

Affordability of Global Bleeding Disorders Treatment



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The High Price of Hemophilia - ASH Clinical News

1 Feb 2020 — The monthly per-member spending for antihemophilic **factor** increased by 62% over 12 months, from \$1.03 to \$1.66; for Medicare, that spending ...

Brian O Mahony

WFH Global Forum, Montreal

November 2023

FIGURE G4a. Mean per capita factor VIII use in 2020 – regional and GNI comparisons of IU/total population: Europe

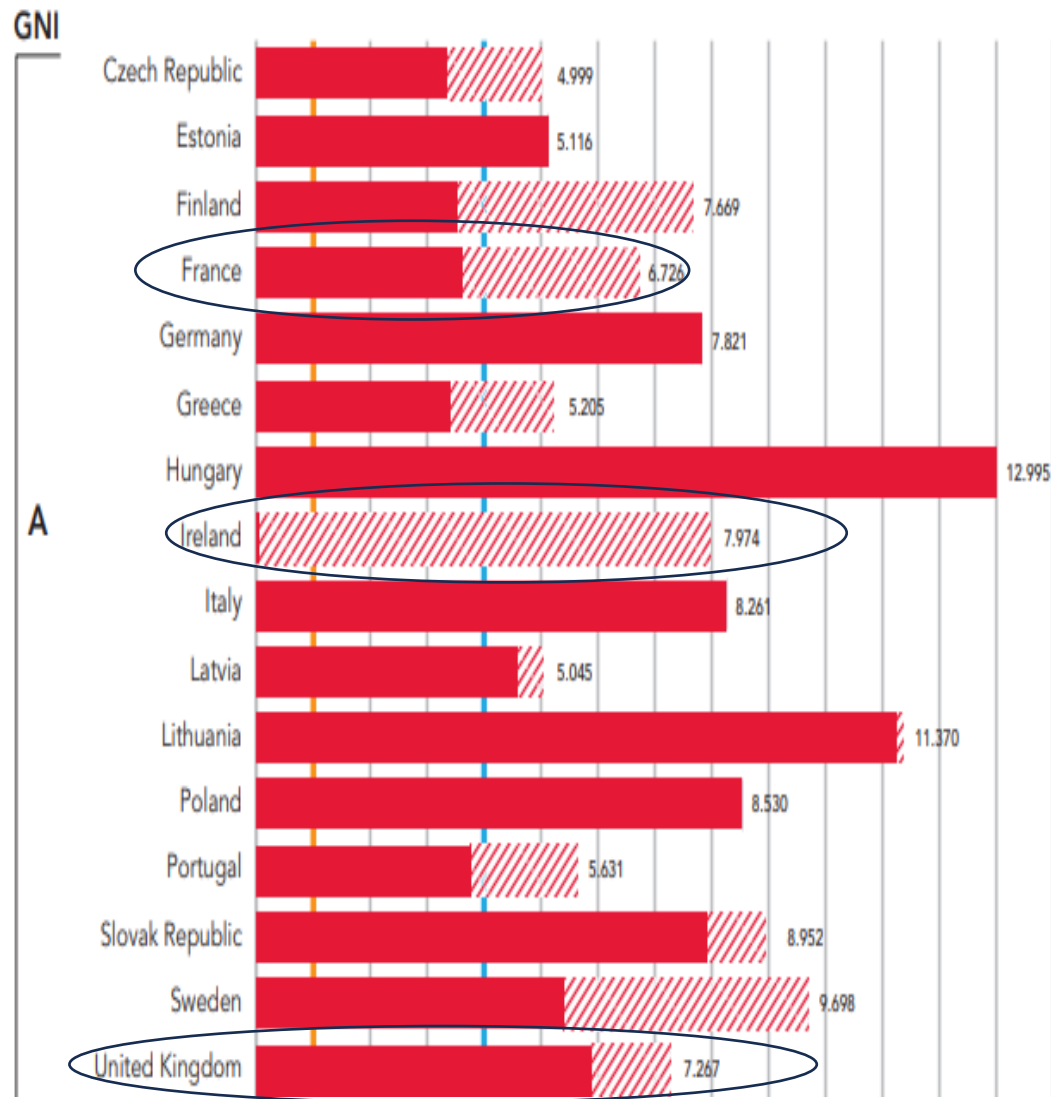
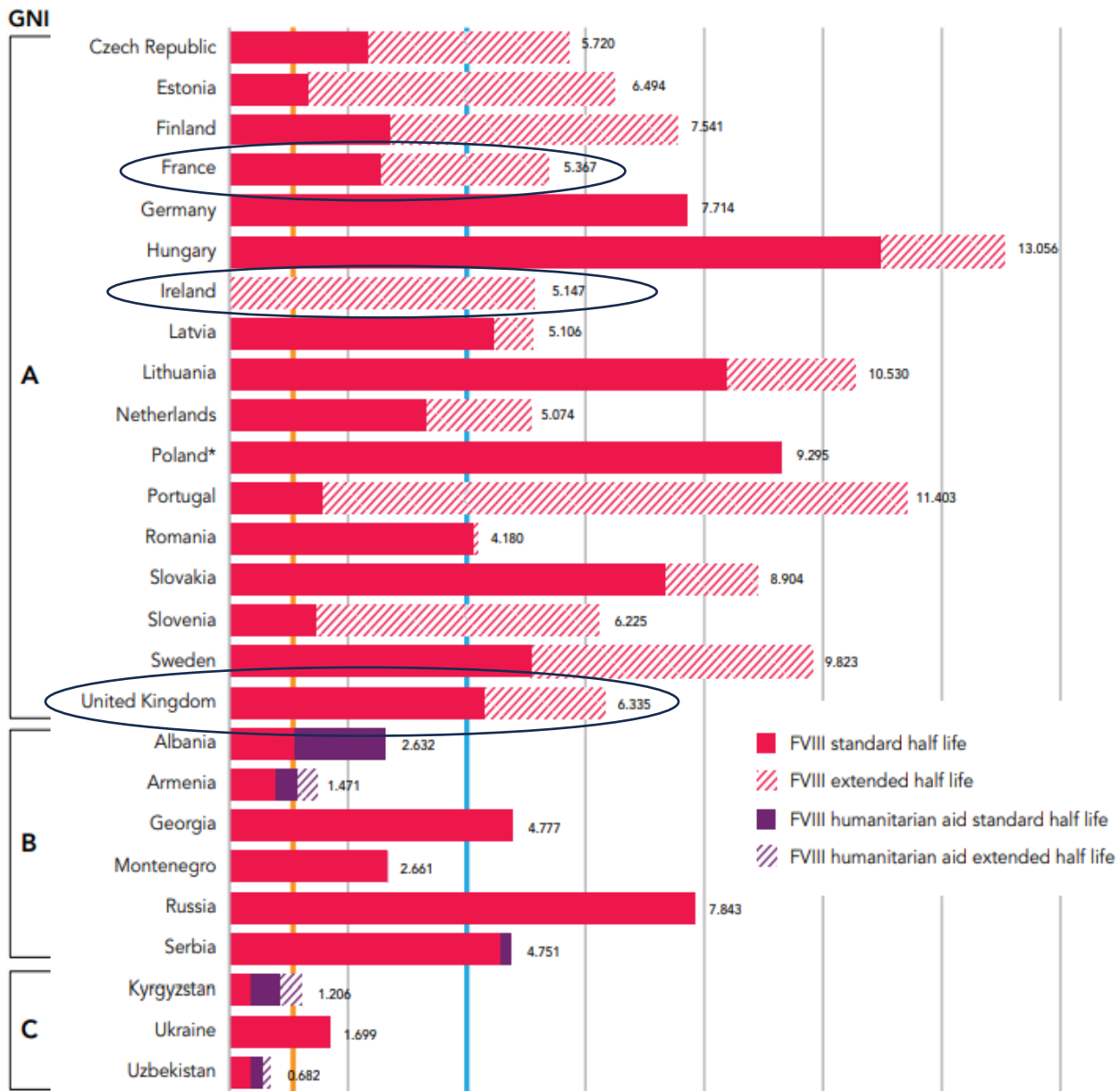


