

CLINICAL AND ECONOMIC IMPACT OF PHARMACOKINETIC-GUIDED SWITCHING FROM STANDARD HALF-LIFE TO EXTENDED HALF-LIFE FACTOR IX IN HAEMOPHILIA B PROPHYLAXIS

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BACKGROUND AND IMPORTANCE

- Extended half-life (EHL) FIX ↑ exposure vs standard half-life (SHL): ↑ protection and reducing infusion burden.
- Evidence on PK-guided switching in routine practice remains limited: real-world evaluation is needed on PK outcomes, clinical bleeding, treatment burden, and costs after switching.

AIM AND OBJECTIVES

- Compare **PK parameters**: half life ($t_{1/2}$), area under the curve (AUC), trough level.
- Compare **clinical outcomes**: annual bleeding rate (ABR) and annual joint bleeding rate (AJBR), target joints.
- Quantify **treatment burden**: infusion frequency, weekly dose).
- Estimate **annual drug cost**: Laboratory Purchase Price without Value-Added Tax (LPP).

MATERIAL AND METHODS

Study design

- Retrospective
- Observational
- Before- after
- Multicentre
- Multidisciplinary

Study disease

Moderate/severe haemophilia B on prophylaxis

Data source

- Clinical: OrionClinic® ;
 - ABR, AJBR, infusion frequency, weekly dose
- PK: WAPPS-Hemo®
 - $t_{1/2}$, AUC, trough level
- Economic: Orion-Logis®
 - drug cost; LPP excluding VAT

Statistics

- Median (Interquartile range, IQR), n (%)
- Paired comparisons; significance level as per protocol
- R v4.4.3

RESULTS

N = 21 (9 paediatric, 12 adults)

No age-group differences in PK improvement ($p > 0.05$)

Outcome	SHL median (IQR)	EHL median (IQR)	p (All)	p <18	p ≥18
Weekly dose (IU/kg/week)	75.0 (57.1–108.1)	33.3 (26.6–47.3)	<0.0001	0.004	<0.001
Infusion frequency (per week)	2.0 (1.0–2.0)	0.7 (0.5–1.0)	<0.0001	0.008	0.002
$t_{1/2}$ (h)	34.5 (28.8–42.8)	133.5 (123.3–165.3)	<0.0001	0.004	<0.001
AUC (IU·h/L)	17,072 (8,742–21,933)	78,175 (53,438–85,433)	<0.0001	0.031	<0.001
Trough level (IU/dL)	3.9 (2.7–6.8)	10.8 (8.0–16.2)	<0.001	0.009	0.009
ABR (n)	1.0 (1.0–2.0)	0.0 (0.0–1.0)	0.009	0.015	0.145
AJBR (n)	1.0 (0.0–1.0)	0.0 (0.0–0.0)	0.095	0.054	0.714

Table 1. PK parameters of SHL and EHL FIX

Figure 1. Treatment burden after PK-guided switch (paired analysis)

