Orphan Drug Policies: Dynamic Global Landscape

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Sr Director Global Policy Programs

21 May 2008

Overview

- Orphan Drug Policy: Today
- Retelling the story: Patient, Product & Policy
- Access equation
- Orphan Drug Policies: Tomorrow
- Role for collaboration

Our Commitment to Patients

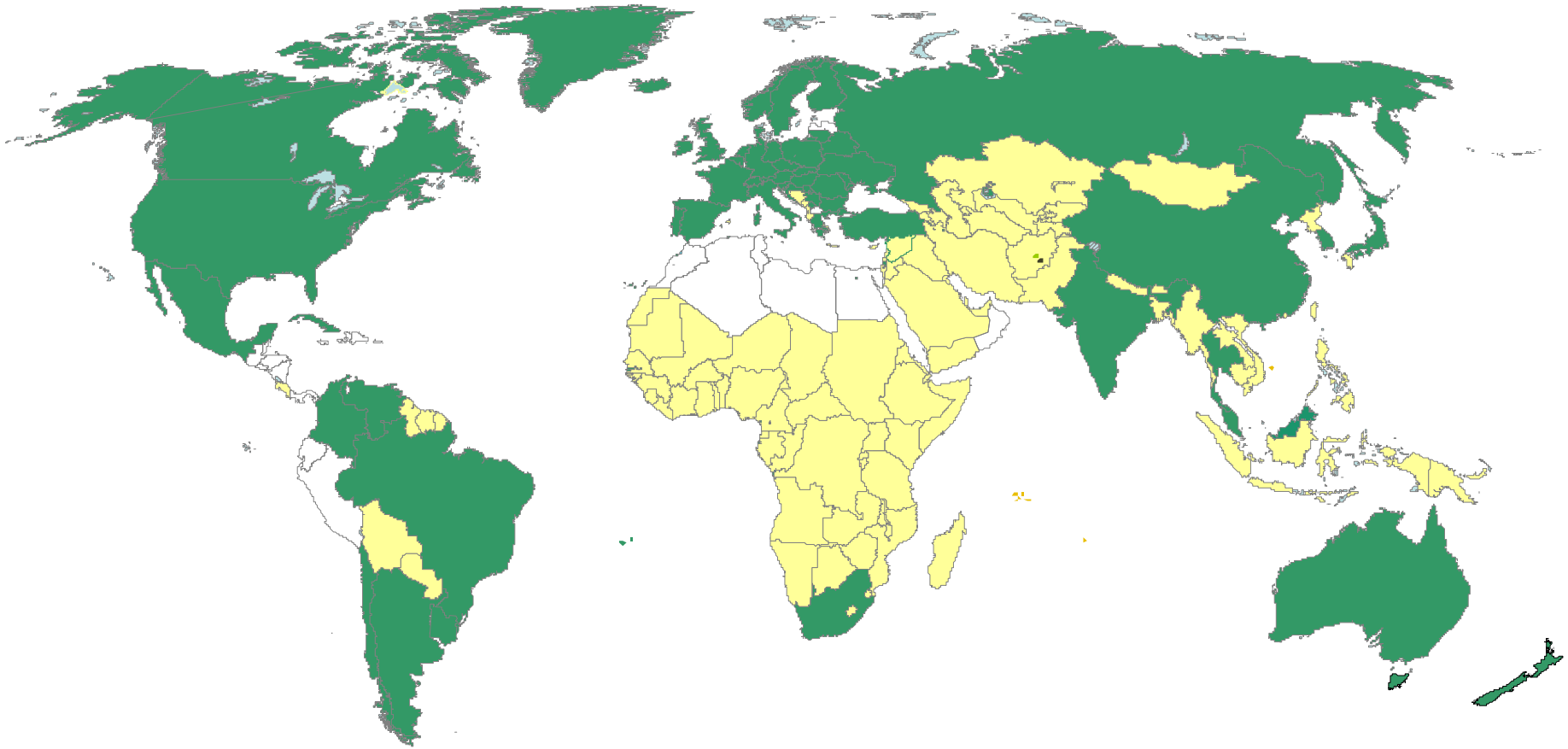
Developing life-saving therapies carries with it the responsibility to increase access to health care for patients around the world

Sponsoring programs to ensure that patients around the world have access to the treatments they need.

Promoting access by working with physicians and governments in developing countries to build sustainable health care systems.

