

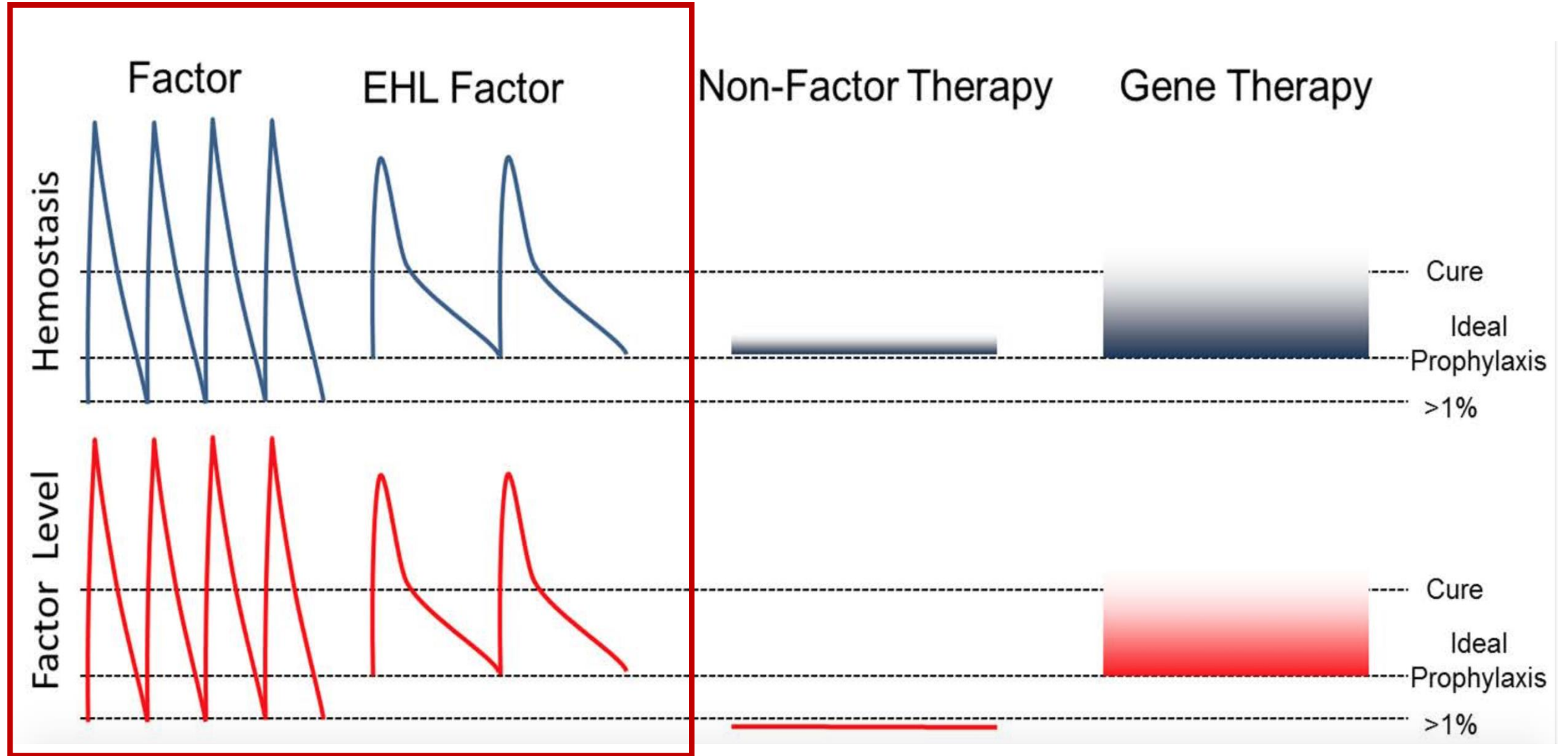
Estimating Value Of Novel Therapies

EHC Round Table

Feb 2018

Declan Noone

Moving to Novel Therapies



Economic Value

Option A

70Kg Patient, FVIII

- Half-Life 12 hours
- 35IU/kg – 71% Rise
- Every 2 Days
- 4.4% Trough
- Infusions/year -182