FIGURE G4a. Mean per capita factor VIII use in 2021 – regional and GNI comparisons of IU/total population: Europe



Affordability - Prices



- Very difficult to get real information
- Most countries do not publish their prices
- **List prices, when published, often bear little reality to actual prices charged**
- In some countries, centres purchase at one price and make available at a higher cost to fund comprehensive case: USA, Netherlands
- Most countries no longer report prices to the WFH Annual Survey
- Emicizumab prices often separate and under separate mechanisms

Australia - No Longer Publish Prices



Demand for factor VIII

Decreased by 13.5% from 2019-20

→ Mostly due to the introduction of Hemlibra

- Recombinant VIII decreased by 13.2%
- Plasma derived FVIII decreased by 15.7%



Demand for factor IX

Increased by 0.5%

- Recombinant FIX increased by 0.5%
- Plasma derived FIX decreased by 2.5%

Expenditure on clotting factors per year and percentage of total blood budget 2009-10 to 2019-20

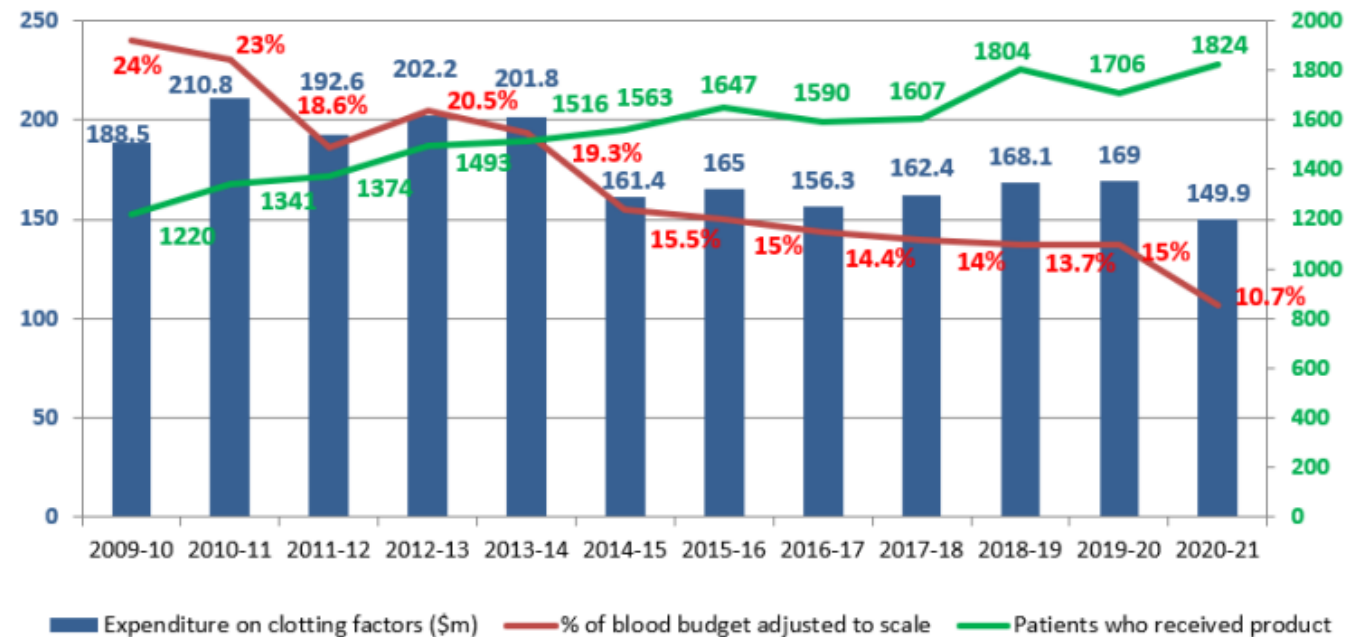


FIGURE 7 - EXPENDITURE ON CLOTTING FACTORS AND PERCENTAGE OF BLOOD BUDGET 2009-10 TO 2020-21

Note: 2020-21 includes Hemlibra, a monoclonal antibody product.

USA: Average Wholesale Prices per IU 2023

FVIII

- Advate €1.75
- Xyntha €1.63
- Kovaltry €2.19
- Adynovate €2.27
- Eloctate €2.36*
- Altuviiiio €4.85*
- Jivi €2.50

- Roctavian € 2.755 million/dose

FIX

- Benefix € 1.53
- Ixinity € 1.88
- Rixubis €1.66
- Alprolix €3.42
- Idelvion € 4.73

- Hemgenix €3.325 million/dose

To ensure that patients have access to the improved bleed protection provided by ALTUVIIIIO, Sanofi will price ALTUVIIIIO at parity to the annual cost of treating a prophylaxis patient on Eloctate® [Antihemophilic Factor (Recombinant), Fc Fusion Protein]. Sanofi will also provide comprehensive patient support services and resources online and at 1.855.MyALTUVIIIIO

US Comparator for Cost of CFC, Emicizumab, Gene Therapy: ICER Report, 2022

Drug	Price per Dose*	Discount from WAC*	Net Price per Year
Valoctocogene Roxaparvovec	\$2,500,000	N/A	N/A
Emicizumab	\$25,706	12% [†]	\$639,543 [†]
Drug	Price per Dose	Discount Relative to Net	Net Price per Year*
Etranacogene Dezaparvovec	\$4,000,000	N/A	N/A
Alprolix	\$13,716	N/A	\$744,303
Benefix	\$5,307	N/A	\$565,391
Idelvion	\$13,419	N/A	\$753,353
Rebinyn	\$13,675	N/A	\$713,552

Affordability

CSL lands FDA approval for hemophilia B gene therapy, sets \$3.5M list price

by Amber Tong on November 23rd, 2022



The FDA has approved the world's first gene therapy for hemophilia B, ushering into the market a treatment that's historic in both what it promises to do and how much it will cost.

