UZ LEUVEN | KINDERGENEESKUNDE

Access to and reimbursement for diagnosis and therapy

Tumor vaccination for brain cancer
Barriers, hurdles, problems, threats, challenges

Medicinal products for orphan diseases

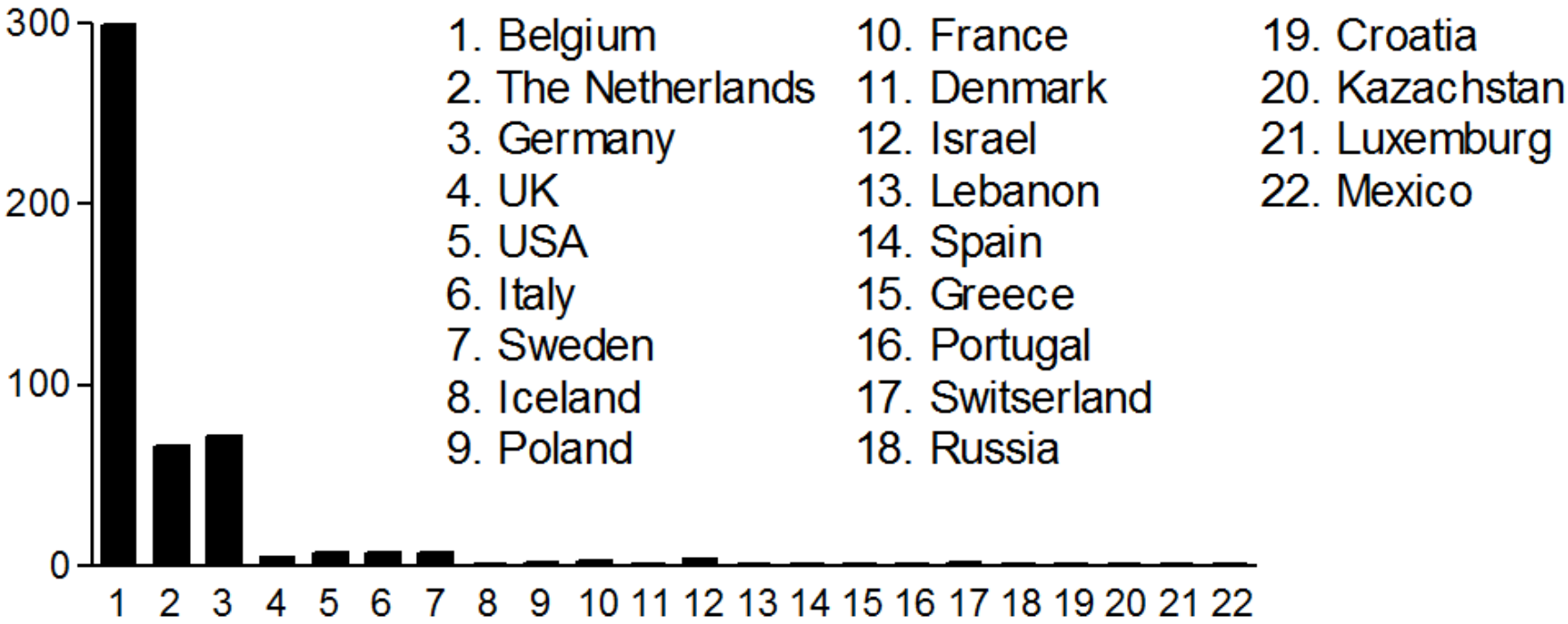
Stefaan Van Gool