Option B

70Kg Patient, FVIII

- Half-Life 18 hours
- 35IU/kg – 71% Rise
- Every 3 Days
- 4.4% Trough
- Infusions/year -122

Economic Value

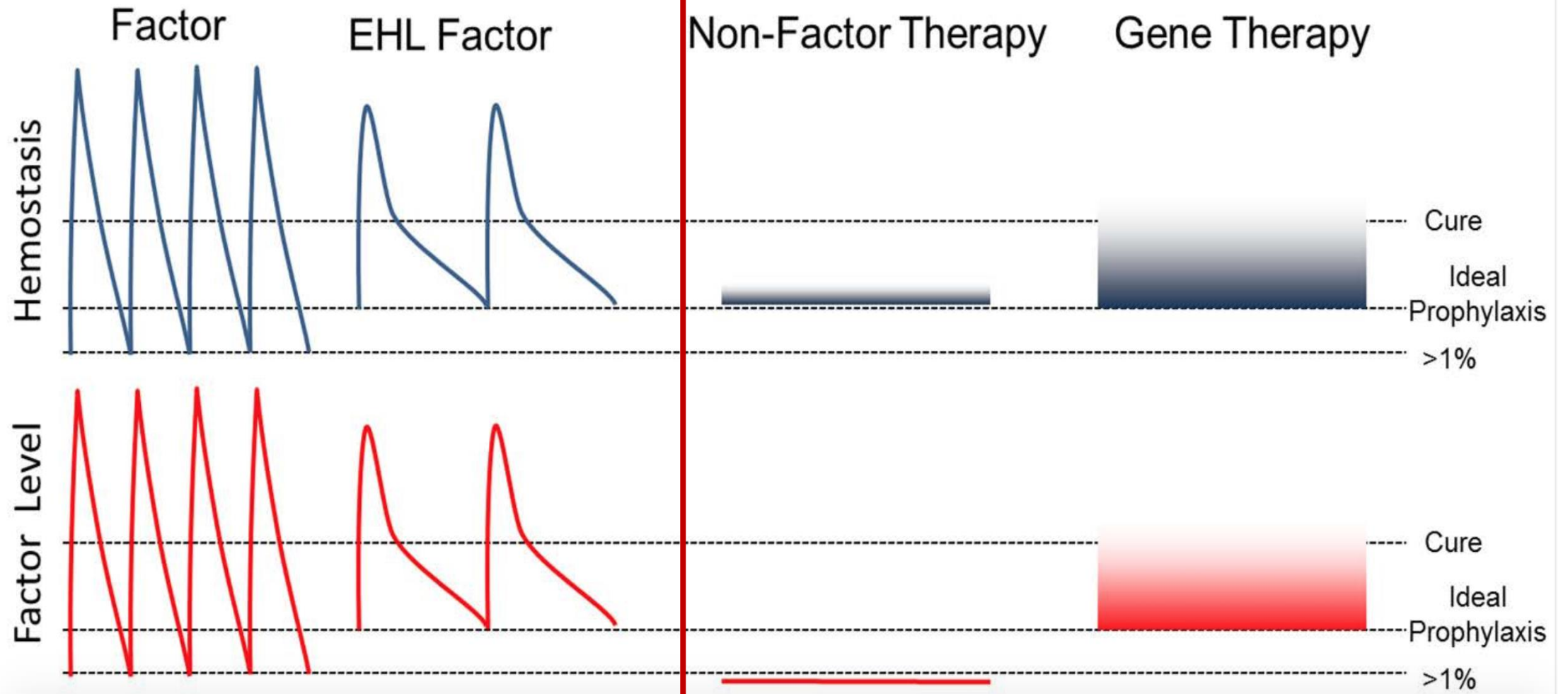
Option A

- Half-Life 12 hours
- 50IU/kg – 100% Rise
- Every 2 Days
- 6.3% Trough
- Infusions/year -182

