Japanese encephalitis vacc





NO orphan drug autorisation

- Caprelsa (vandetanib)
- Inlyta (axitinib)
- Iressa (gefitinib)
- Tarceva (erlotinib)
- Tyverb (lapatinib)
- Votrient (pazopanib)
- Xalkori (crizotinib)
- Zelboraf (vemurafenib)

Orphan drug autorisation

- Glivec (imatinib)
- Nexavar (sorafinib)
- Sprycel (dasatinib)
- Tassigna (nilotinib)
- Sutent (sunitinib)

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Oliver Twist, Roman Polanski, Charles Dickens.

M. Doms



Zinc acetate



Thalidomide



“On fera sécher au soleil le sang tiré d’un jeune homme sain ...”

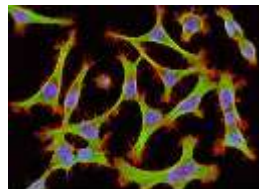
Lemery, Pharmacopée Universelle. 1647



Hernietta Lacks



HeLa cells



22

Hernietta Lacks

ATCC® IN PARTNERSHIP WITH LGC

PRODUCTS SERVICES STANDARDS

Home » Products » All Products » CCL-2™

HeLa (ATCC® CCL-2™)

Organism: *Homo sapiens, human* / Cell Type: *epithelial*

GENERAL INFORMATION		CHARACTERISTICS
Organism	<i>Homo sap</i>	
Tissue	cervix	
Cell Type	epithelial	
Product Format	frozen	
Morphology	epithelial	

DOCUMENTATION

SEARCH BY KEYWORD OR CATALOG NO.

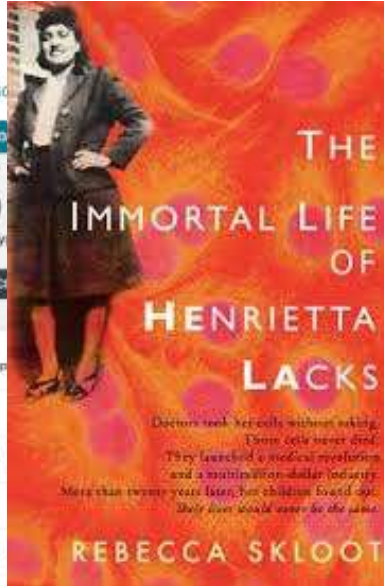
ADVANCED SEARCH

PRODUCT SUPPORT ABOUT

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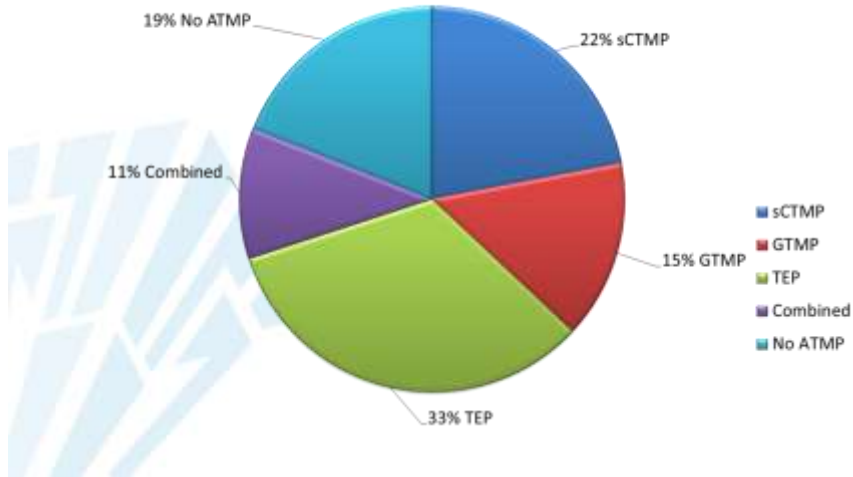
Advanced Therapy Medicinal Products