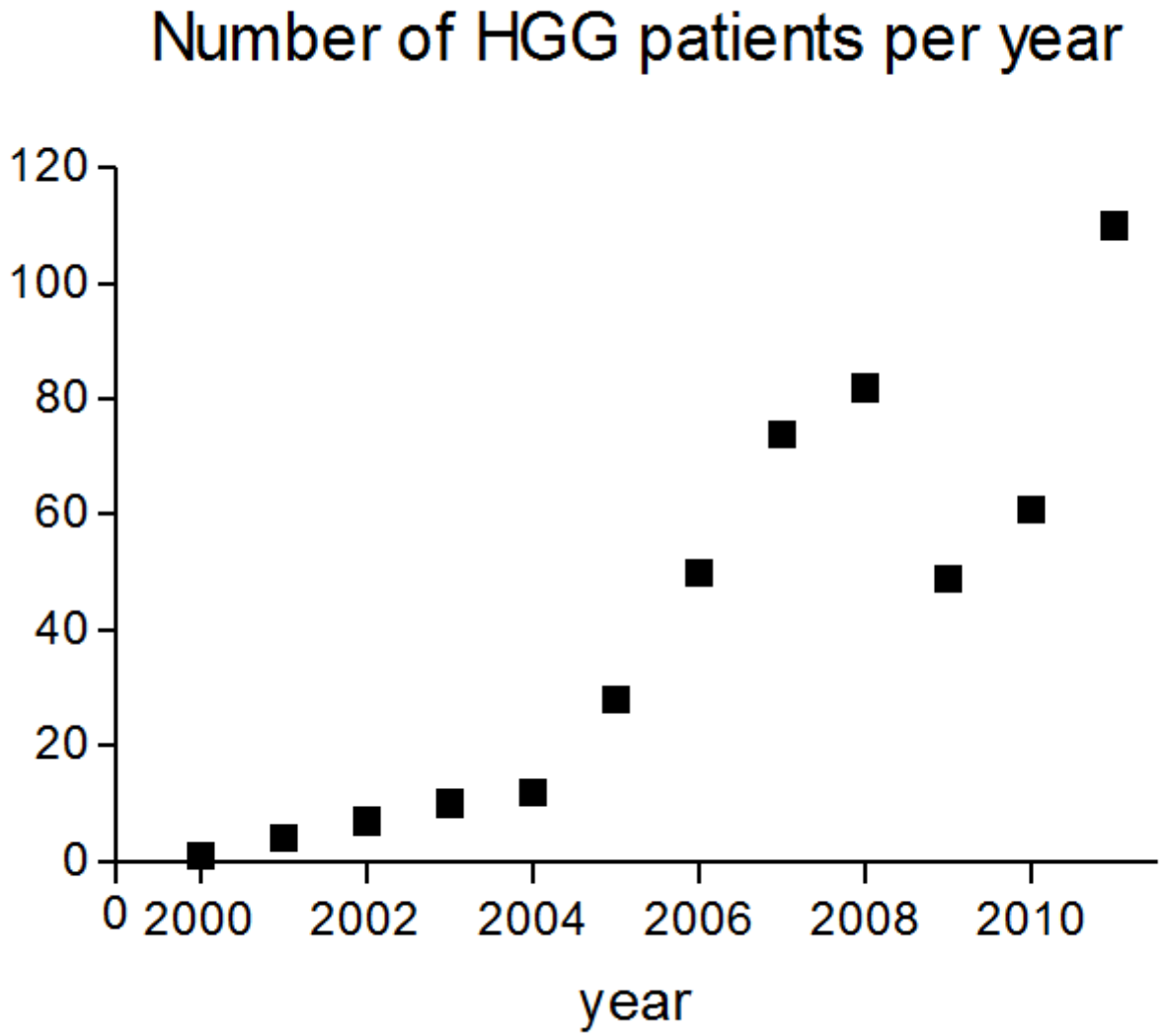
No conflict of interest

- Shareholder: NO
- Grants: FWO-V, IWT, Stichting tegen kanker, VLK
- Consultant: NO
- Employee: NO
- Paid Instructor: NO
- Speakers Bureau: NO
- Charity: www.olivia.be, www.hmrf.be, www.jekfoundation.org

