



TENDERS AND PROCUREMENT OF COAGULATION FACTOR CONCENTRATES : A EUROPEAN SURVEY OF 38 COUNTRIES

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Clinical involvement in alternative processes

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HAEMOPHILIA IN BELGIUM

- Haemophilia A 800*
- Haemophilia B 200*
- Medical doctors 40.000



Population : 10.600.000 inhabitants

FVIII annual consumption : 80 Millions units/year

* Association des Hémophiles AH-VH.

COST OF CLOTTING FACTOR CONCENTRATES TO TREAT PATIENTS WITH HAEMOPHILIA IN BELGIUM

Therapeutic agent	Cost in € 2002	Cost in € 2009
Factor VIII	27.839.925	61.670.978
Factor IX	2.139.053	5.883.035
Novoseven	3.054.971	2.583.762
FEIBA	150.084	570.127
Total	33.184.033 €	70.707.902 €

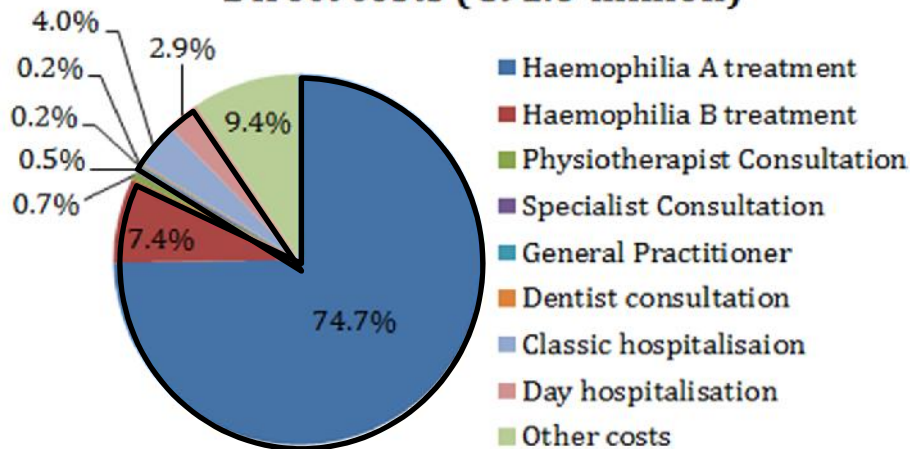
Estimated lifetime cost of haemophilia in Belgium

Total costs: €97.3 million (for 14 new cases)
 €7.8 million/incident case

94.3%

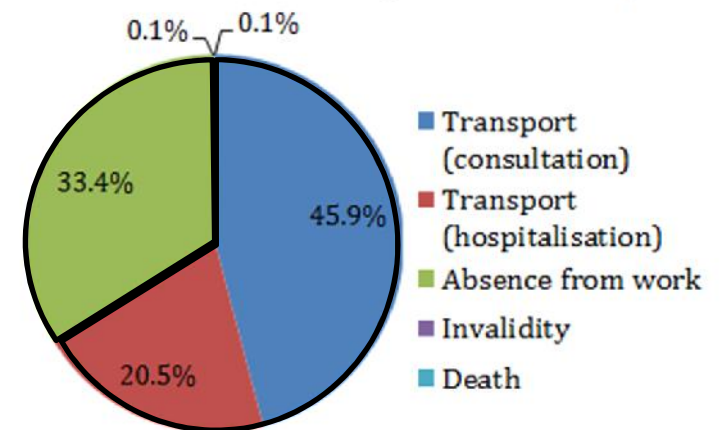
5.7%

Direct costs (€91.8 million)



> 4/5 Treatment
 7% Hospitalisations

Indirect costs (€5.6 million)



2/3 Transport
 1/3 Absence from work

Cost of concentrates available in Belgium

Concentrate	Name	Dosing	Cost
FVIII	Advate (Baxter)	250-500-1000-1500 units	253 -1481 euros
	Kogenate (Bayer)	250-500-1000 units	258 - 987 euros
	Refacto (Pfizer)	250-500-1000-2000 units	212 - 1631 euros
	Factane (CAF-DCF)	1000 units	894 euros
	Octanate (OctaPharma)	250-500-1000 units	223-867 euros
FIX	Benefix (Pfizer)	500-1000-2000 units	387-1521 euros
	Nonafact (CAF-DCF)	500-1000 units	273-537 euros
		500-1000 units	249-489 euros
	Octanine (Octapharma)		
FVIII-VWF	Wilate (octapharma)	450-900 units (FVIII)	420-832 euros
	Haemate-P (ZLB)	500-1000 units (FVIII)	273-537 euros
VWF	Wilfactin (LFB)	1000 units (VWF)	894 euros
FVIIa	NovoSeven	50-100-250 x 103	592-2967 euros
FII,VII,IX,Xa	FEIBA (Baxter)	250-500-1000 units	792 euros