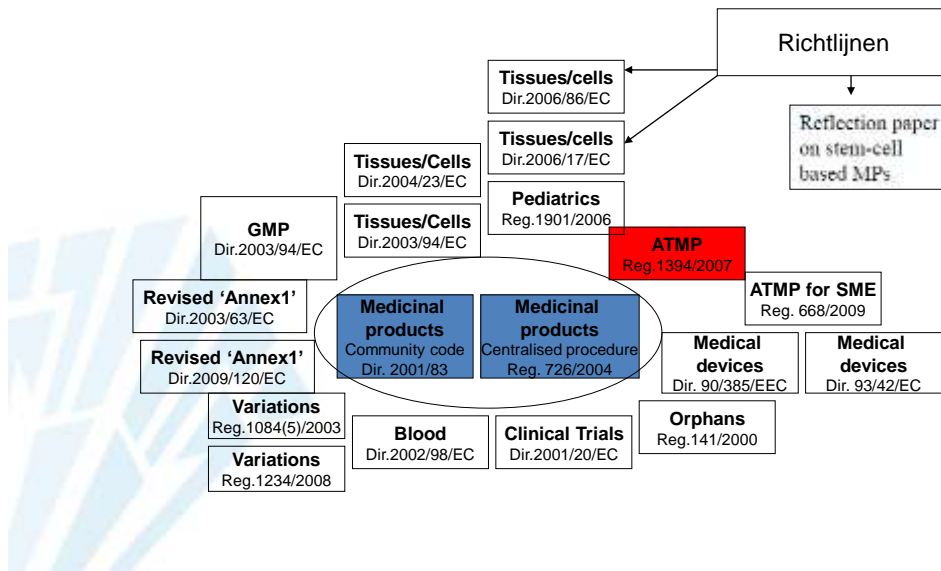
AND



Classification ATMP



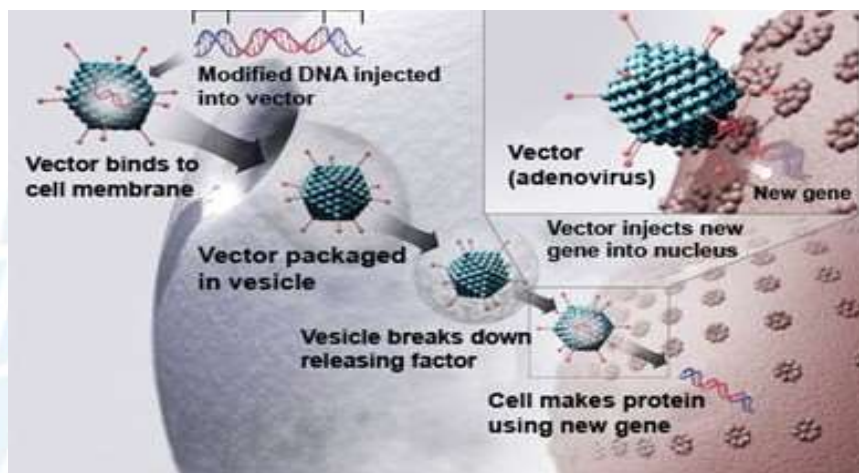
European Legislation



Hospital exemption in Europe

LIDSTAAT	NATIONALE AUTORITEIT (1)	RICHT-SNOER (2)	RET-ROUWREISWEG (3)	(IMPORT) EXPORT (4)	GMP (5)	GP (6)	TOEGEBORRE ATP's (9.7)
UK	MHRA	Ja + Speciale (8)	Aparte notitieer (8)	Nee (9) Tweelkens ziekenhuizen binnen de UK- WDL (8)	Ja (8) (10)	Nee (8)	Tegenstrijdige bronnen: 1 of 0?
Ierland	IMB	Ja	/	/	Ja (8)	/	0
Frankrijk	ANSM	Nee	/	Nee (9)	Ja (9)	Verantwoordelijke persoon gespecificeerd (9)	0
Duitsland	PEI	Ja	Specificatie in de AMG (sectie 4b §2) (12)	Nee (11) Ongespecificeerde afwijking voor gezondheidszorg (bv. artspraktijk) in TFG (sectie 14, sub- sectie 2) (11)	/	Specifieke voorwaarde in AMG (sectie 15, sub- sectie 3a) HAAK ook op voor- hospital exemption? (10)	0
Oostenrijk	BASG/AGES	Nee	/	/	/	/	0
Nederland	IGZ	Ja	Opgenomen in notitieer van ME (12)	Nee (12)	Ja (12)	Ja (12,13)	3 (13)

Glybera (Alipogene Tiparvovec)