Accessibility for immunotherapy against malignant glioma

- Information and visibility of new technologies
  - To the medical community → scientific approach
  - To the patients → balance in layman language and communication fora
- Administrative hurdles, medical challenges: from cell traffic to patient traffic: **collaboration**
- Inclusion criteria within clinical trials
- Immunotherapy platform Leuven: www.itpl.be

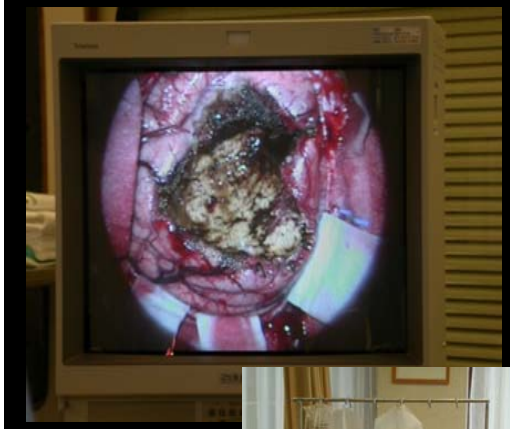

300
200
100
0
1 2 3 4 5 6 7 8 9 10 11 12 13 14 15 16 17 18 19 20 21 22
1. Belgium
2. The Netherlands
3. Germany
4. UK
5. USA
6. Italy
7. Sweden
8. Iceland
9. Poland
10. France
11. Denmark
12. Israel
13. Lebanon
14. Spain
15. Greece
16. Portugal
17. Switserland
18. Russia
19. Croatia
20. Kazachstan
21. Luxemburg
22. Mexico

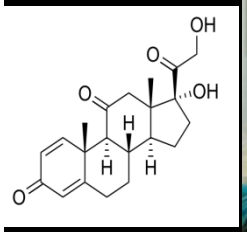

Number of HGG patients per year
120
100
80
60
40
20
0
0
2000
2002
2004
2006
2008
2010
year

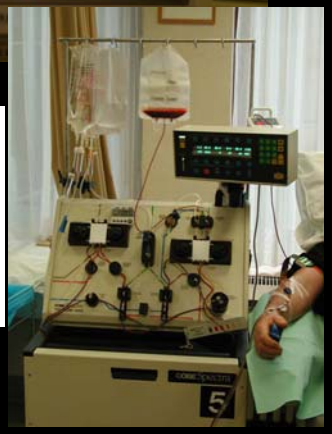
Accessibility in danger
New developments almost blocked

- Clinical trial
  - EudraCT application
  - Clinical Trial Center in hospital: financial dissection
  - Ethical committee
  - Federal Agency of Medicine and Health Products (FAMPH)
    - Tissue bank and GTP
    - IMP: full dossier
    - GMP
    - GCP

Regulations block easy application of a cell culture **procedure** with autologous material for other types of cancer, because the end product is considered as a drug.







Viability
Identity
Purity
Potency
Sterility

Medicinal product: Dir 2001/83/EC, Dir 2004/27/EC
ATMP: defined in Part VI Annex I to Dir 2001/83/EC
Investigational ATMP: Clinical trials Dir 2001/20/EC
GCP Dir 2005/28/EC; all IMP in GMP
Non-commercial trials; member state authorisation

Regulation 2007/1394/EC; Hospital exemption; implicit no clinical trial
Dir 2004/23/EC includes Clinical trial material; member state; GTP

New York Times: June 28; 2009
"Grant system undercuts major cancer advances"

This work is supported by

- **Olivia Hendrickx Research Fund**
- **Herman Memorial Research Fund**
- **James E. Kearney Memorial Fund**
- **Electrabel Netmanagement Vlaanderen**
- **CAF, Baxter, Schering-Plough**
- **Private families**
- **Service Clubs**
- **Belgian Federation Against Cancer**
- **Actie zware apparatuur Van Mechelen**
- **Emmanuel van der Schueren Fonds**
- **Fund for Scientific Research – Flanders**
- **IWT: TBM program**