- USA: Launch price of Hemgenix \$3.5 million
 - : Launch price of Roctavian \$ 2.5 million
- Current annual cost of prophylactic treatment in USA:
 - : FIX \$715,000-\$ 750,000, FVIII/ Emicizumab \$500,000- \$650,000
- Launch prices in USA equivalent to 5 years current treatment cost
- *“Value should not be determined exclusively by long term cost offsets- current standard of care priced excessively high”**
- *“US pricing for CFC and Emicizumab represent a failure of competition”**
- List price of Roctavian in Germany € 1.5 million – 5 years treatment cost
- List price of Hemgenix in UK £ 2.6 million
 - : Discounted price submitted for economic review

Netherlands

- Very high list prices.
: Actual price up to 80% lower
- Centres get low price and add on an amount- similar to US 340B

- “ List” Prices:
- **Idelvion €7.52/IU**
- Alprolix €2.21/IU
- Elocta € 1.04/IU
- Hemlibra € 82.53/mg

🔗 Idelvion injpdr fl 1000ie + solv 2,5ml +toebeh

Albutrepenonacog alfa, Csl behring bv

🏠 €7,521.00 per piece (Refunded)

🔗 Alprolix injpdr 1000ie + solvens 5ml + accessories

Eftrenonacog alfa, Swedish orphan biovitrum (the netherlands) b.v.

🏠 €2,215.59 per piece (Refunded)

🔗 Elocta injpdr fl 1000ie + solv 3ml + toebeh

Efmoroctocog alfa, Swedish orphan biovitrum (the netherlands) b.v.

🏠 €1,039.61 per piece (Refunded)

🔗 Adynovi injpdr fl 1000ie + solv 2ml+syst

Rurioctocog alfa pegol, Bmodesto b.v., Austria

🏠 €1,039.59 per piece (Refunded)

🔗 Hemlibra injvlst 30mg/1ml (30mg/ml) vial

Emicizumab, Orifarm b.v., Germany

🏠 €2,475.85 per piece (Refunded)

Belgium

- Official List prices – not actual prices paid

- Advate €0.61
- Adynovi €0.79
- Jivi €0.74
- Benefix € 0.60
- Alprolix €1.20
- Idelvion € 1.87
- Hemlibra € 73.93/mg

	Statut	Code	Idée prix par flacon	Quantité
Advate 250 Unités/2 ml	H	0032699	154 €	
Advate 500 Unités/2 ml	H	0032731	301 €	
Advate 1.000 Unités/2 ml	H	0032665	594 €	
Advate 1.500 Unités/2 ml	H	0032673	967 €	
Advate 2.000 Unités/5 ml	H	0032681	1224 €	
Advate 3.000 Unités/5 ml	H	0032723	1885 €	
Adynovi 500 unités/2 ml	H	0032475	429 €	
Adynovi 1000 unités/2 ml	F	0032442	845 €	
Adynovi 2000 unités/5 ml	F	0032459	1572 €	
Alprolix 250 Unités/5 ml	H	0070863	299 €	
Alprolix 500 Unités/5 ml	U	0070889	590 €	
Alprolix 1000 Unités/5 ml	U	0070848	1173 €	

France: Haemophilia A

- SHL FVIII €0.25-€0.30/IU
- EHL FVIII €0.55/IU

Emicizumab € 77.10/mg (list)