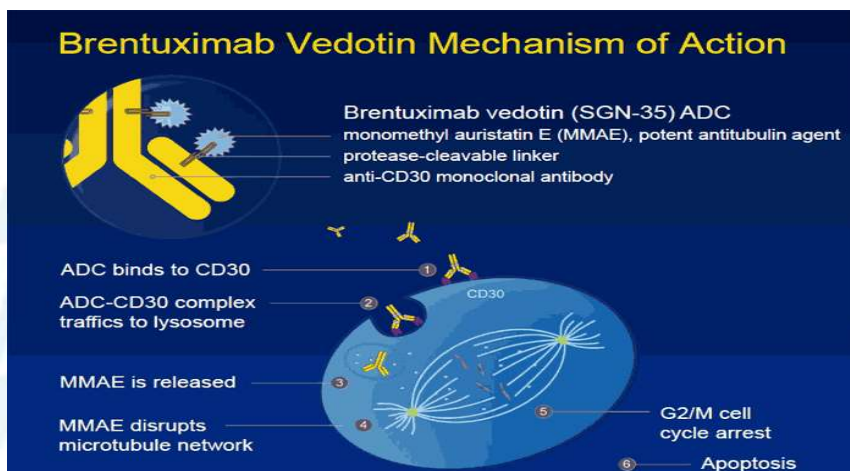
Kalydeco (ivacaftor)

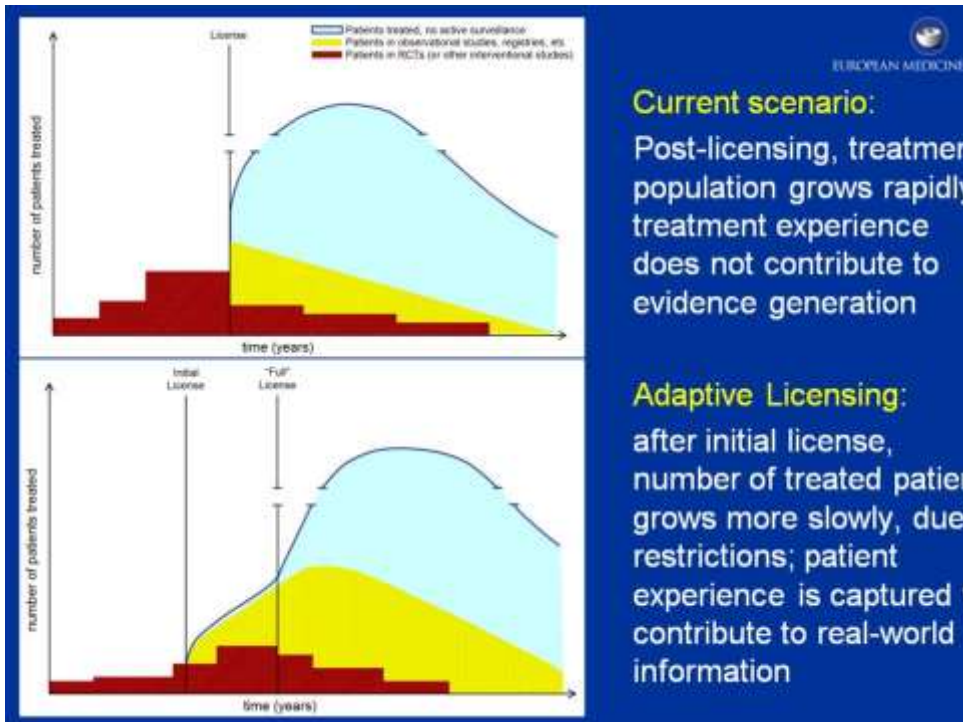


- Subpopulation CF (genetic mutation G551D)
- Personalized medicine: treats genetic defect
- PPP: contributions of the CF community & research funded by NIH et al
- Clinical trials in CF patients