Academia-driven research
Towards Evidence-based medicine

National health system

Genzyme

Governmental awareness on Rare Diseases :

- High awareness with a paradox between the need for therapy for these Rare Diseases and the reluctance because of their high costs
- Discussions for healthcare budget 2011 led to the setup of a budget enabling an accelerated access for orphan drugs (budget of 3,5 Mio – Royal Decree is expected)
- Implementation of a National Plan for Rare Diseases : phase 1 presented and approved by Minister of Social Affairs, some implementations presented during the Belgian Presidency (H2 2010), phase 2 implementation accepted end 2011

Orphan drug legislation:

- OD are perceived as very expensive and responsible for increase of budget
- Most OD are in fact related cancers and payers are concerned by the salami slicing strategy
- Currently, under class 1 procedure (no HE data needed) : provisional reimbursement with a revision within 12-36 months
- Specific reimbursement conditions (Chap IV) + college
- No tax to pay on turn-over as long as orphan drug status
- Future : ATU-system like to get earlier access (proposal in the national plan for Rare Diseases)

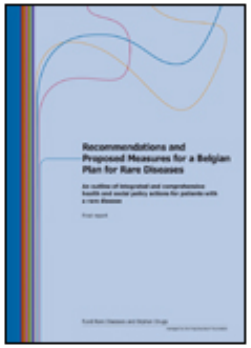
Early Temporary Reimbursement is one of 13 Recommendations

Publications

Home > Publications > Recommendations and Proposed Measures for a Belgian Plan for Rare Diseases...

Recommendations and Proposed Measures for a Belgian Plan for Rare Diseases - Final Report

An outline of integrated and comprehensive health and social policy actions for patients with a rare disease (2011)



- **Content**

In the framework of the 'Programme for the amelioration of the life quality of patients with a chronic disease', the Fund Rare Diseases and Orphan Drugs has been supported by the Minister of Social Affairs and PublicHealth, Ms. Laurette Onkelinx. The Fund, managed by the King Baudouin Foundation, got the specific assignment, through a contract with the Belgian National Institute for Health and Disability Insurance (RIZIV/INAMI), to delineate in close collaboration with the stakeholders recommendations and proposals for measures which could form the basis of a Belgian Plan for Rare Diseases.

A first set of recommendations and proposed measures were bundled together in an interim report - Phase I - and handed over to the Minister in May 2010.

The final report proposes a thought-out range of global and integrated actions in the healthcare management and the social policy for patients with a rare disease. Ultimately a comprehensive framework is aimed to improve the access and equity to prevention, diagnosis, treatment, information and rehabilitation for patients suffering from rare diseases.

The 42 recommendations and proposed measures in this report cover 5 main principles:

- expertise and multidisciplinarity
- collaboration and networking
- knowledge, information and awareness
- equity in access
- governance and sustainability

This final report was handed to the Minister of Social Affairs and Public Health, Ms. Laurette Onkelinx, in October 2011.

Author(s)