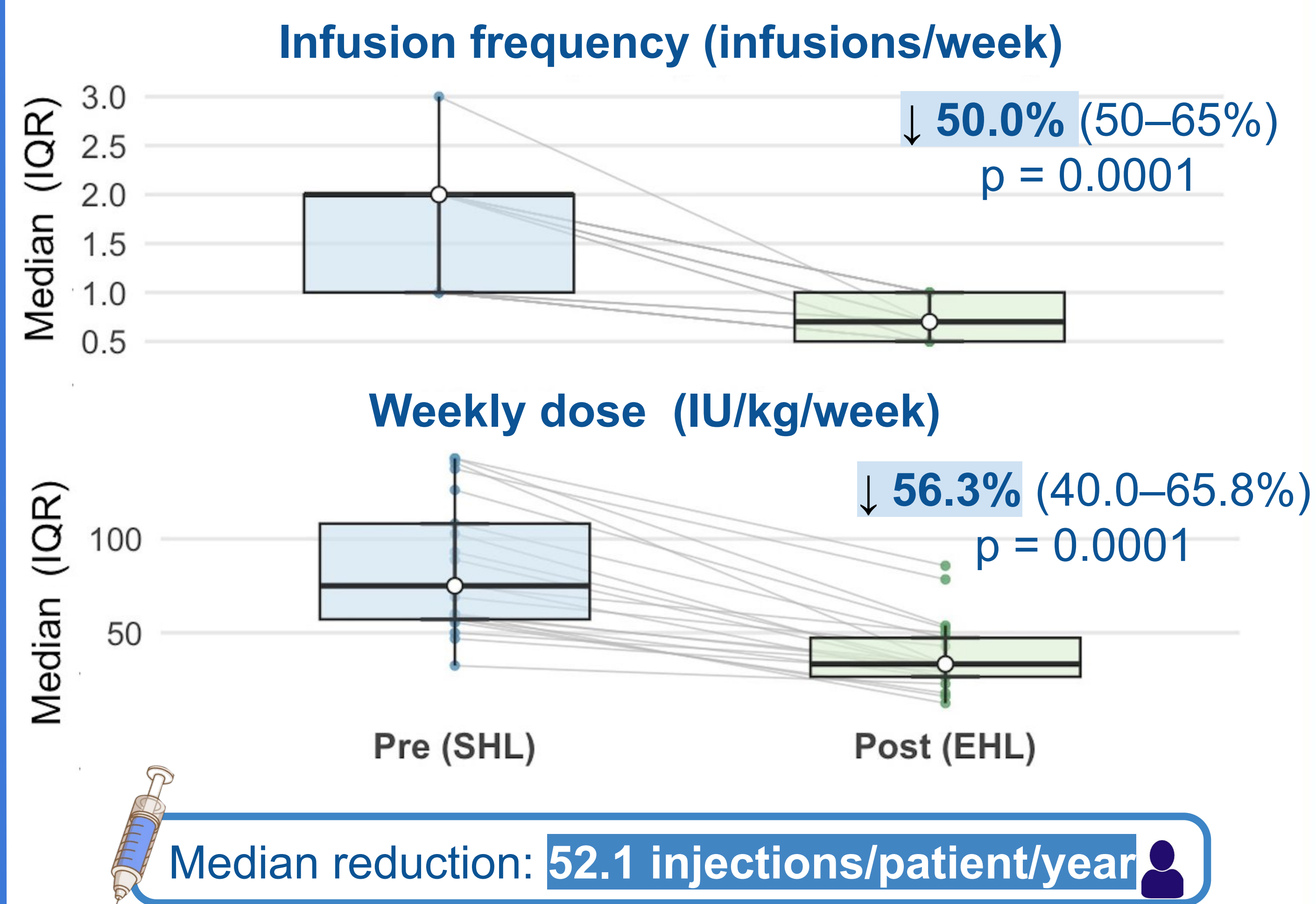


Figure 2. $t_{1/2}$ and AUC ratios of FIX products by patient

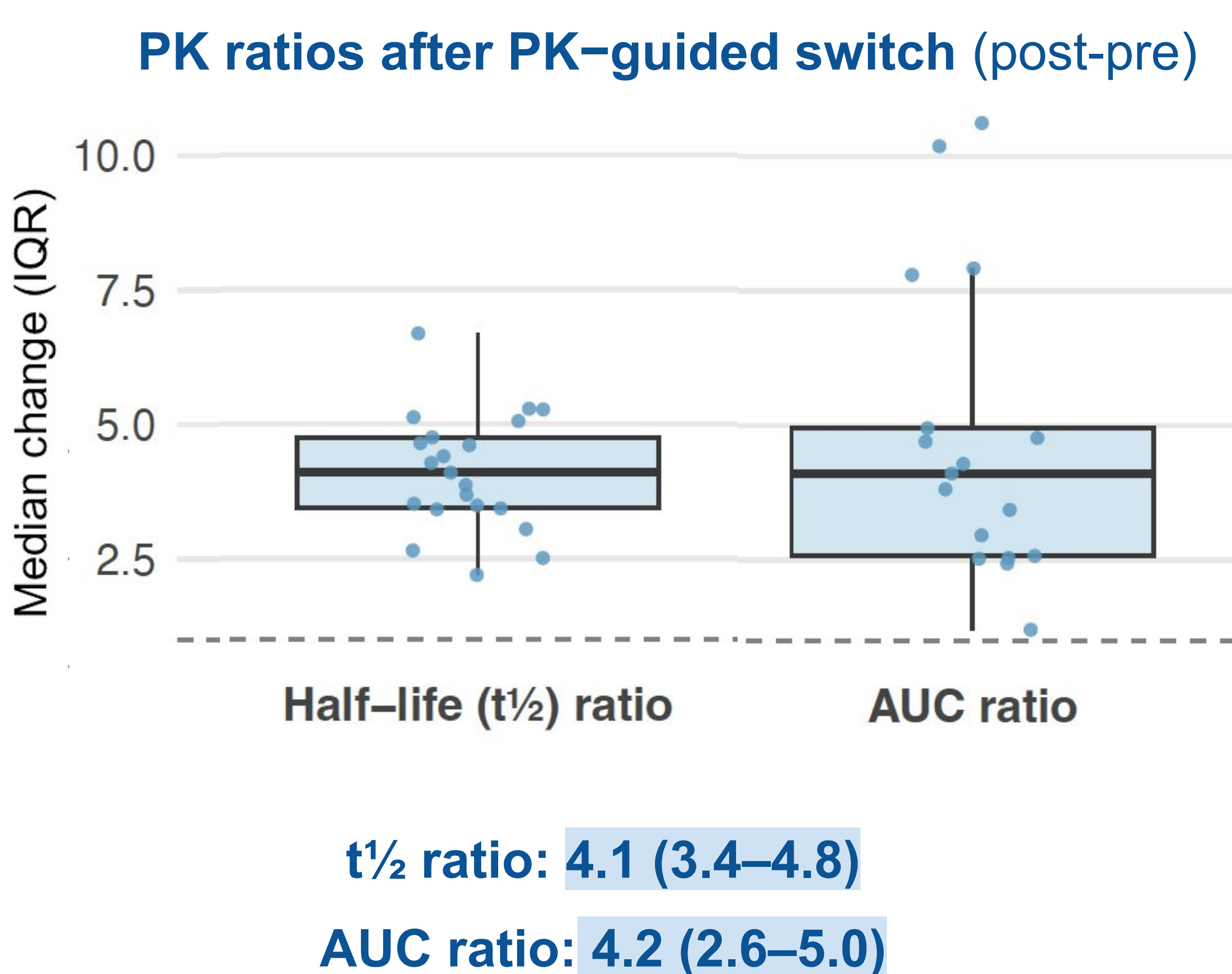
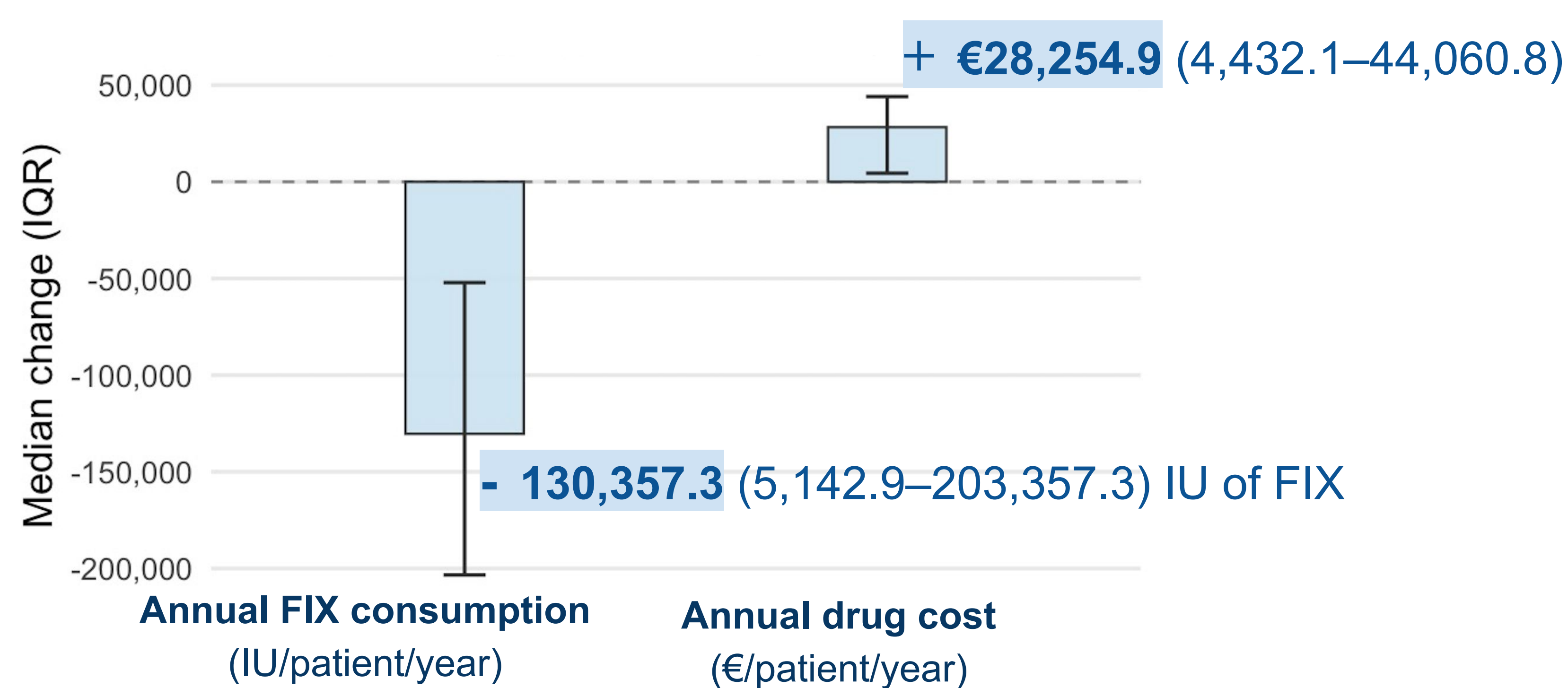


Figure 3. Change in annual FIX consumption and cost after switch (post-pre)



Clinical outcomes

ABR (SHL) 1.0 (0.0-1.0) → ABR (EHL) 0.0 (0.0-0.0) : ↓ ($p=0.009$)

Resolution: 5 out of 6 (83.3%) target joints resolution

CONCLUSIONS

- PK-guided switching to EHL FIX markedly improved PK: ~ 4-fold increases in $t_{1/2}$ and AUC).
- Treatment burden substantially decreased (fewer infusions and lower weekly dose).
- Despite lower FIX consumption, drug-related annual costs increased ; broader real-world studies including indirect and healthcare resource costs are needed.

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