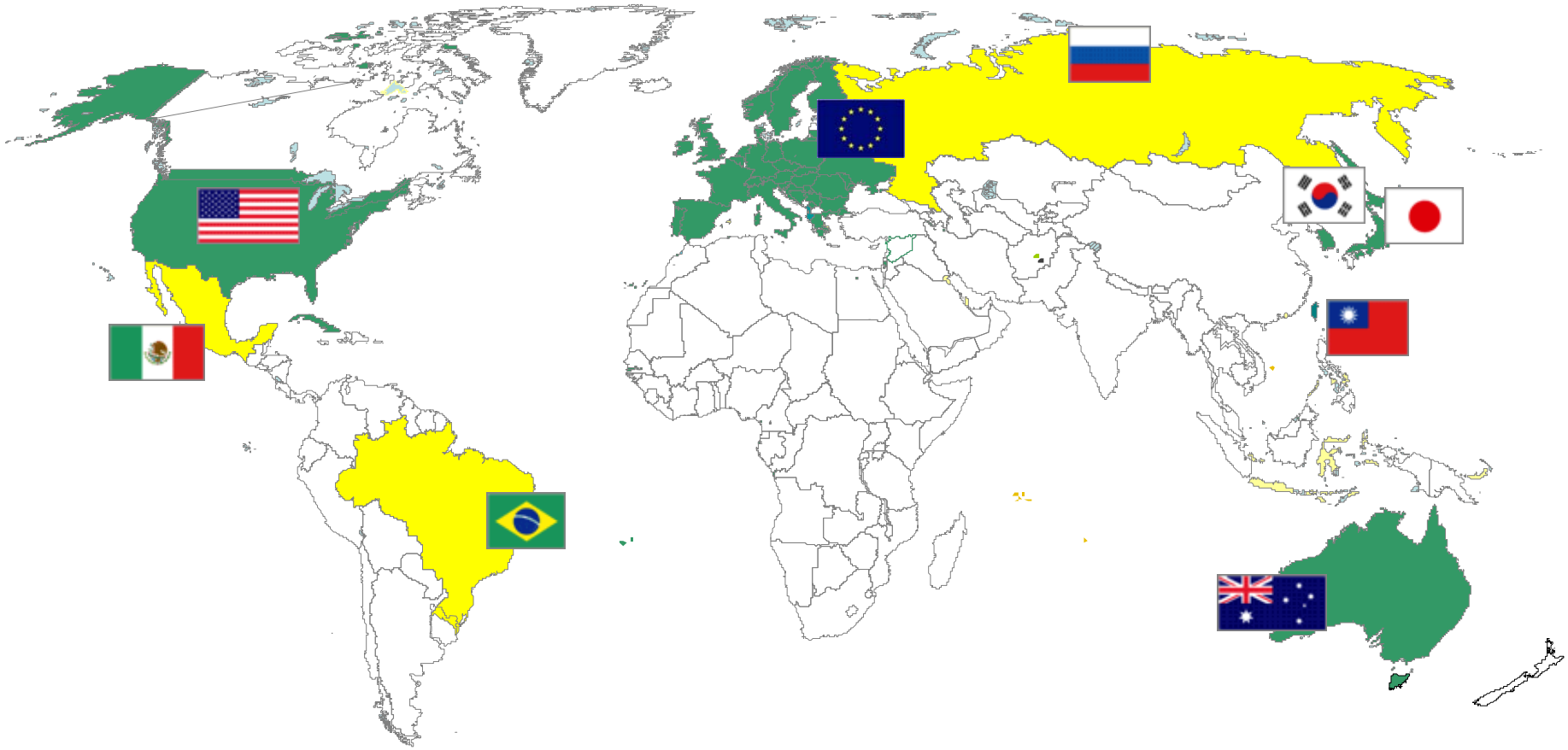
Genzyme Supports Treatment of Patients in ~90 countries

- No presence
- Direct

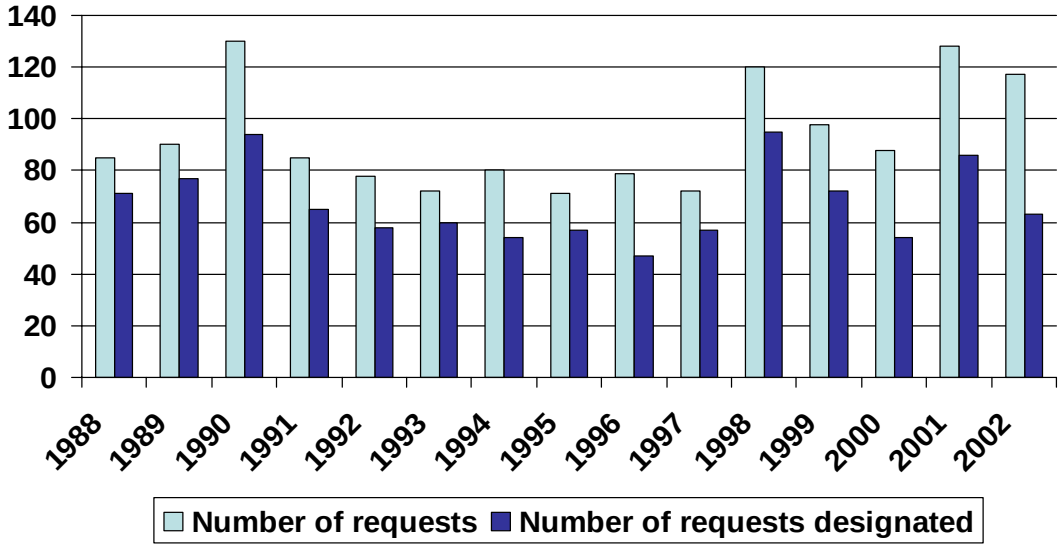


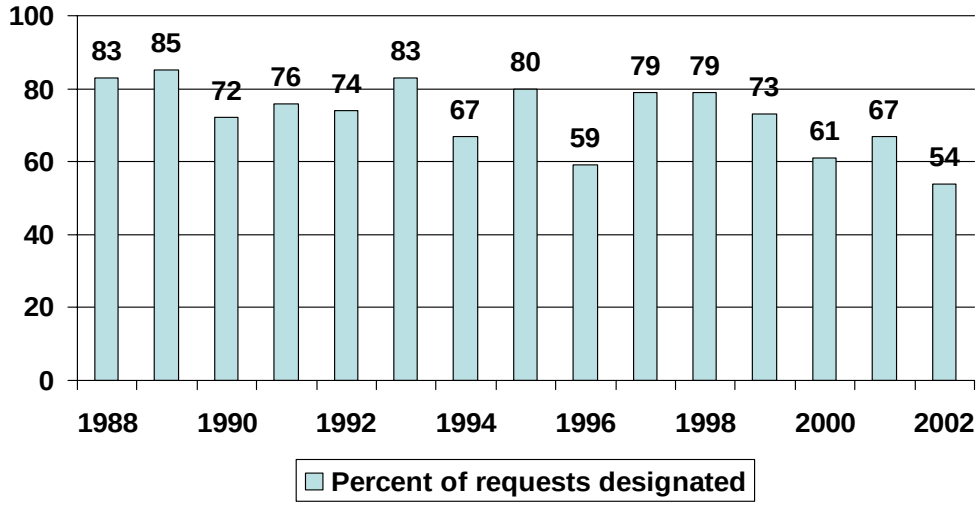
Countries with Orphan Drug Policies are Few

