



Product selection and economic aspect

Declan Noone
Irish Haemophilia Society
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European Haemophilia Procurement Methods

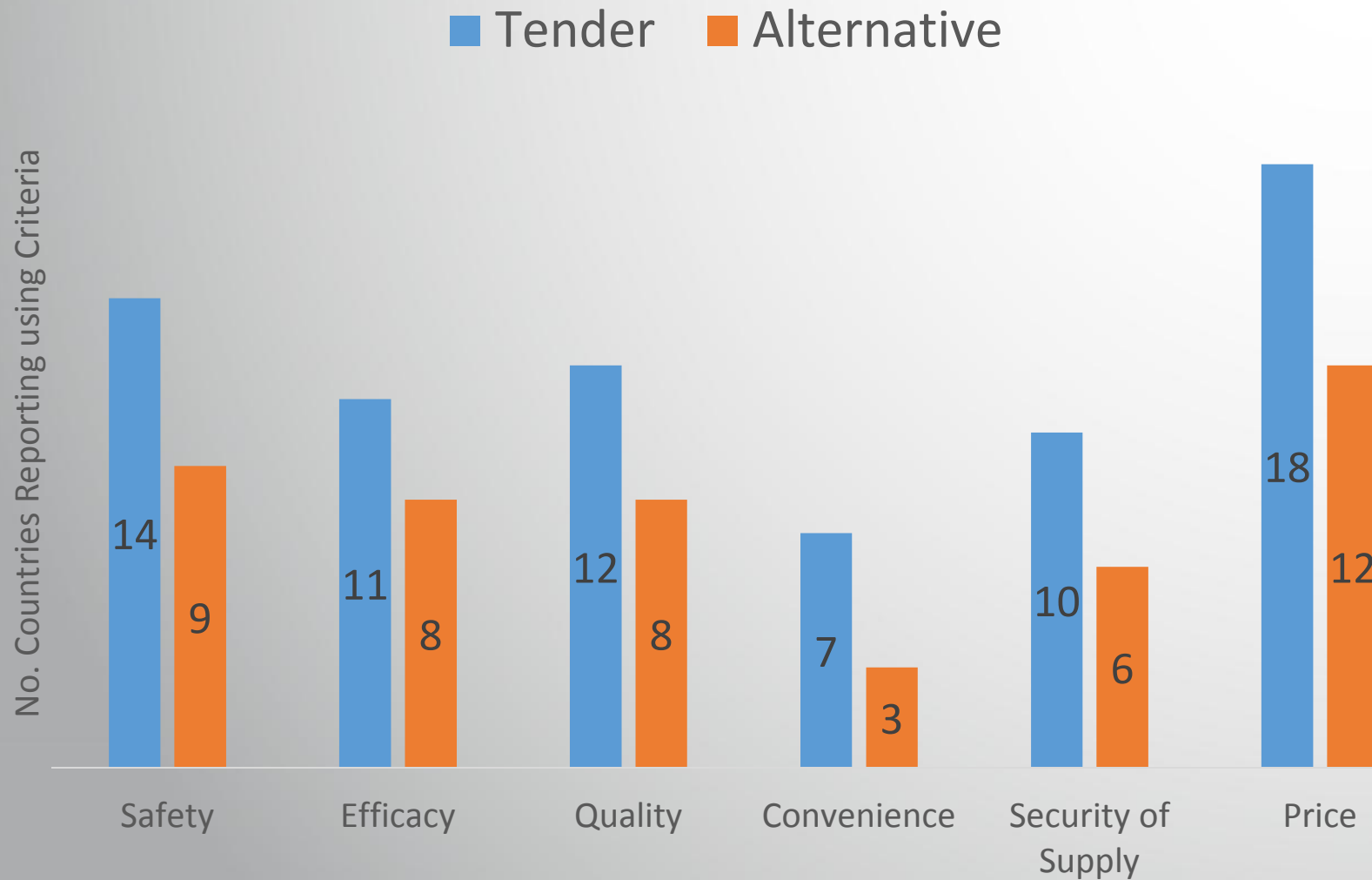
- Wide variation of methods
- Alternative methods
 - Lowest price on national basis
 - No assessment of other criteria
 - Preference of individual hospitals, states, insurance
 - Assessment minimal, volumes limited and unpredictable, limited transparency on price
 - National maximum price
 - Limited review of products, limited transparency on price, less impetus for co-ordination
 - All products available