The 10 European Principles of Hemophilia Care

1. A central hemophilia organisation with supporting local groups
2. National hemophilia patient registries
3. Comprehensive care centres and hemophilia treatment centres
4. Partnership in the delivery of hemophilia care
5. Safe and effective concentrates at optimum treatment levels
6. Home treatment and delivery
7. Prophylaxis treatment
8. Specialist services and emergency care
9. Management of inhibitors
10. Education and research



European principles of haemophilia care


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Summary. As the management of haemophilia is complex, it is essential that those with the disorder should have ready access to a range of services provided by a multidisciplinary team of specialists. haemophilia centres may also be necessary. There should be arrangements for the supply of safe clotting factor concentrates which can also be used in home treatment and prophylaxis programmes.

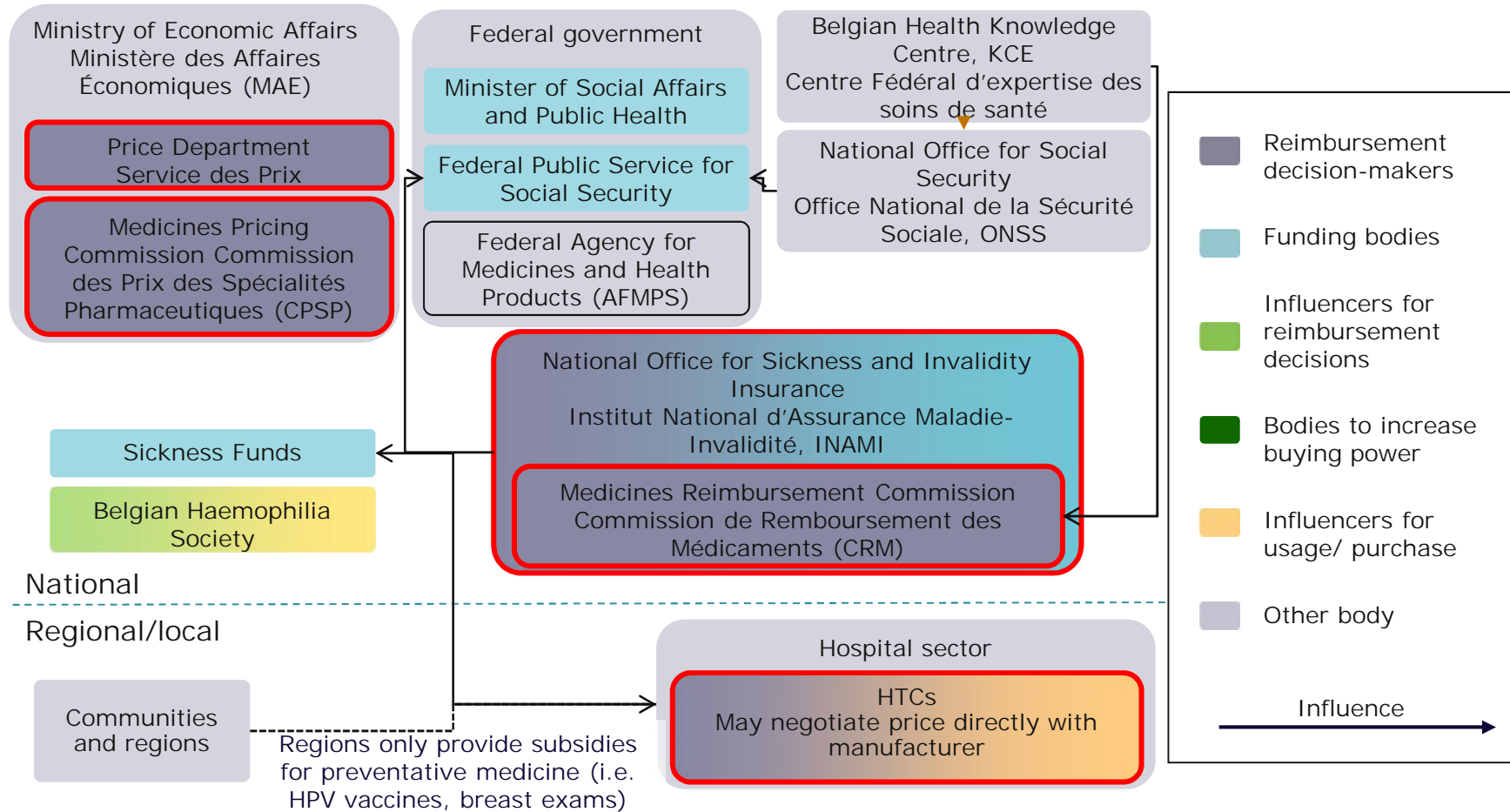


Summary of adherence to Principles 1,2 and 3 and 7 according to country

Country	No of Centres	Principle 1 Central Organisation	Principle 2 Patient Registry	Principle 3 All patients treated in CCC/HTC	No of CCC/HTC per Million inhabitants	Principle 7 % of Children on prophylaxis	Principle 7 % of Adults on prophylaxis
Belgium 	1	No	No	No	0.83	75-100	50-75