- Partial
- Formal



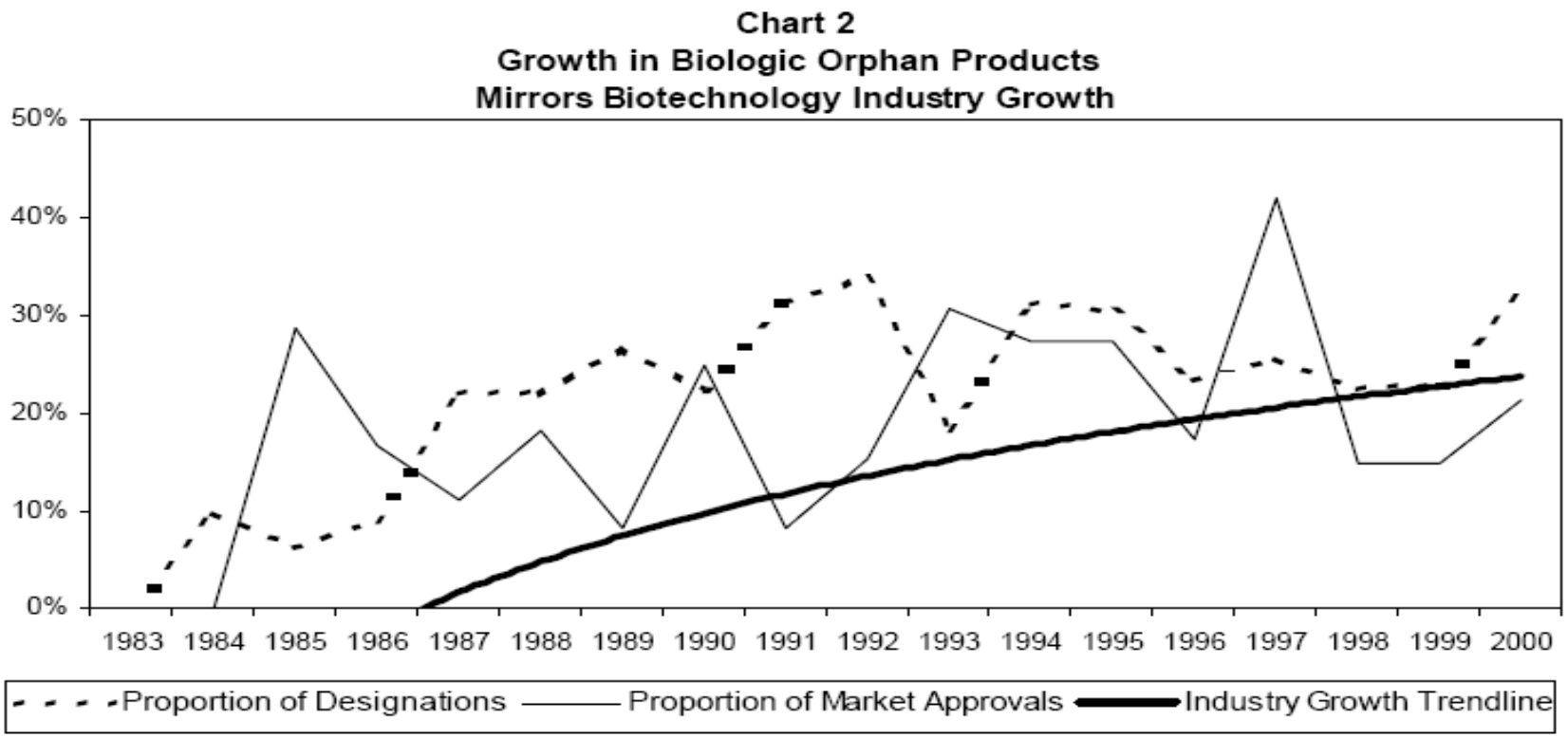
Orphan Drug Policy: Measures of Success





- >325 Orphan Products approved since 1983 in US
- >45 Orphan Products approved since 2000 in EU