- Alain Denis - Pascale Gruber - Peter Raeymaekers

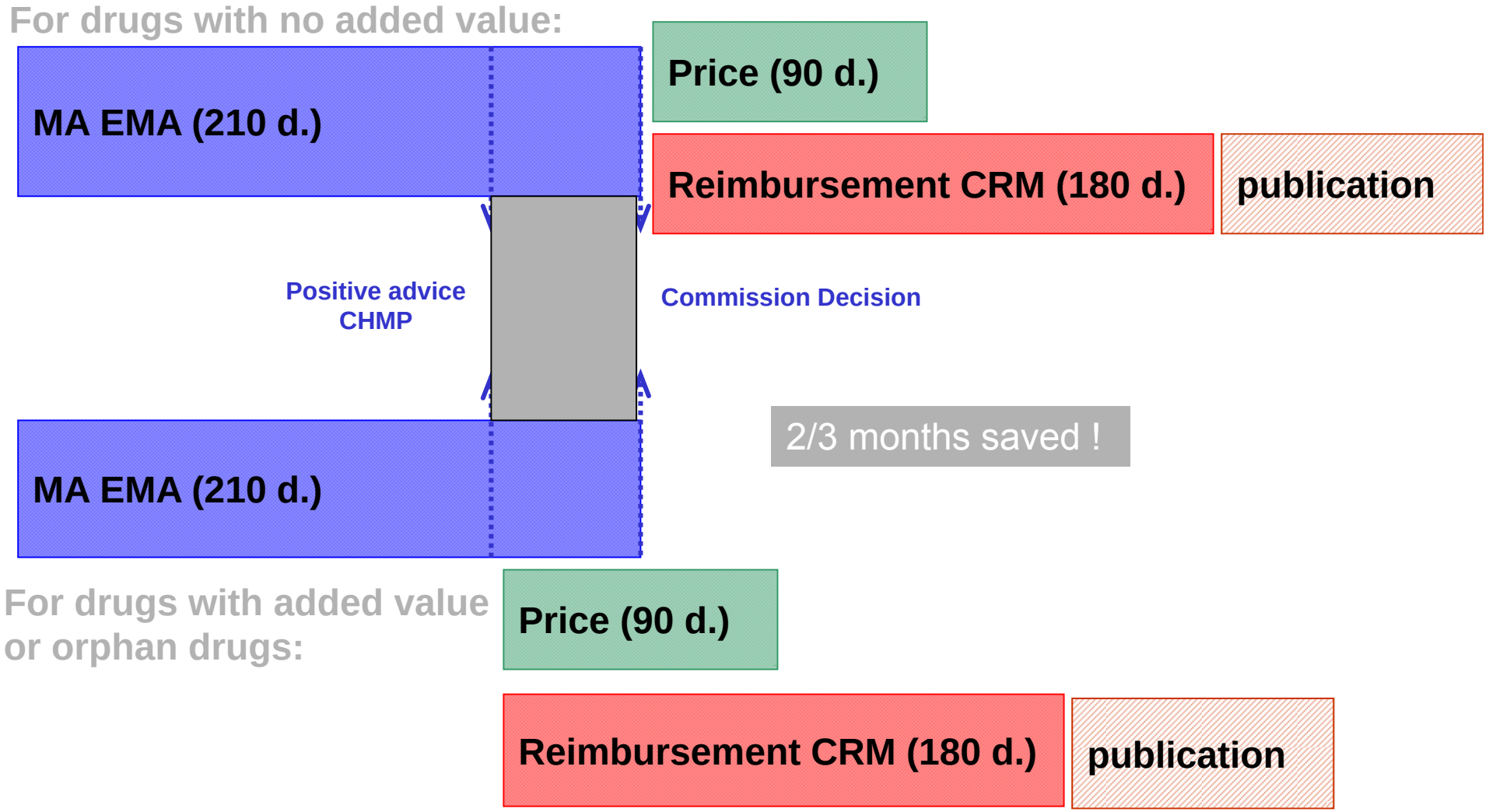
Practical details

- Availability: available
- Technical data: Electronic publication ; 2011 ; 188 p. ; A4 ; free ; from 21/10/2011