France	1	Yes	Yes	Yes	0.71	75-100	1-25
Germany	2	Yes	Yes	No	0.89	75-100	50-75
Greece	1	Yes	Yes	Yes	0.37	75-100	1-25
Italy	3	Yes	Yes	Yes	0.81	75-100	1-25
Netherlands	2	Yes	No	Yes	0.78	75-100	50-75
Norway	1	Yes	No	Yes	0.40	75-100	50-75
Poland	1	Yes	No	No	0.84	75-100	1-25
Portugal	1	No	No	No	3.77	75-100	1-25
Slovakia	1	Yes	Yes	Yes	7.78	75-100	1-25
Spain	3	No	Yes	Yes	0.91	75-100	1-25
Sweden	1	Yes	No	Yes	0.32	75-100	75-100
Switzerland	1	Yes	Yes	No	1.27	75-100	1-25
United Kingdom	2	Yes	Yes	Yes	1.06	75-100	50-75
	Total 21	79% Yes	57% Yes	64% Yes	Median 0.84 IQR0.62-1.11		



KEY STAKEHOLDER INTERACTIONS ON PRICING, MARKET ACCESS AND PRESCRIBING



HTC, Haemophilia Treatment Centre



Reimbursement of Medicines in Belgium

Federal Government

Marketing authorization	Minister of Public Health
Price setting	Minister of Economy
Reimbursement	Minister of Social affairs



COMMISSION OF MEDICINES REIMBURSEMENT

30 members

- **22 voting members :**
 - 7 academics
 - 8 insurers
 - 4 physicians association
 - 3 pharmacists association
- **8 non voting members :**
 - 4 representative Minister (Social affairs, Budget, Economy and Public Health)
 - 1 INAMI / RIZIV
 - 3 industry





PRINCIPLES - CRITERIA FOR EVALUATION AND ASSESSMENT

1. THERAPEUTIC VALUE –ADDED VALUE

= efficacy + safety + effectiveness + applicability + convenience
as determined by
MORBIDITY – MORTALITY – QUALITY OF LIFE