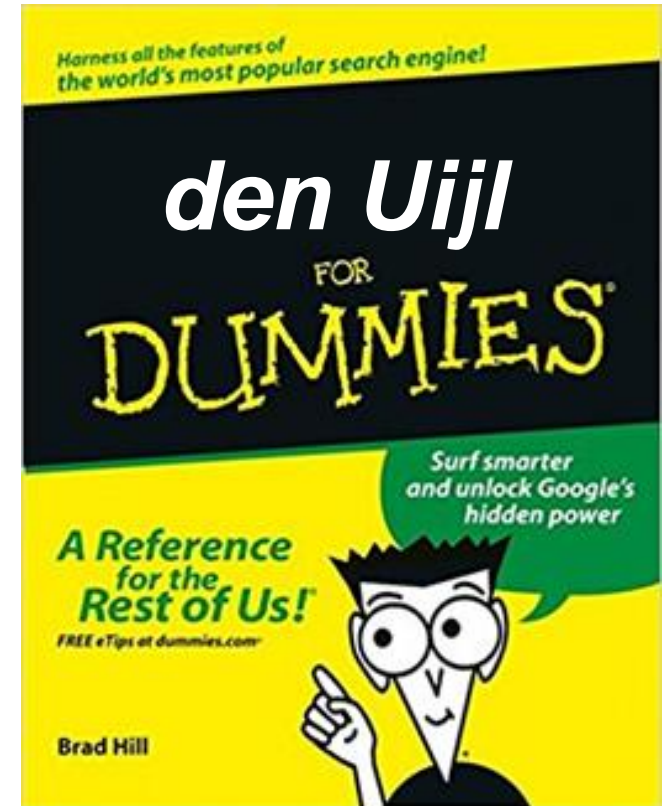
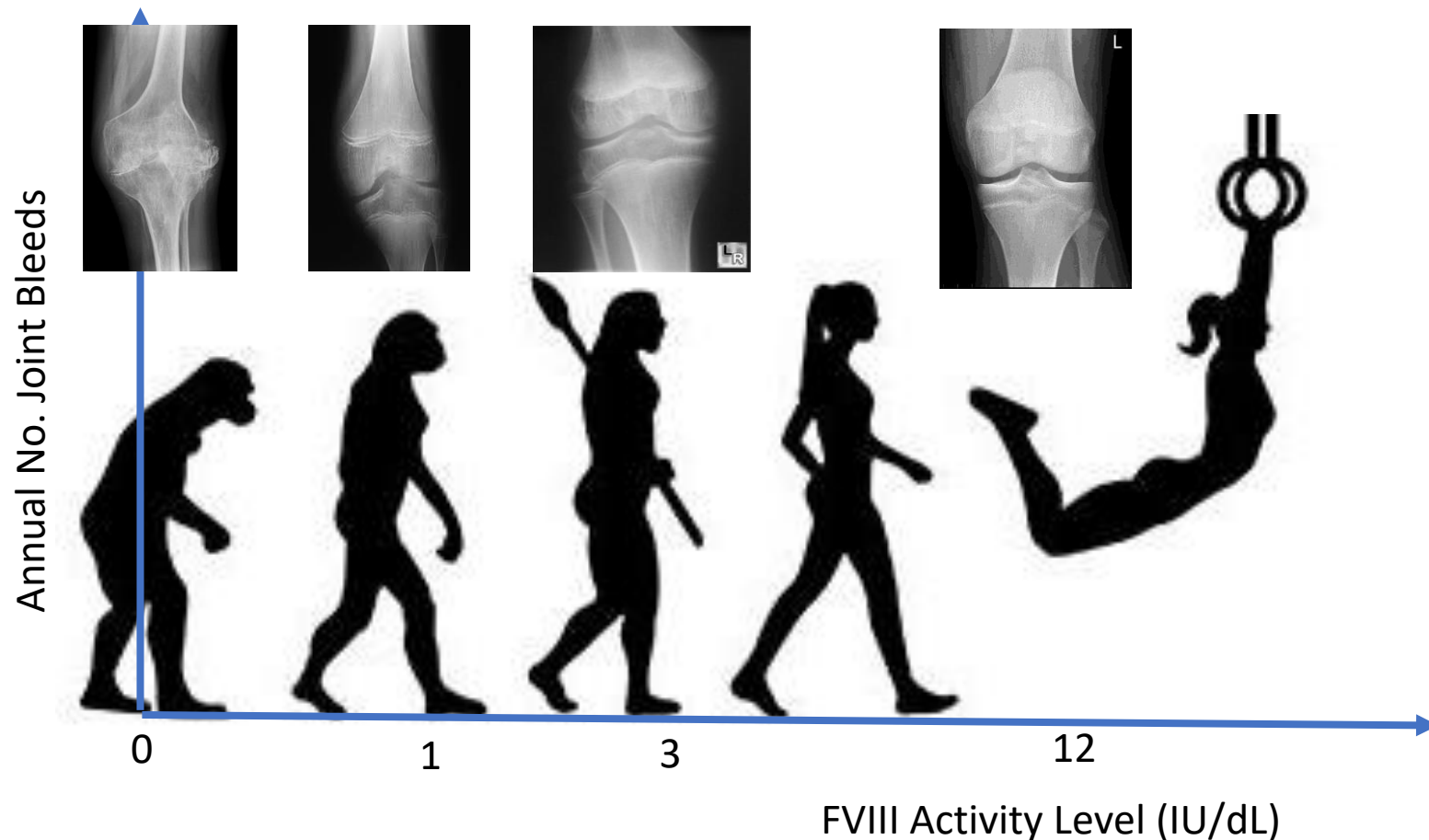
Option B