Biotechnology offers solutions to Rare Diseases



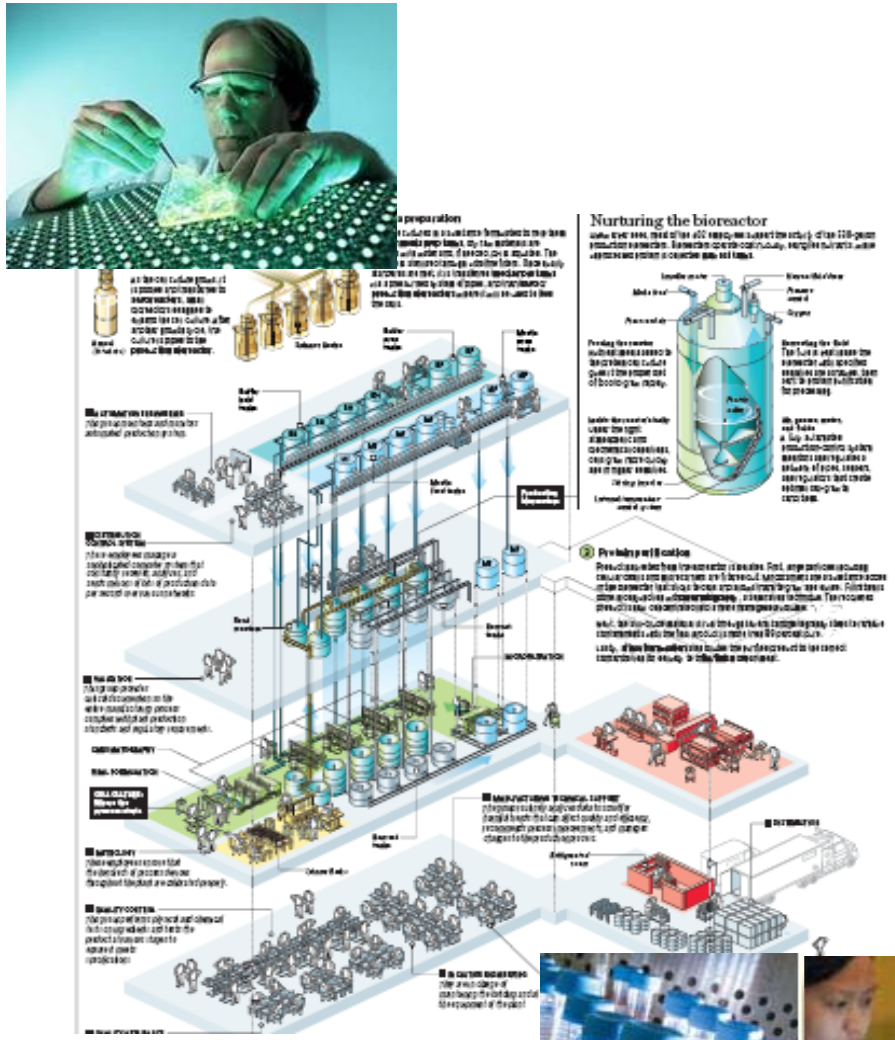
US DHHS OIG: ODA Implementation & Impact, 2001

Patient's Story

Patricia Hand

Helen Walker

Product's Story



STOP

Canadian Agency for Drugs and Technologies in Health

IQWiG Institute for Quality and Efficiency in Health Care

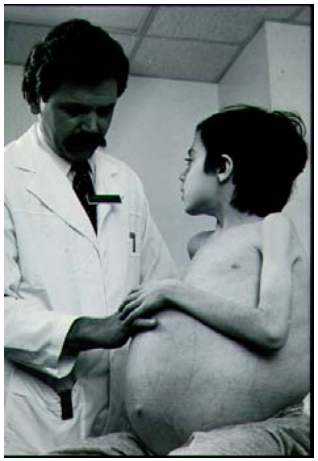
FDA

European Medicines Agency emea

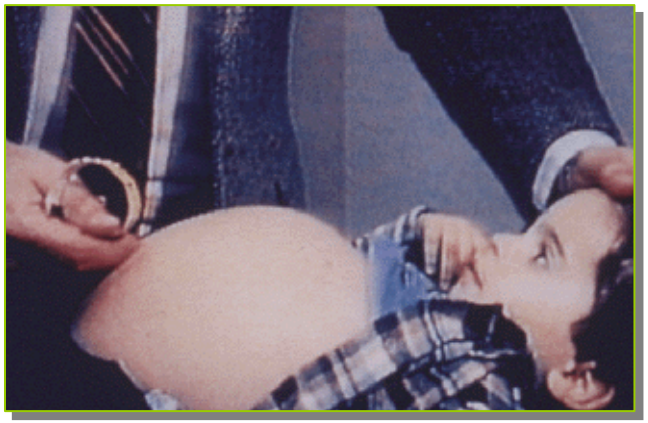
Australian Government
Department of Health and Ageing

NHS
National Institute for Health and Clinical Excellence

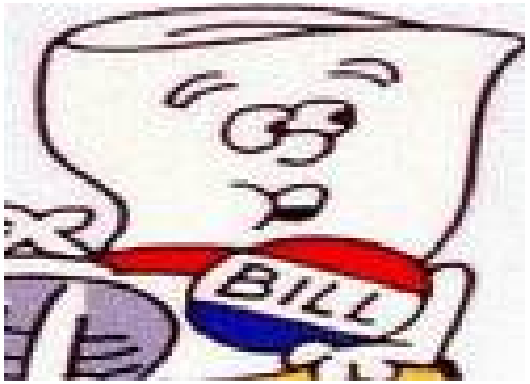
Policy's Story



Awareness



Appreciation



Action

FDA U.S. Food and Drug Administration

FDA Home Page | Search FDA Site | FDA A-Z Index | Contact FDA

The Orphan Drug Act (as amended)

CONGRESSIONAL FINDINGS FOR THE ORPHAN DRUG ACT

The Congress finds that---

(1) there are many diseases and conditions, such as Huntington's disease, myoclonus, ALS (Lou Gehrig's disease), Tourette syndrome, and muscular dystrophy which affect such small numbers of individuals residing in the United States that the diseases and conditions are considered rare in the United States;

(2) adequate drugs for many of such diseases and conditions have not been developed;