class 1 added value
class 2 comparable value
class 3 generics copies

1. PRICE and LEVEL OF REIMBURSEMENT

2. IMPORTANCE IN CLINICAL PRACTICE (social and/or therapeutic needs)

3. BUDGETARY IMPACT

4. ratio COST / THERAPEUTIC VALUE



PRICE AND LEVEL OF REIMBURSEMENT

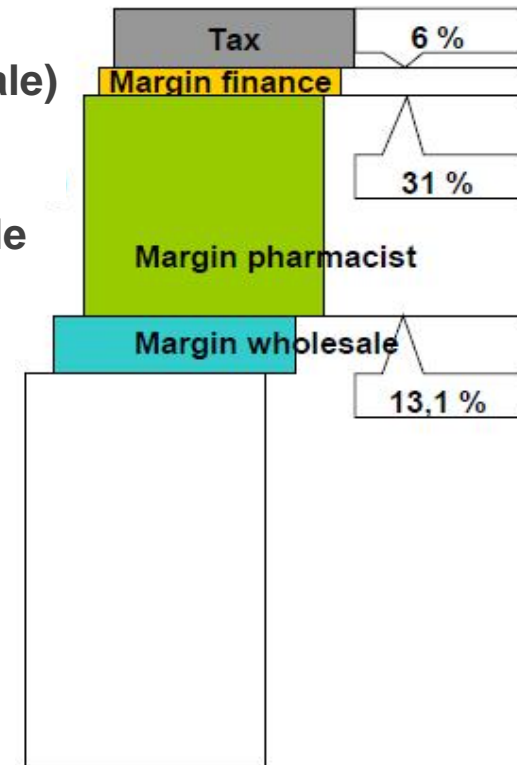
Public Price = Ex Factory Price + Margin Wholesale + Margin Pharmacist + (margin finance) + Tax

Margin Wholesale = 13,1% of (Ex Factory price + margin wholesale)
= « ceiling » (maximum) at 2,18 €

Margin Pharmacist = 31% of (Ex Factory price + margin wholesale + Margin pharmacist)
= « ceiling » (maximum) at 7,44 €

Margin finance = « extra » (pre-financing) margin for pharmacist and wholesale for « expensive drugs »
= 3% of Ex Factory price minus 0,4314 € (if the ex factory price is higher then 29,35 €)
= 3,38% of Ex Factory price minus 0,48604 € (if the ex factory price is higher then 53,36 €)

Tax = 6% on (Ex Factory price + Margin Wholesale + Margin Pharmacist + margin finance)



EUROPEAN STRATEGIES TO OPTIMISE HAEMOPHILIA FUNDING

Strategy	Country	Principle	Aim
National Tenders for Coagulation Factors	UK Ireland	Create competition between concentrates Involvement of all partners in a rigorous decision-making process: patients, doctors, health authorities,...	Ensure safe reliable supply of FVIII Manage usage of clotting factors (consumption per centre) Reduce product prices by leveraging economies of scale Impact on physicians and patients depends upon the aim and methods of the tender
Haemophilia Budget Capitation	Germany	Fixed reimbursement per patient e.g., tariff of €60,000 per patient per year	Reduction of costs
Centralisation of Haemophilia Care	UK Spain	Limited number of specialised centres (2–3 per country) Care provided exclusively in these centres	Better control of FVIII consumption / use Tender business to reduce cost of concentrates
Health Technology Assessment	Sweden	To assess the clinical and cost effectiveness of pharmaceutical products	To decide which products will be reimbursed by society Will the Swedish HTA set the precedent for future reviews across Europe?

BELGIAN MEDICAL SURREALISM





The ambiguity and paradox of haemophilia care in Belgium

- Availability of plasma-derived and recombinant FVIII or FIX concentrates
 - No prescription restriction
 - No limitations of use of concentrates
 - Home treatment and prophylaxis widely available
 - Cost per FVIII unit : 0.7-0.9 Euro

- Process of setting official treatment centres just initiated
- Limited funding for haemophilia care (excluding replacement)
- No national registry
- No formal national certification
- No auditing