- Half-Life 18 hours
- 35IU/kg – 71% Rise
- Every 3 Days
- 4.4% Trough
- Infusions/year -122

Moving to Novel Therapies



Trough Level Not Just a “Surrogate Marker”



Other Considerations

- We shouldn't Invest in Peaks?



- Adherence Issues or Just Thin Ice?

- EOD – 6 am each morning
 - 4% Trough
 - Bleed below 3%
- Life Happens!!



- Economics

- A **rational** behaviour decision-making process is based on making choices that result in the most optimal level of benefit or utility for the individual.

Known Unknown's – Individual Potential

Spontaneous Bleeds
to a
Spontaneous Life

**Aim for the sky and
you'll reach the ceiling.
Aim for the ceiling and
you'll stay on the floor.**

New Job, New
Opportunity, Career
Advancement
But
“Undue Economic
Burden”

Leaving Home
“Unprotected”



The Future's Future
Hopes – For Free!!!!



So How Do We Include these?

Relevance of Health Problem

- disease severity, population size, goals of the health system

Relevance of Technology

- completeness and validity of evidence

Safety

- added safety and tolerability

Effectiveness

- added efficacy / effectiveness, patient reported outcomes, compliance with guidelines, problems of available alternatives

Financial Aspects

- direct financial impact on NHS, impact on other sector's expenses, cost-effectiveness, impact on efficiency of health care offering