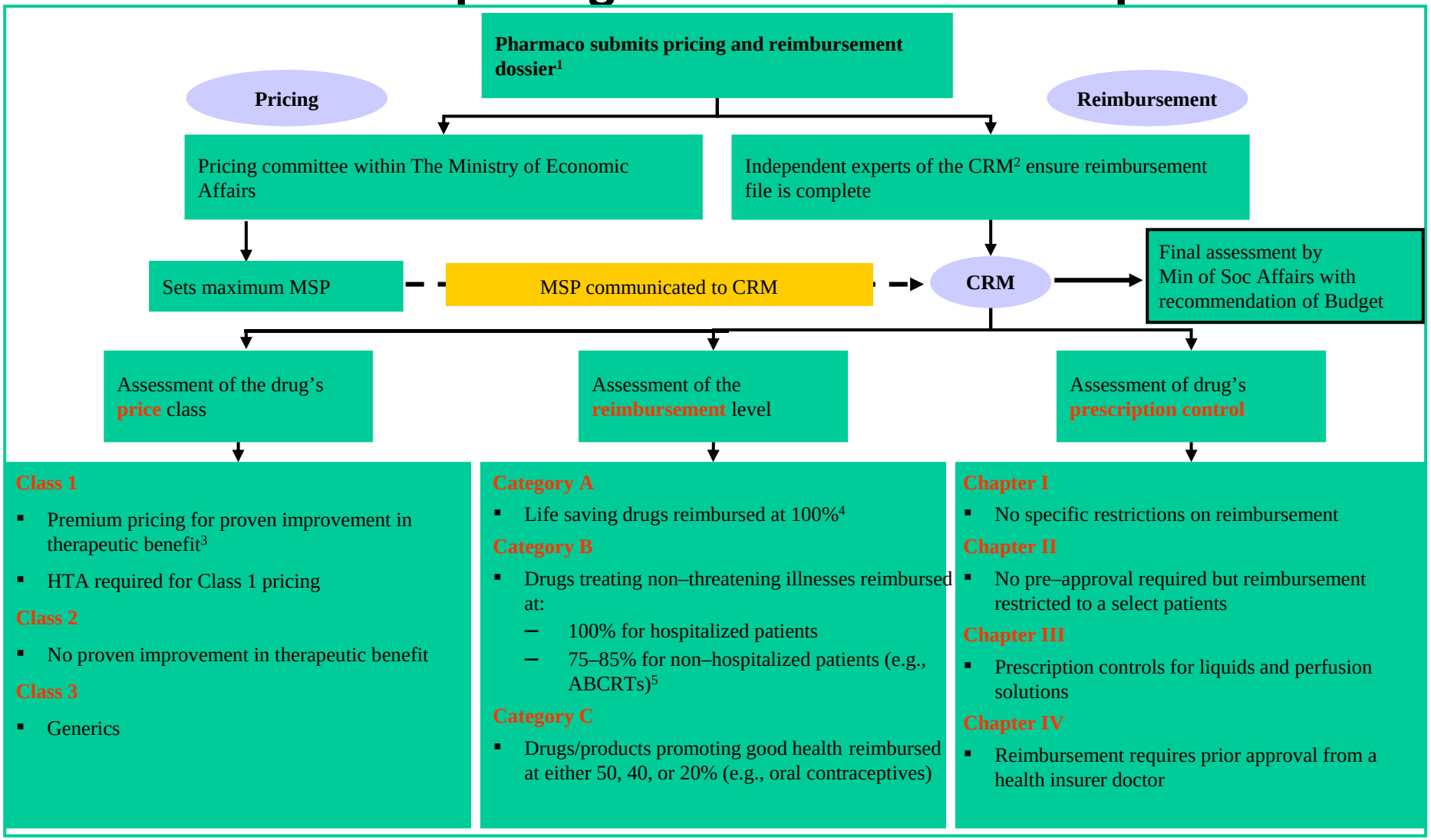
Genzyme

Genzyme

Pricing and reimbursement timeline in Belgium



Overview of the pricing and reimbursement procedure



1 Dossier submitted after receiving market authorization from AFMPS. Application to AFMPS can be filed concurrently with EMA submission
2 Part of the Ministry of Health
3 Defined as the degree of innovation of the drug
4 Patient co-pay applies
5 Tysabri officially reimbursed by state at 75% and any additional cost exceeding maximum patient co-pay of 10,80 euros
SOURCE: Espicom 2010; Pharma Handbook 2009; Obig report 2006; interviews

Prepared by McKinsey

Genzyme

Conclusion

- ATMP for orphan diseases
  - Most academic
  - Administrative hurdles inappropriate
- Medicinal products for orphan diseases
  - Slight improvement in the last two years