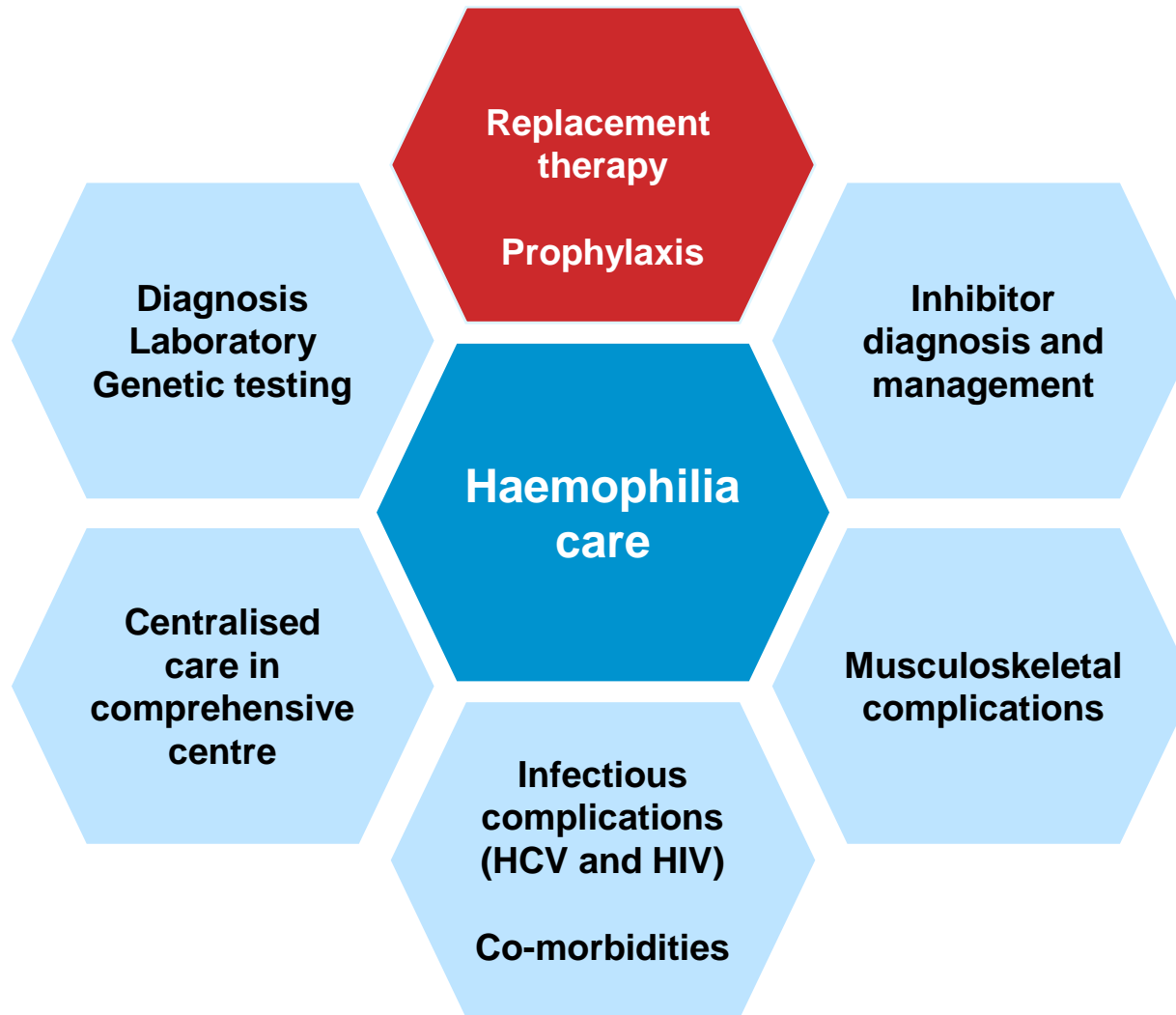
More on Haemophilia care in Belgium

- F8 and F9 concentrates can be prescribed by GPs
- Patients can select the hospital of their choice, even if not hosting an HTC
- Immunotolerance can be initiated in hospitals with no HCCC
- Vague definition of haemophilia specialists