Equity of Access

- fair opportunity of access

Social Aspects

- impact on social needs, stakeholder pressures / needs

Organisational

- system capacity and good use within organisations

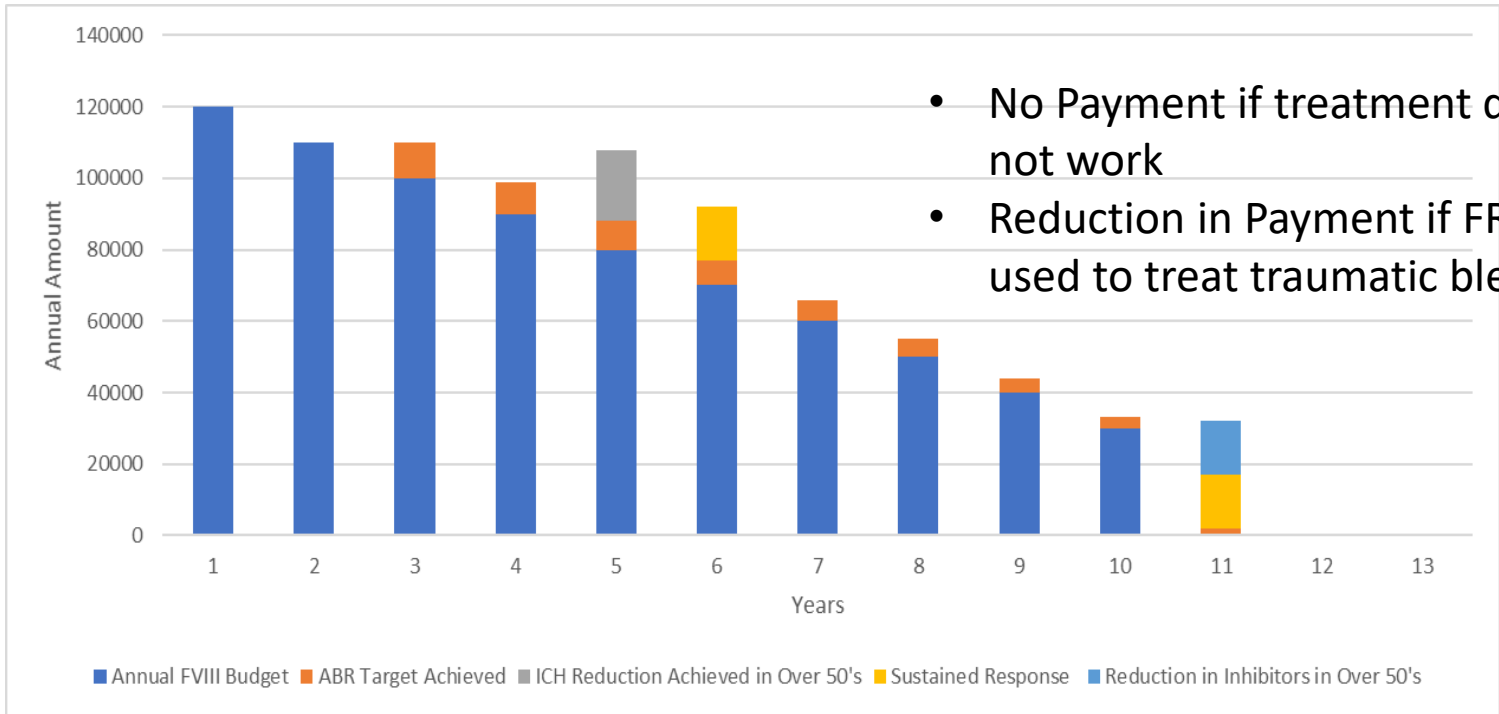
Legal/ Ethical Aspects

- adherence to legal requirements



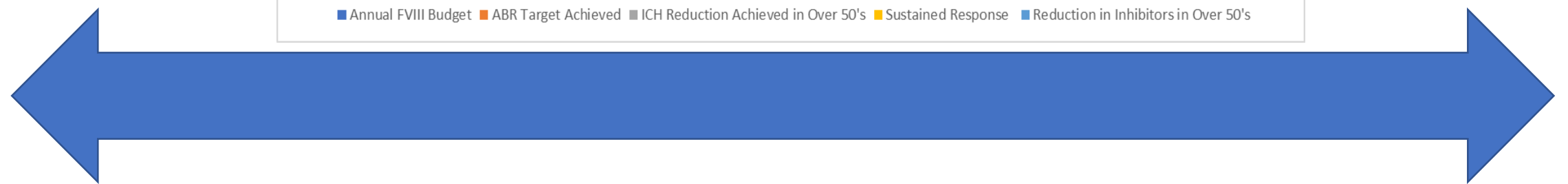
Gene-Therapy Value Based Assessments

- Managed Entry / Conditional Approval / Outcomes Based Pricing



Payment Model
One-Off
Multi-Payer Systems

Payment Model
Multi Year
Single Payer Systems

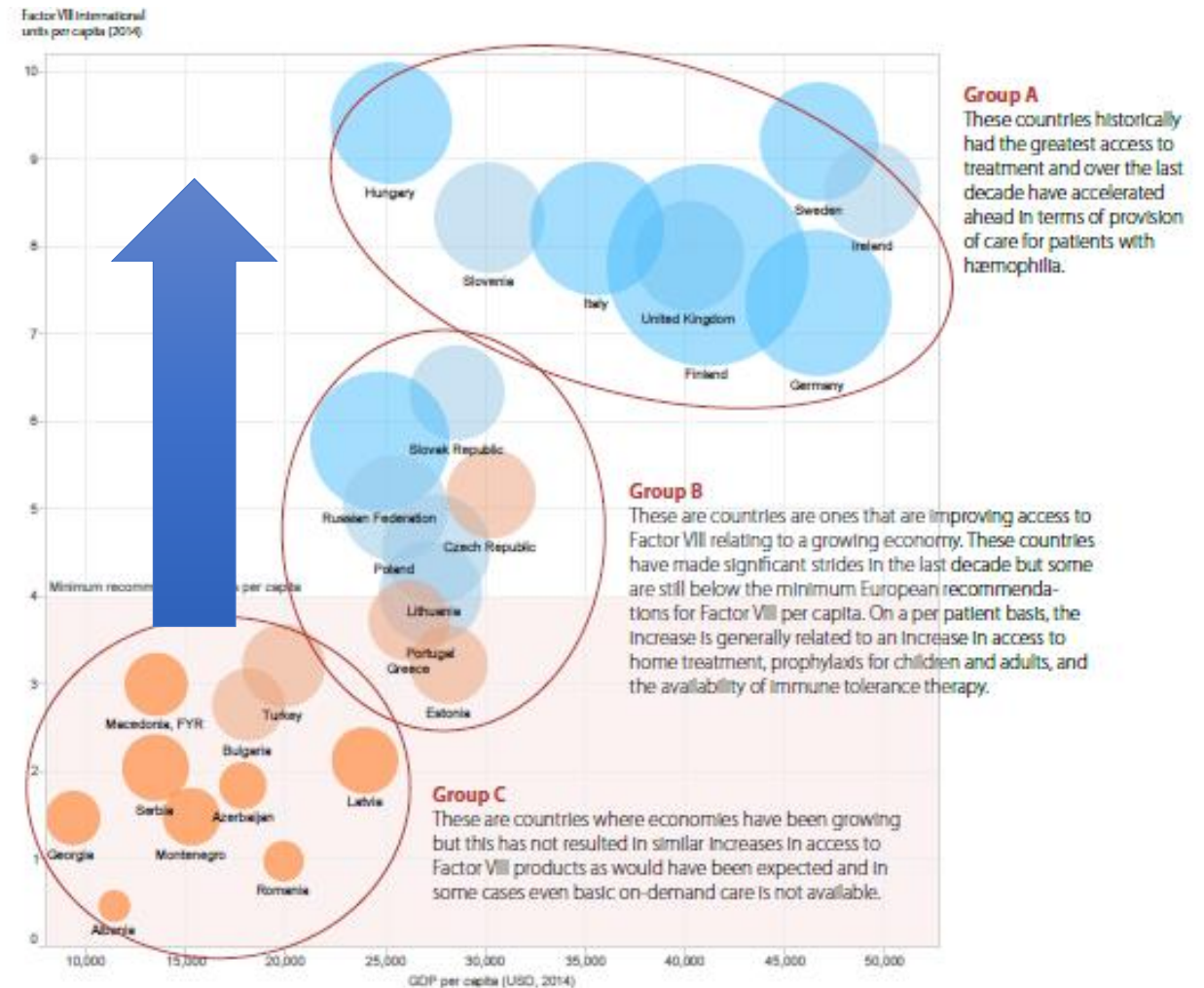


EHC Survey 2015

Significant disparity of access across Europe



- 3 Distinct Groups
 - Countries who have had access and are accelerating in terms of access
 - Countries who have had limited access with limited economic resources but improvements in co-ordination of care and tendering have resulted improvements
 - Countries with limited resources with disparate approaches to care and purchasing
- **PARTNERS** programme assist countries move beyond economic constraints!!



Values of New Therapies

- Exciting time for the Economists as well as patients!!!!
 - Outcome Based Approaches best way forward
- Including the appropriate measures
 - For PWH of today and tomorrow
- "If everyone is moving forward together, then success takes care of itself." -
-Henry Ford
- Multi-year planning essential
 - Scalpel vs. Hatchet approaches to procurement