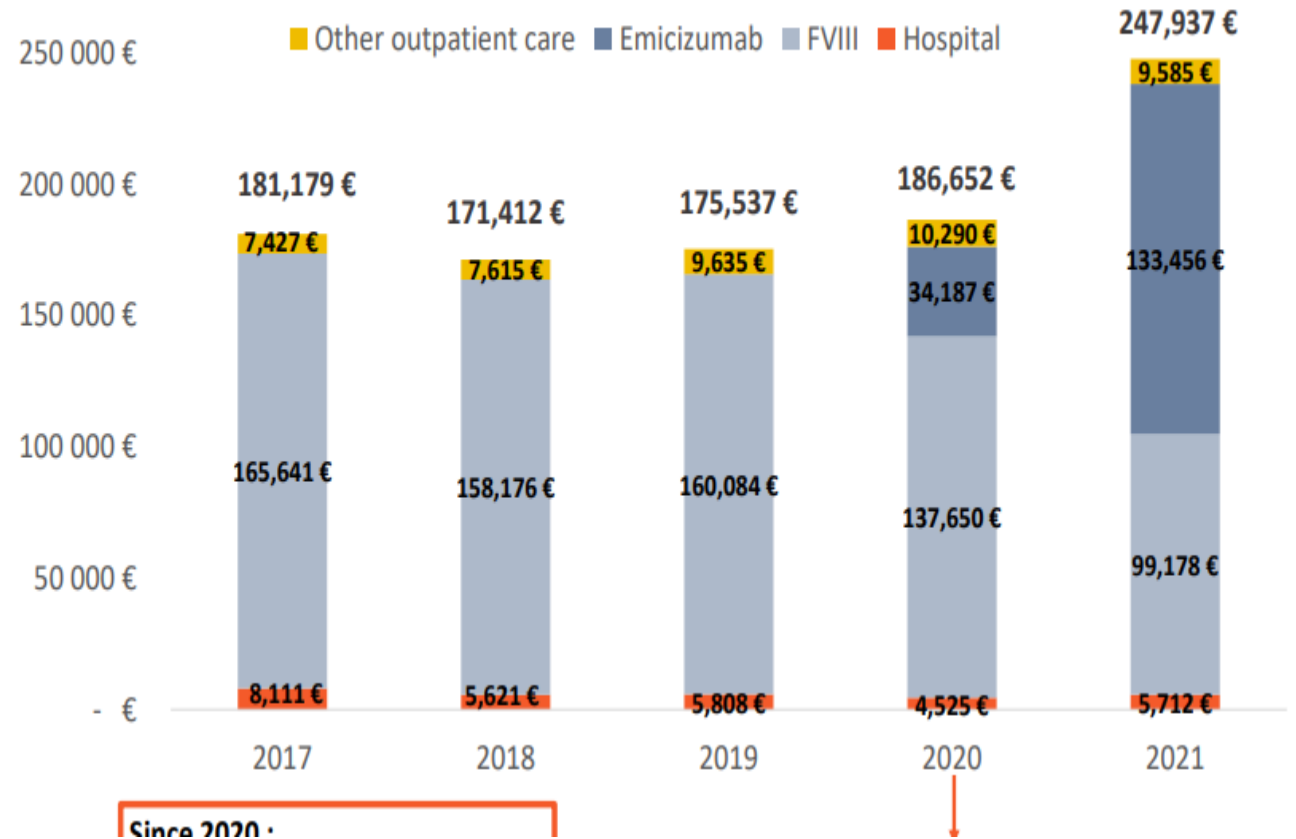
Actual cost linked to cost of EHL prophylaxis

Mean FVIII Prophylaxis/ year

€ 247,937*

* Includes ambulatory care and hospital costs

FIGURE 3: Evolution of costs between 2017 and 2021 (without inhibitor – prophylaxis)