Negative aspects of haemophilia funding in Belgium



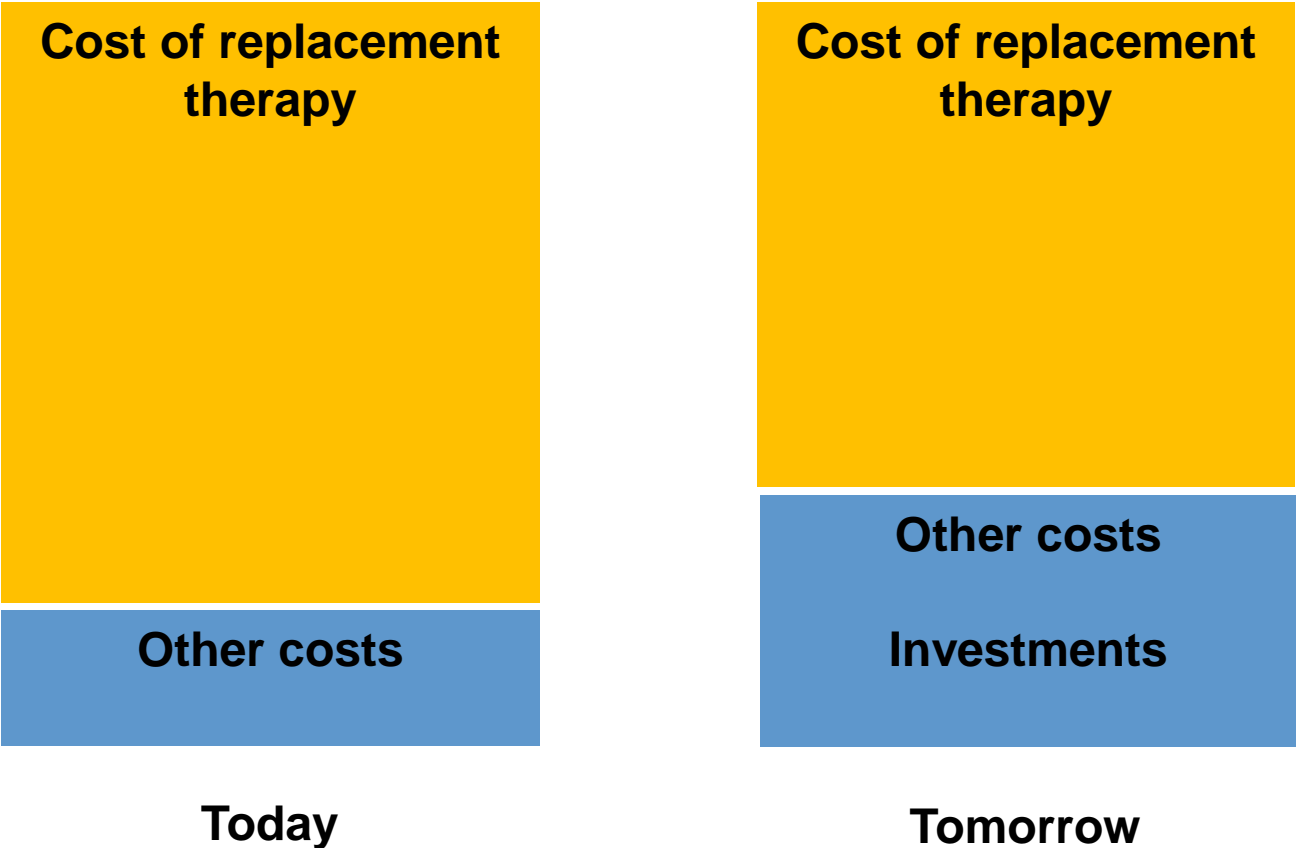
- ☹️ Public price of F8 is high - close to 0.8-0.9 euros/FVIII unit
- ☹️ 90 % of haemophilia funding for replacement therapy
- ☹️ No initiative (that I know) to reduce the price
- ☹️ Limited funding for haemophilia centers (1500 euros expected per severe patient)
- ☹️ No national registry and no comprehensive view of concentrates prescription and use
- ☹️ No reliable tracing of concentrates use
- ☹️ Prescribing policy heterogenous and not driven by economical constraints



Haemophilia Multidisciplinary Team

- **Adult haemophilia specialist**
- **Paediatric haemophilia specialist**
- **Haemostasis laboratory**
- **Physiotherapist**
- **Liver diseases specialist**
- **Infectious diseases specialist**
- **Orthopaedic surgeons**
- **Geneticist**
- **Psychologist**
- **Social worker**
- **Clinical research unit**
- **Patients association (AHVH / WFH)**
- **Pharmacy**
- **Pharmaceutical companies**