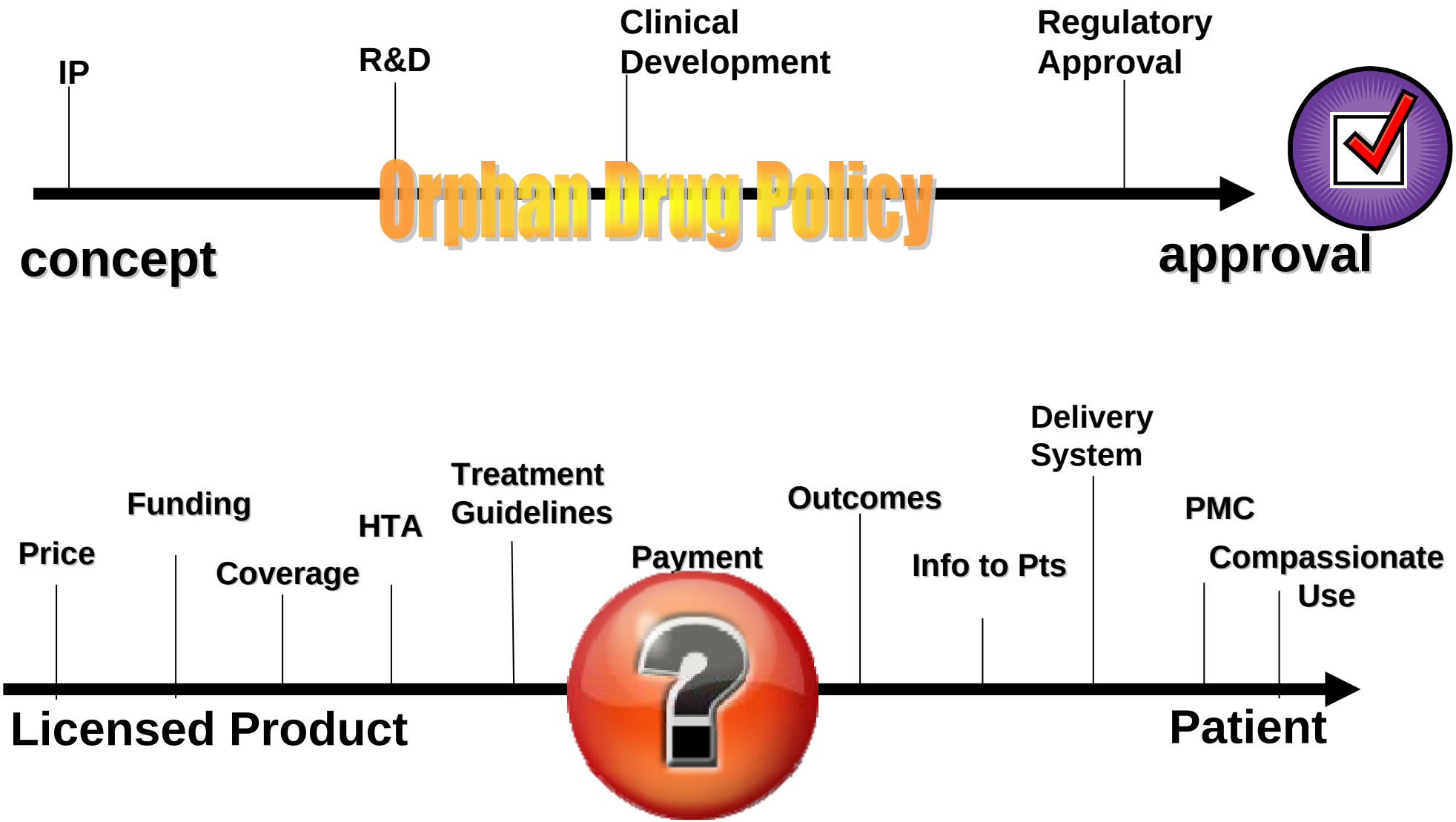
(3) drugs for these diseases and conditions are commonly referred to as "orphan drugs";

(4) because so few individuals are affected by any one rare disease or condition, a pharmaceutical company which develops an orphan drug may reasonably expect the drug to generate relatively small sales in comparison to the cost of developing the drug and consequently to incur a financial loss;

(5) there is reason to believe that some promising orphan drugs will not be developed unless changes are made in the applicable Federal laws to reduce the costs of developing such drugs and to provide financial incentives to develop such drugs; and

(6) it is in the public interest to provide such changes and incentives for the development of orphan drugs.

Access Equation is Two-Fold



Considerations for Tomorrow's Orphan Drug Policies

#1) "Correlation Creep"

Country Legislation | Science | Innovation | Technology

$$\frac{\text{Orphan Drug}}{\cancel{\text{Rare Disease}}} \times \frac{\cancel{\text{Rare Disease}}}{\cancel{\text{Genetic}}} \times \frac{\cancel{\text{Genetic}}}{\cancel{\text{Biotech}}} \times \frac{\cancel{\text{Biotech}}}{\text{Dev Costs}} = \frac{\cancel{\text{Dev Costs}}}{\cancel{\text{Rarity}}}$$