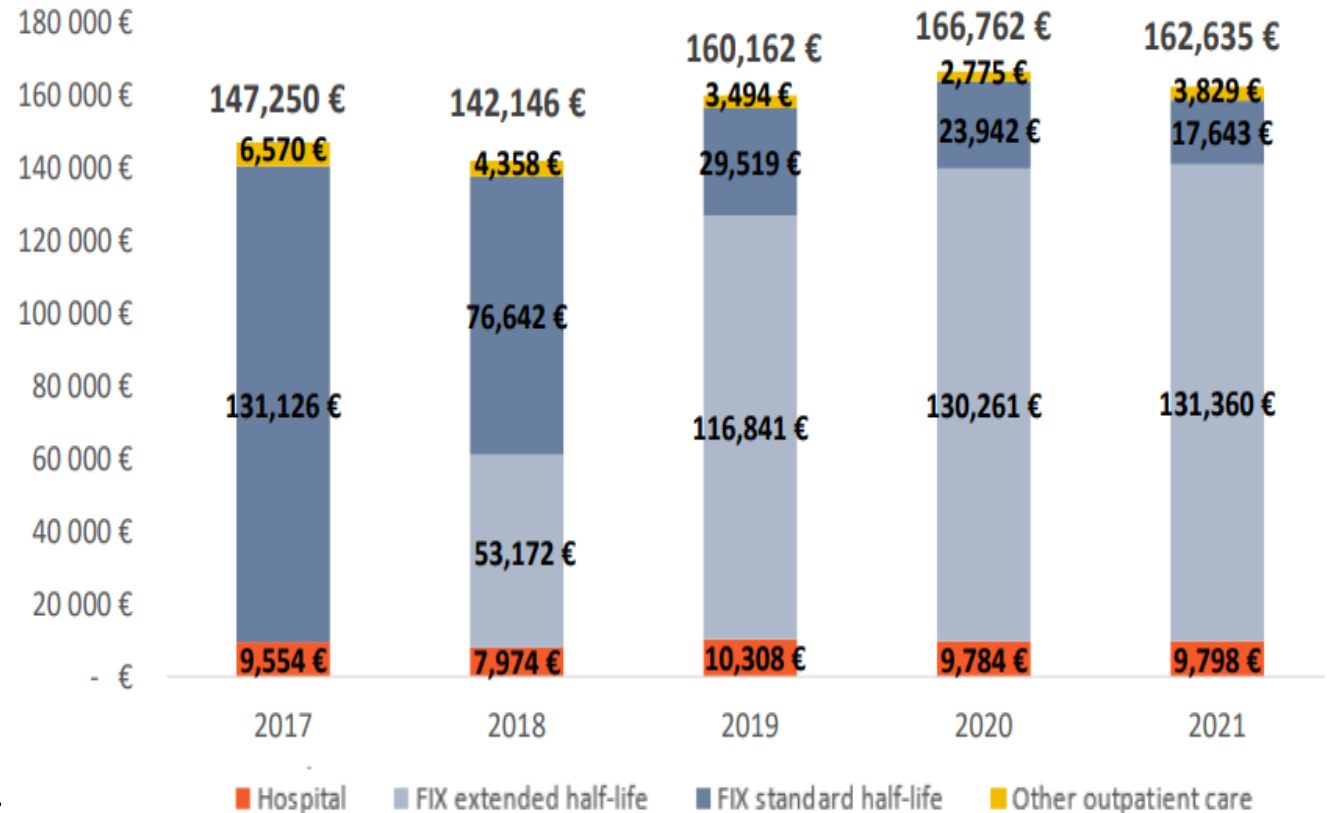
France Haemophilia B

- Idelvion €1.71/IU
- Alprolix € 0.86/IU
- SHL recombinant FIX €0.45/IU

- Mean annual cost prophylaxis
- € 162,635*

*Includes ambulatory care and hospital costs

FIGURE 3: Evolution of costs between 2017 and 2021 (without inhibitor – prophylaxis)



Five-Year Costs/Person with Haemophilia A: List Prices - Not Actual Prices Paid

Product	EU country	Emicizumab (€/mg) <i>product list price</i>	5-yr cost /person (€)
Emicizumab Loading dose: 3 mg/kg weekly for 4 weeks followed by 1.5 mg/kg once weekly <i>(maintenance treatment)</i>	UK	80.51	2.532.404
	France	77.13	2.420.730
	Italy	55.07	1.734.174
	Spain	76.70	2.404.307
	Germany	64.66	2.048.088
rFactor VIII Fc 85.4 IU/kg/weekly	UK	0.89	1.500.408
	France	0.65	1.095.435
	Italy	0.65	1.095.435
	Spain	0.59	994.318
	Germany	0.53	893.201

* ≥12 years of age

EMI: emicizumab; HA: haemophilia A; rFVIII: recombinant factor VIII

Di Minno G. and Castaman GC.; calculations based on: Mancuso ME et al J. Med. Economics. 2022;25(1):1068-75

Poland (High Income Country)- Prices per IU

- Emoclot €0.04
- Fandhi € 0.05
- Immunate € 0.06
- Octanate €0.09
- Wilate €0.08
- Pharmavate € 0.04
- Advate €0.10
- Novoeight €0.08
- Immunine € 0.08
- Octanine €0.07
- Benefix € 0.26
- Hemlibra €53.78/mg

South Africa: Public Tender Prices to End 2024

- Public and Private healthcare prices can differ
- Haemosolvate € 0.17
- Haemosolvex FIX € 0.20
- Kovaltry € 0.38

NAPPI CODE	NBI CODE	PRODUCT	VOL	SINGLE EXIT PRICE (VAT EXCL)	SINGLE EXIT PRICE (VAT INCL)
820342009	10349	Bioplasma FDP	200 ml	R1,312.46	R1,509.32
854522018	10345	Bioplasma FDP	50 ml	R 328.11	R 377.32
641560005	10351	Haemosolvate® Factor VIII 300 IU	10 ml	R 1,084.14	R1,246.76
600759028	10352	Haemosolvate® Factor VIII 500 IU	10 ml	R 1,705.46	R1,961.28
813648009	10341	Haemosolvate® Factor VIII 500 IU: 1000 IU pack	2 x 10 ml	R 3,410.92	R3,922.56
800767004	10390	Haemosolvex® Factor IX (500 IU)	10 ml	R2,002.65	R2,303.04