Rebalancing cost of haemophilia care



How to invest in a more sustainable haemophilia care beyond replacement therapy

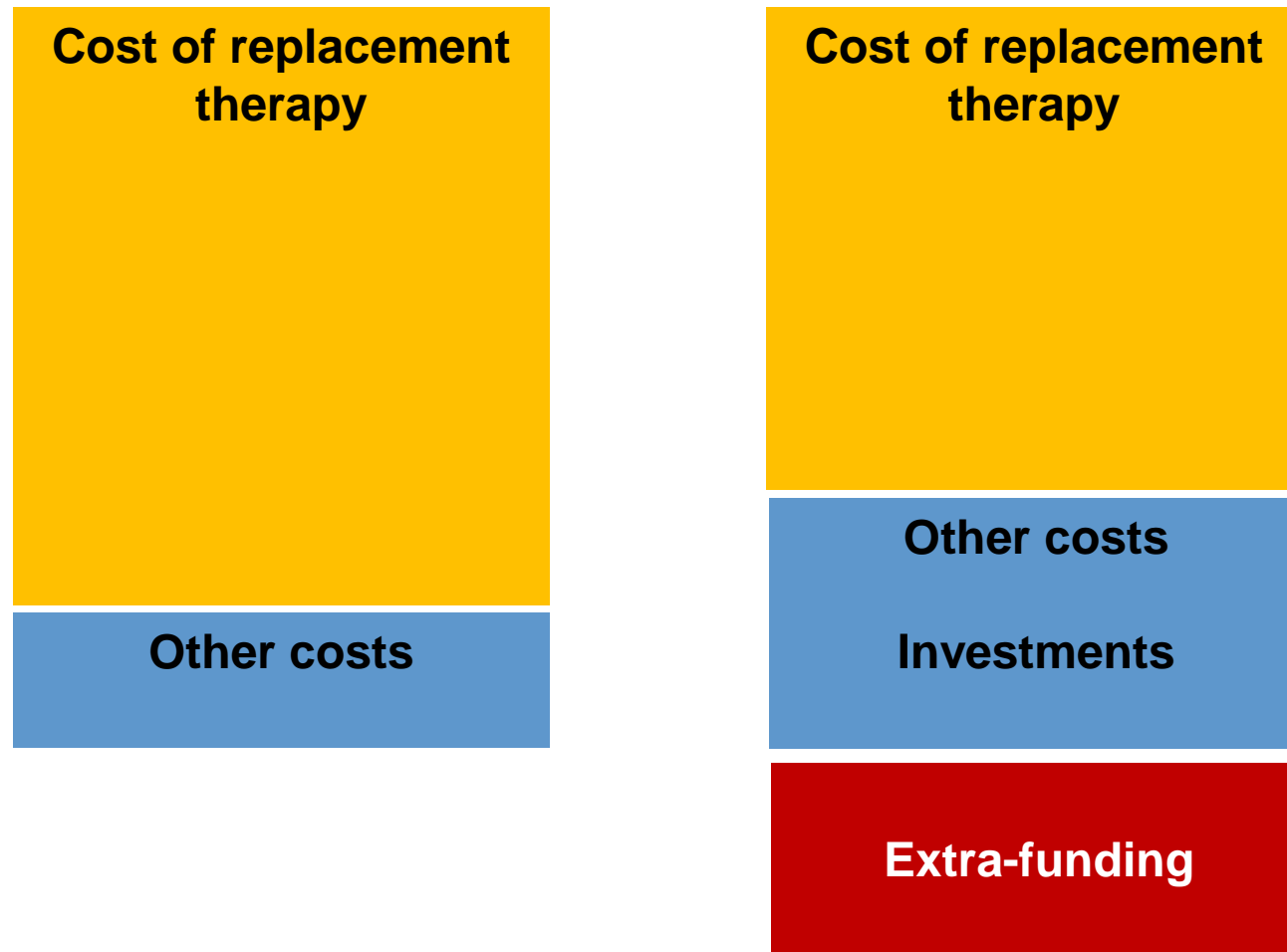
- National interactive registry
- Traceability of concentrates
- Standardised clinical follow-up
- Standardised outcome collection

- Patients education to new concepts - modalities of haemophilia care (PK, individualised therapy, optimised replacement according to lifestyle, recognition of bleeds)

- Management, care and prevention of co-morbidities
- Treatment of all HCV+ patients with new treatments

- Promotion of social and professional insertion
- Psychological follow-up,...
- ...

Rebalancing cost of haemophilia care



Concluding remarks

- Access and reimbursement of concentrates are critical for haemophilia care
- Cost of concentrates should not impact negatively on the multidisciplinary and complex care of haemophilia and implementation of new treatment options
- Validity of current cost estimate of replacement therapy should be revisited (cost per unit versus cost of annual-treatment)
- Financing of haemophilia should be cost-effectively rebalanced between replacement therapy and global care
- Global modalities of haemophilia funding should ideally be discussed and agreed between all stakeholders, including treaters, patients' association.