European Tender Procurement Models



- National or co-ordinated regional purchase
- National expert procurement committee
 - 2 year commitment
- Regular review
 - 1-3 year contracts
- Predictable volumes
 - Companies
 - Payers
 - Clinicians
 - Patients

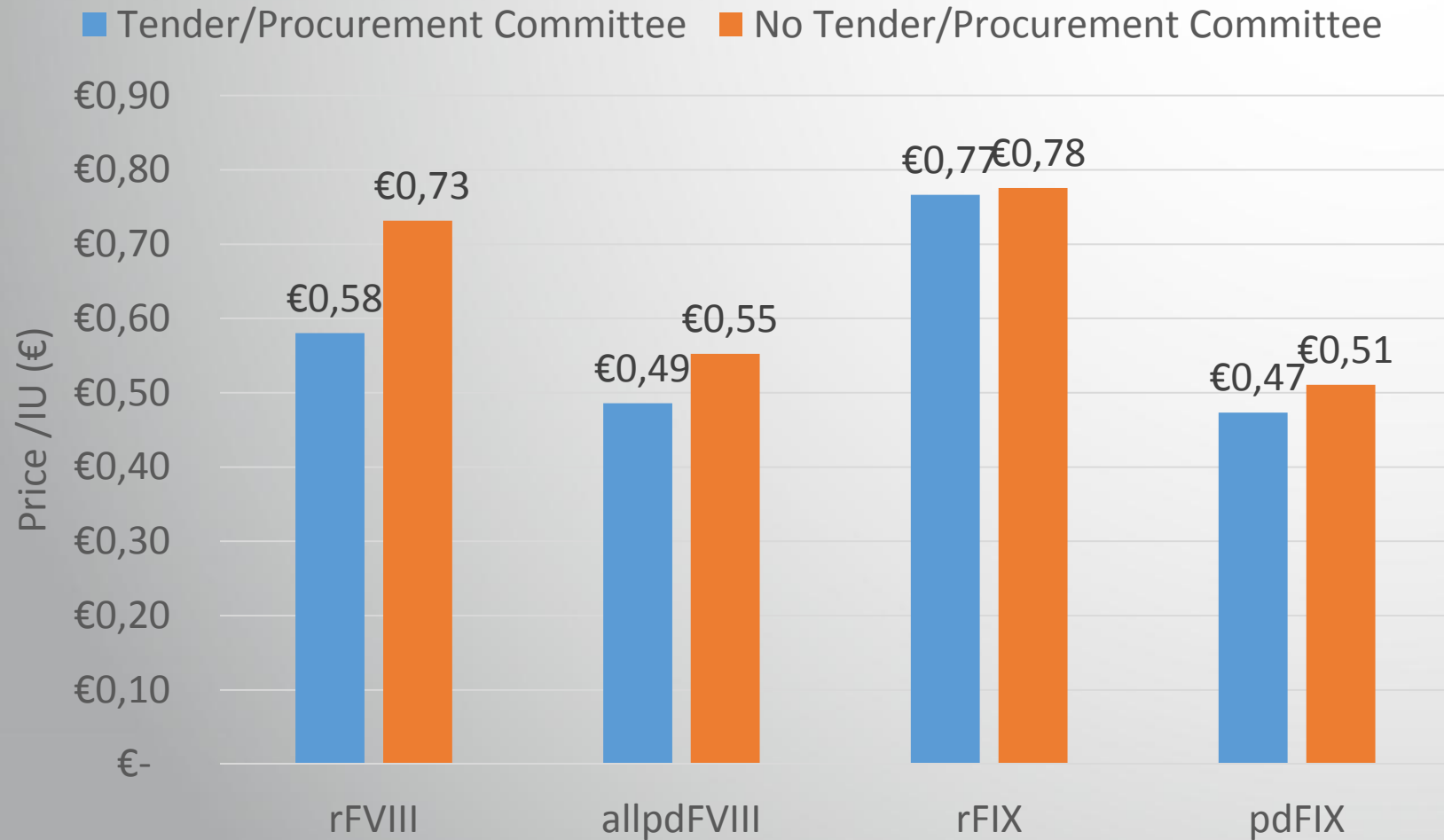
Main Criteria Reported for Assessing CFC's