Item No	Item Specification	Registered Product Name	Delivered Price in ZAR	Pack Size Offered: Unit Pack
7	RECOMBINANT ANTIHAEMOPHILIC FACTOR VIII, 1000 IU INJECTION, STANDARD HALF-LIFE, 1 DOSE VIAL PLUS DILUENT	Kovaltry 1000	R5 836.25	1 x 1 Dose Vial plus Diluent
8	RECOMBINANT ANTIHAEMOPHILIC FACTOR VIII, 250 IU INJECTION, STANDARD HALF-LIFE, 1 DOSE VIAL PLUS DILUENT	Kovaltry 250	R1 459.06	1 x 1 Dose Vial plus Diluent
9	RECOMBINANT ANTIHAEMOPHILIC FACTOR VIII, 500 IU INJECTION, STANDARD HALF-LIFE, 1 DOSE VIAL PLUS DILUENT	Kovaltry 500	R2 918.13	1 x 1 Dose Vial plus Diluent

		Registered Product Name	Delivered Price in ZAR	Pack Size Offered: Unit Pack
4	HUMAN COAGULATION CONCENTRATE COMPLEX: FACTOR VIII COMPLEX 300 IU INJECTION, UNIT CONTAINING - A HIGH SPECIFIC FACTOR VIII (FACTOR VIII: C) ACTIVITY - VON WILLEBRAND FACTOR (FACTOR VIII: VWF) ACTIVITY - VON WILLEBRAND FACTOR ACTIVITY: FACTOR VIII ACTIVITY - 1:1 KIT FOR RECONSTITUTION AND INJECTION	Haemosolvate Factor VIII 300 IU	R1 183.67	1 x 1
5	HUMAN COAGULATION CONCENTRATE COMPLEX: FACTOR VIII COMPLEX 500 IU INJECTION, UNIT CONTAINING - A HIGH SPECIFIC FACTOR VIII (FACTOR VIII: C) ACTIVITY - VON WILLEBRAND FACTOR (FACTOR VIII: VWF) ACTIVITY - VON WILLEBRAND FACTOR ACTIVITY: FACTOR VIII ACTIVITY - 1:1 KIT FOR RECONSTITUTION AND INJECTION	Haemosolvate Factor VIII 500 IU	R1 757.73	1 x 1
6	HUMAN COAGULATION CONCENTRATE COMPLEX: FACTOR VIII COMPLEX 1 000 IU INJECTION, UNIT CONTAINING - A HIGH SPECIFIC FACTOR VIII (FACTOR VIII: C) ACTIVITY - VON WILLEBRAND FACTOR (FACTOR VIII: VWF) ACTIVITY - VON WILLEBRAND FACTOR ACTIVITY: FACTOR VIII ACTIVITY - 1:1 KIT FOR RECONSTITUTION AND INJECTION	Haemosolvate Factor VIII 500 IU (2 x 500 IU pack)	R3 515.48	1 x 1
11	HUMAN COAGULATION CONCENTRATE COMPLEX: FACTOR VIII COMPLEX 500 IU INJECTION, UNIT CONTAINING - A HIGH SPECIFIC FACTOR VIII (FACTOR VIII: C) ACTIVITY - VON WILLEBRAND FACTOR (FACTOR VIII: VWF) ACTIVITY - VON WILLEBRAND FACTOR ACTIVITY: FACTOR VIII ACTIVITY - 1:1 KIT FOR RECONSTITUTION AND INJECTION	Haemosolvate Factor VIII 500 IU	R1 757.73	1 x 1
12	HUMAN COAGULATION CONCENTRATE COMPLEX: FACTOR VIII COMPLEX 1 000 IU INJECTION, UNIT CONTAINING - A HIGH SPECIFIC FACTOR VIII (FACTOR VIII: C) ACTIVITY - VON WILLEBRAND FACTOR (FACTOR VIII: VWF) ACTIVITY - VON WILLEBRAND FACTOR ACTIVITY: FACTOR VIII ACTIVITY - 1:1 KIT FOR RECONSTITUTION AND INJECTION	Haemosolvate Factor VIII 500 IU (2 x 500 IU pack)	R3 515.48	1 x 1
15	HUMAN COAGULATION FACTOR CONCENTRATE: FACTOR IX COMPLEX, 500 IU INJECTION, UNIT CONTAINING: - CONCENTRATE OF COAGULATION FACTORS II (PROTHROMBIN), VII (PROCONVERTIN), IX (CHRISTMAS FACTOR) AND X (STUART-PROWER FACTOR)	Haemosolvex Factor IX	R2 186.50	1 x 1