High Cost

Orphan Drug = High Cost

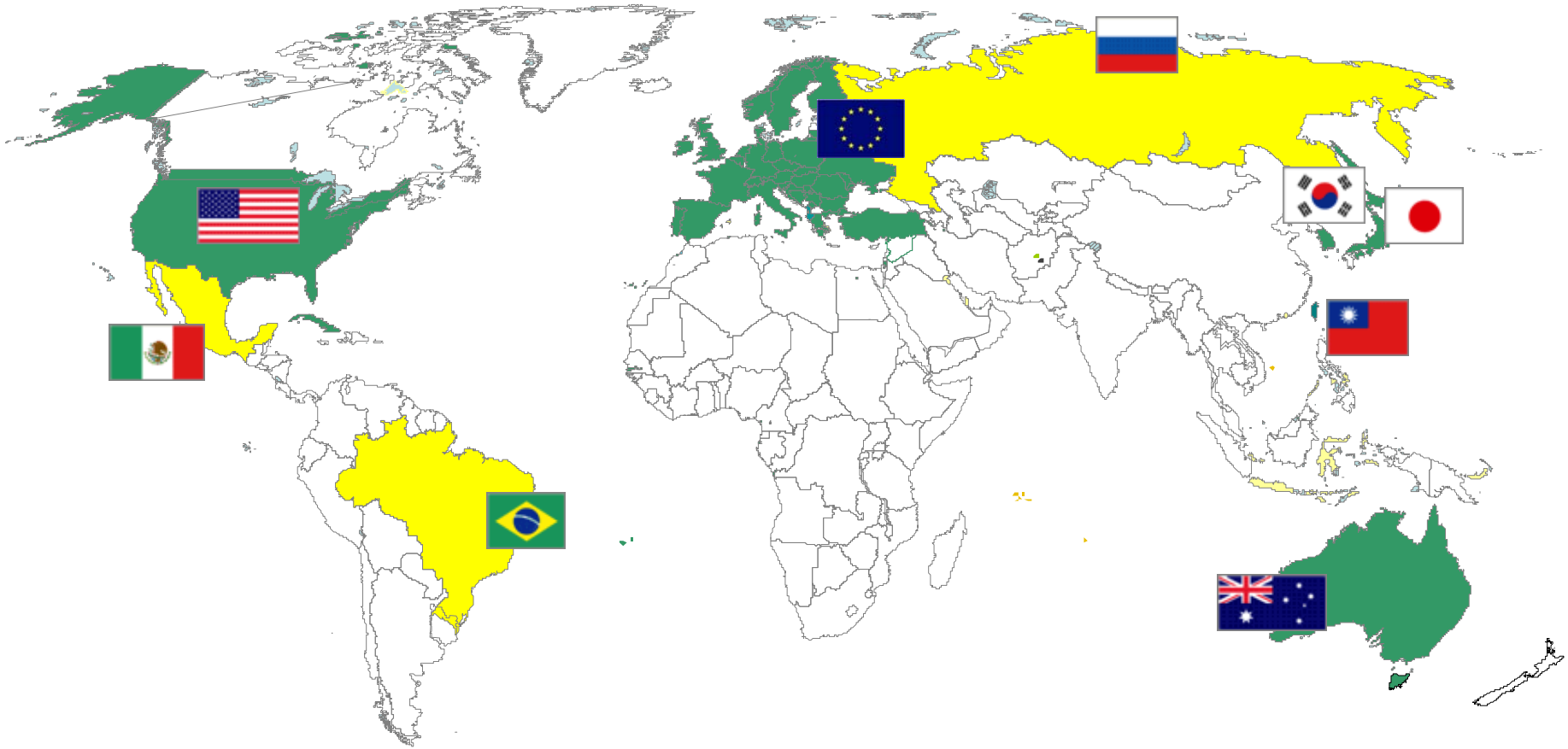
Orphan Drug = Life Saving

Considerations for Tomorrow's Orphan Drug Policies (cont.)

#2) "First Encounter" for Many Countries

-[Research], Registration, Reimbursement

- Partial
- Formal



Considerations for Tomorrow's Orphan Drug Policies (cont.)

#3) Rare Diseases in the Global [Public] Health Agenda

Editorials

Are drugs for rare diseases "essential"?

Marcus M Reidenberg[a]

In 1977, the first report of the WHO Expert Committee on the Use of Essential Drugs defined essential drugs as those needed to satisfy the health-care needs of the majority of the population. This was done in order to fulfil a mandate to assist Member States in selecting and obtaining essential medicaments for their populations. The Expert Committee then developed criteria for determining if a drug fitted this definition and published a Model List of Essential Drugs as an example of how the concept of essential drugs could be implemented.

A brief review of the state of medications at that time explains the need for this assistance: resources were limited in many countries, so the goal was to use them wisely, and many drugs marketed around the world were ineffective or irrational combination products. A review of the evidence of efficacy of all prescription drugs on the market in the United States starting in 1966 found that about one-third of the over 3000 marketed drugs were not effective.[1] In addition, there were frequently several effective drugs in the same therapeutic class: all did not need to be stocked by health service pharmacies. The essential drugs concept and the methods for its

In this issue, Stolk et al. identify changes in the policies of some governments to facilitate the discovery and development of drugs for uncommon diseases. They propose an additional complementary Orphan Medicines Model List to include drugs for rare diseases in the Essential Medicines Programme of WHO.[4] They propose criteria for including a drug in the suggested list; cost is not one of the How should treatments for uncom or rare diseases be considered, if a for an essential medicines list? Afte there are more than 6000 rare dise and related conditions listed on the National Institutes of Health web

Aristotle raised the principle of distributive justice, the proper distr tion of benefits and burdens, to ad this question.[5] Is it right for one pe to benefit from a health service and another patient to be ignored only cause of the prevalence of their illne Is there a better way to select which medicines to purchase than to pur only those for common problems? patients with rare diseases irrelevan the health-care needs of a populat

One way to make decisions ab

formal analysis may not be needed for decision-making while technical competence in cost–effectiveness analysis is developed to help with more difficult decisions.