ADCETRIS: lymphoma





Generics & Biosimilars

spot the difference





First-Aid-Kit



Home treatment

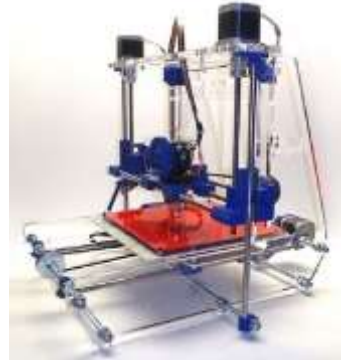


Risk Minimisation Activities



- 22 orphan drugs

3D Printing in Medicine



Pharmaco-Economy

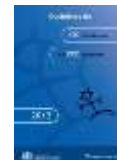


- Performance based agreement
- Early treatment access
- Conditional reimbursement
- Multi-criteria decision analysis



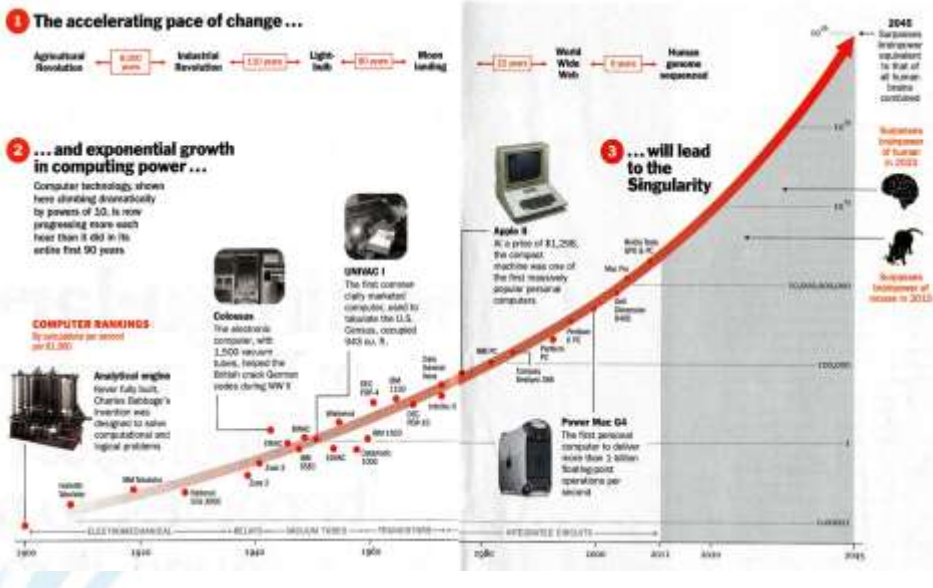
Multi-criteria decision analysis

- A set of methods and approaches to aid decision making, where decisions are based on more than one criterion, which make explicit the impact on the decision of all the criteria applied and the relative importance attached to them.



Patient-Reported Outcome Measures



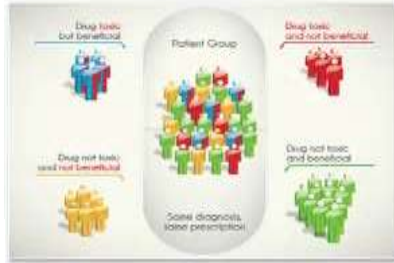


Pharmaco-Genetics

Companion Diagnostics

**PRECISION
MEDICINE**

Drug Prescription and Diagnosis Among the same Patient Group



Integrated Delivery Systems



TheKingsFund

Ideas that change health care

Canterbury
District Health Board
To Plan, Provide & Monitor

Approach to coordinated care

Maccabi has a comprehensive, fully integrated health information and communication system with a comprehensive database that includes more than 18 years of data on almost 2 million members. Maccabi functions on the basis of a decentralized organizational approach.



Figure 1: Maccabi coordinated care. Networking and Decision Support Systems are at the heart of delivering coordinated care to Maccabi members.

Maccabi, 2011 - <http://www.intel.co.uk/content/dam/www/public/us/en/documents/white-papers/coordinated-health-care-from-maccabi-han-sintel-paper.pdf>



1. Prevention
2. Orientation
3. Pharmaceutical Care



Pharmaceutical Compounding



CONTRA-INDICATION

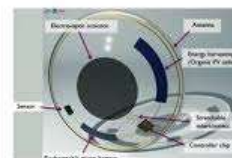




Internet of (wearable) things



Example of smart contact lens (Google)



One of the possible architecture for a smart multifocal contact lens (imec/Ghent University)



I of Things – I of Everything



The Onlife Initiative



Synthetic Biology



YES or NO Questions

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