HP10-2021BIO: SUPPLY AND DELIVERY OF BIOLOGICAL PREPARATION <https://www.health.gov.za/wp-content/uploads/2021/03/HP10-2021BIO-Contract-Circular-03-Nov-2020.pdf>

Pricing Data 2022

- 118 countries reported data to WFH Annual Global Survey in 2021
- Data on prices available from only 12 countries
- Data on price of Emicizumab available from only 13 countries

- Price data on CFCs obtained from an additional 14 countries
- Price data on Emicizumab from an additional 7 countries

- Total: Data on CFC prices from 26 countries
: Data on Emicizumab prices from 20 countries

Country Classification by Income

Group	July 1, 2021 (new)
Low income	< 1,045
Lower-middle income	1,046 - 4,095
Upper-middle income	4,096 - 12,695
High income	> 12,695

Country data or partial data on :

- 17 High income (HIC)
 - : 11 in the European Union
- 7 Upper middle income (UMIC)
- 6 Lower middle income (LMIC)
- 1 Low income (LIC)

CFC Prices per IU in High Income Countries

	Range	Median	Mean
Plasma Derived FVIII (13)	€0.04 - €0.59	€0.17	€0.24
Recombinant FVIII (11)	€0.07 - €1.30	€0.19	€0.37
Plasma derived FIX (7)	€0.07 - €0.70	€0.31	€0.37
Recombinant FIX (6)	€0.25 - € 0.68	€0.49	€0.46
EHL FVIII (16)	€0.08 - €0.87	€0.46	€0.45
EHL FIX (10)	€0.49 - €2.17*	€1.37	€1.40

CFC Prices per IU in Upper Middle Income Countries

	Range
Plasma Derived FVIII (4)	€0.06 - €0.25
Recombinant FVIII (3)	€0.19 - €0.38
Plasma derived FIX (1)	€0.06
Recombinant FIX (0)	
EHL FVIII (1)	€0.16
EHL FIX (0)	

CFC Prices per IU in Lower Middle Income Countries

	Range	Median	Mean
Plasma Derived FVIII (8)	€0.06 - €0.31	€0.10	€0.10
Recombinant FVIII (3)	€0.06 - €0.16		
Plasma derived FIX (0)			
Recombinant FIX (1)	€0.09		
EHL FVIII (0)			
EHL FIX (0)			
LIC: 1 price: Plasma derived FVIII: €0.02			

Prices by Income Category

	HIC	UMIC	LMIC	LIC
• Plasma derived	€0.04 - €0.70	€0.06 - €0.16	€0.06	€0.02
• Recombinant	€0.07 - €1.30	€0.19 - €0.38	€0.06	
• EHL FVIII	€0.08 - €0.87	€0.16		
• Lowest prices not necessarily in lower income				
• Prices also depend on procurement system, volumes purchased				
• In some HICs using significant amounts of Emicizumab, volumes of FVIII purchased decreased significantly- unit costs may rise				

Emicizumab Prices/ mg.

	Range	Median	Mean
High Income (12)	€14.62 - €82.86	€50.89	€51.29
Upper Middle (7)	€8.57 - €48.26	€17.00	€18.26
Lower Middle (2)	€7.35 - €14.41		

Annual average cost/ patient quoted (5) €25,000-€200,000

Data excludes prices in USA, France

Conclusions

- Publicly available information on prices often limited to list prices- bear no relation to reality
- Companies like to maintain high list prices for reference pricing purposes
- Procurement system, duration, volumes important factors
- Haemophilia treaters and National patient organisations need to be involved and need knowledge on price trends
- Possible to negotiate competitive prices for LIC, LMIC, UMIC

Conclusions

- In HICs, complex mix of therapies on market (CFCs, EHLs, Mimetics, Gene Therapy)- move away from simple tender – MCDA
 - :Can also use framework agreements or dynamic purchasing systems
- Can compare using cost per patient/ year- already in place with Emicizumab in some countries
- For Gene Therapy- outcome based or annual payment models may be based on cost of adult prophylaxis/ year x agreed number of years
- Knowledge is power

Thank You

My sincere appreciation to:

WFH for sharing Survey data

Individual clinicians and patient organisation leaders who provided information on their national prices

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Edit profile

Brian O Mahony

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