If the definition of an essential medicine is to be changed to include

Policy and Practice

"Rare essentials": drugs for rare diseases as essential medicines

Pieter Stolk,[a] Marjolein JC Willemen,[a] & Hubert GM Leufkens[a]

Abstract Since 1977, the WHO Model List of Essential Medicines (EML), published by WHO, has provided advice for Member States that struggle to decide which pharmaceutical technologies should be provided to patients within their public health systems. Originating from outside WHO, an incentive system has been put in place by various governments for the development of medicines for rare diseases ("orphan drugs"). With progress in pharmaceutical research (e.g. drugs targeted for narrower indications), these medicines will feature more often on future public health agendas. However, when current definitions for selecting essential medicines are applied strictly, orphan drugs cannot be part of the WHO Essential Medicines Programme, creating the risk that WHO may lose an drugs in its policy sphere by composing a complementary lementary list of "rare essentials" could aid policy-makers o these drugs and stimulate relevant policies. Furthermore, iseases can be resolved. In this paper we propose selection e point for future work towards an extensive WHO Orphan

spañol. يمكن الاطلاع على الملخص بالعربية في صفحة 750.

ras published from outside gs" movement ly in affluent 1980s to cre- evelopment of es.[4] Because of tial, such drugs harmaceutical d market. ms of prioritiz- ting incentives e orphan drug HO Essential any differences nd conceptual oming increas- nare common ntial medicines h orphan drugs ority agenda of urgent popu- a high disease s may change ome onto the

market. For example, orphan drugs currently constitute about 15% of new centralized authorizations in the European Union (EU), there is increasing attention for "rare diseases" in emerging countries (e.g. Egypt, India) and more spin-offs of orphan drug innovations with implications for drug treatment in general (e.g. imatinib mesylate, used for the treatment of chronic myeloid leukaemia).[5] In this paper, we review recent advances in the fields of orphan drugs and essential medicines, and propose how WHO may develop an approach to provide useful advice to Member States that want to improve access to treatments using orphan drugs. For this purpose, we would like to recommend the creation of a complementary WHO Model List for Orphan Medicines as an addition to the current EML. Furthermore, we aim to provide a framework for analysing future questions surrounding the selection of "essential orphan medicines", or "rare essentials".

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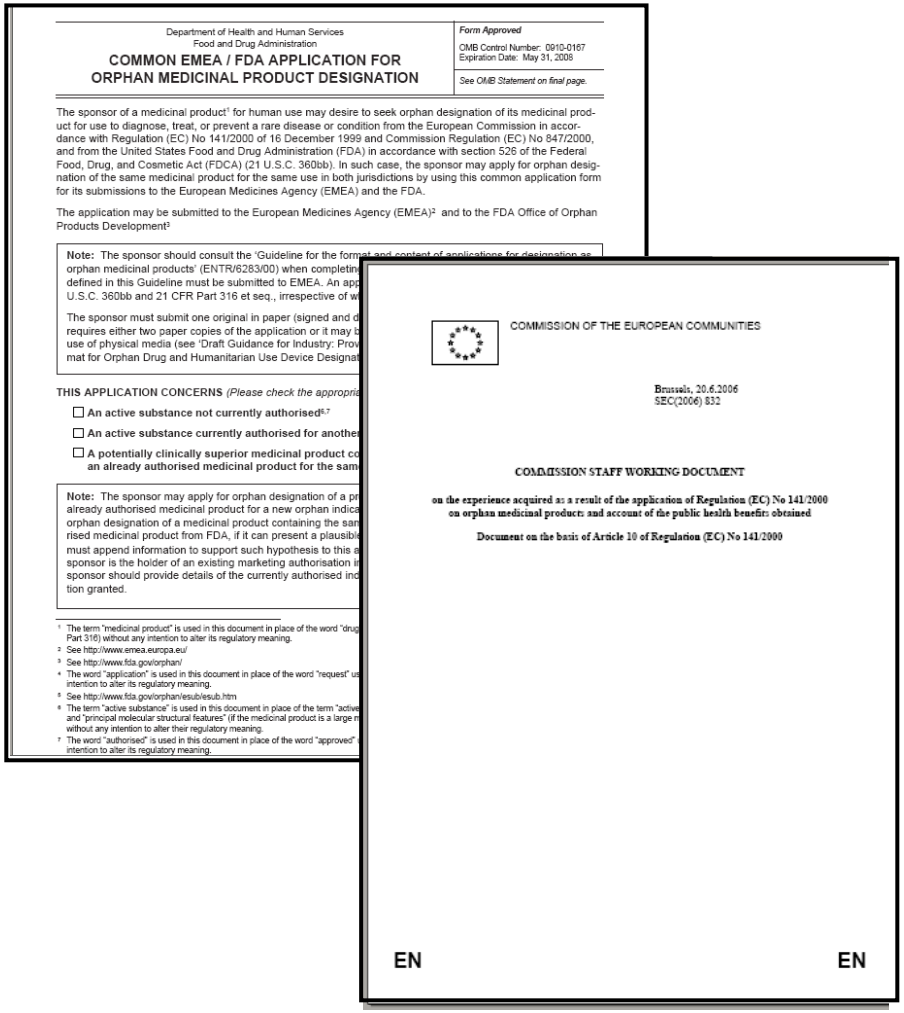
- Healthcare Priority Setting
- European Commission Public Health Program
  - 2003/2008 v 2008/2013
- WHO "Essential" Medicines List
- Linkage with Neglected Diseases Initiatives
- Local Manufacture of Orphan Drugs

Considerations for Tomorrow's Orphan Drug Policies (cont.)

#4) Role of International Collaboration



Department of Health and Human Services
Food and Drug Administration

COMMON EMEA / FDA APPLICATION FOR ORPHAN MEDICINAL PRODUCT DESIGNATION

Form Approved
OMB Control Number: 0910-0167
Expiration Date: May 31, 2008

See OMB Statement on final page.

The sponsor of a medicinal product[1] for human use may desire to seek orphan designation of its medicinal product for use to diagnose, treat, or prevent a rare disease or condition from the European Commission in accordance with Regulation (EC) No 141/2000 of 16 December 1999 and Commission Regulation (EC) No 847/2000, and from the United States Food and Drug Administration (FDA) in accordance with section 526 of the Federal Food, Drug, and Cosmetic Act (FDCA) (21 U.S.C. 360bb). In such case, the sponsor may apply for orphan designation of the same medicinal product for the same use in both jurisdictions by using this common application form for its submissions to the European Medicines Agency (EMEA) and the FDA.

The application may be submitted to the European Medicines Agency (EMEA)[2] and to the FDA Office of Orphan Products Development[3]

Note: The sponsor should consult the 'Guideline for the format and content of applications for designation as orphan medicinal products' (ENTR/6283/00) when completing
defined in this Guideline must be submitted to EMEA. An app
U.S.C. 360bb and 21 CFR Part 316 et seq., irrespective of w

The sponsor must submit one original in paper (signed and d
requires either two paper copies of the application or it may b
use of physical media (see 'Draft Guidance for Industry: Prov
mat for Orphan Drug and Humanitarian Use Device Designat

THIS APPLICATION CONCERNS *(Please check the appropria*

- ☐ **An active substance not currently authorised**[6,7]
- ☐ **An active substance currently authorised for anothe**
- ☐ **A potentially clinically superior medicinal product co an already authorised medicinal product for the sam**

Note: The sponsor may apply for orphan designation of a pr
already authorised medicinal product for a new orphan indica
orphan designation of a medicinal product containing the sam
rised medicinal product from FDA, if it can present a plausibl
must append information to support such hypothesis to this a
sponsor is the holder of an existing marketing authorisation i
sponsor should provide details of the currently authorised ind
tion granted.

[1] The term "medicinal product" is used in this document in place of the word "drug
Part 316) without any intention to alter its regulatory meaning.
[2] See http://www.emea.europa.eu/
[3] See http://www.fda.gov/orphan/
[4] The word "application" is used in this document in place of the word "request" us
intention to alter its regulatory meaning.
[5] See http://www.fda.gov/orphan/esub/esub.htm
[6] The term "active substance" is used in this document in place of the term "active
and "principal molecular structural features" (if the medicinal product is a large m
without any intention to alter their regulatory meaning.
[7] The word "authorised" is used in this document in place of the word "approved"
intention to alter its regulatory meaning.

COMMISSION OF THE EUROPEAN COMMUNITIES

Brussels, 20.6.2006
SEC(2006) 832

COMMISSION STAFF WORKING DOCUMENT

on the experience acquired as a result of the application of Regulation (EC) No 141/2000 on orphan medicinal products and account of the public health benefits obtained

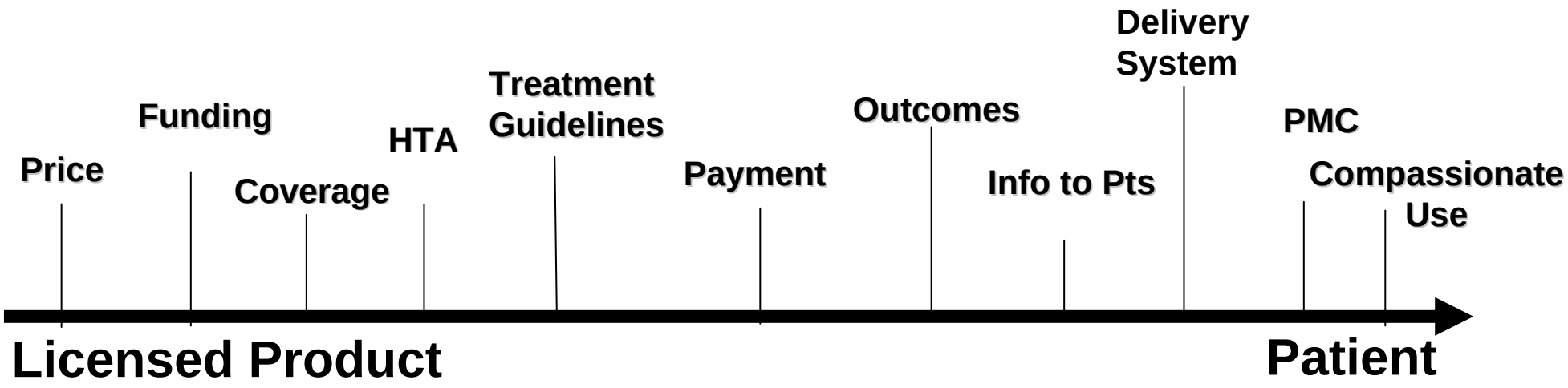
Document on the basis of Article 10 of Regulation (EC) No 141/2000

EN EN

- Global Rare Disease Community
  - Multi-stakeholder
- Common Designation Application Pathway
- Common Language
  - WHO ICD Initiatives
- Common Purpose
- Sharing of Best Practice

Considerations for Tomorrow's Orphan Drug Policies (cont.)

#5) Orphan Drug Designation in the Access Equation (Part II)



- How could Orphan Drug Status apply in this policy environment?
  - Short term: "Chance for Life"
  - Long term: Linking innovation to access / Sustainability

Summary

- Orphan Drug Policies have made a significant impact in the lives of patients with rare diseases
- Biotechnology has played an important role
- Access equation is only partially addressed
- Tomorrow's Orphan Drug Policies should
  - Keep rare disease patients as the primary focus
  - Apply OD status in entire access equation
  - Be interconnected with global public health priorities
  - Leverage 25 years of policy-making experience
  - Preserve and value innovation
  - Encourage international collaboration

Thank You