Effect of Procurement Committee



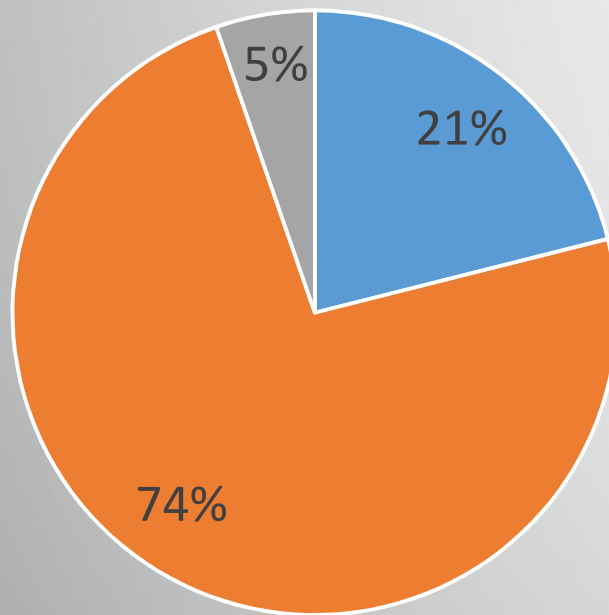
Irrelevant of tender or alternative approach



Use of a Registry to Predict Factor Volumes

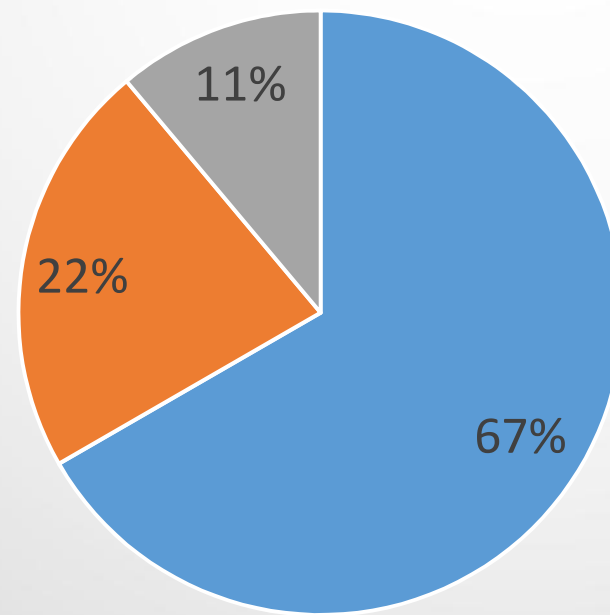


Tender



- No Registry used
- National
- Hospital

Alternative



- No Registry used
- National
- Hospital



Additional Components

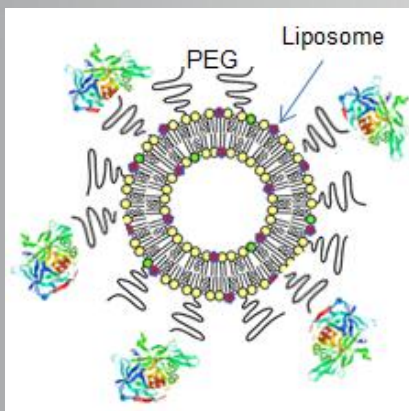
- Significant burden on haemophilia budgets
- Tax - disparate
 - No tax on any product (5)
 - Recombinant (20) vs. Plasma-derived (19)
 - Essential vs. Luxury tax bands
 - Variation between 2 to 25%
 - Hospital vs. Home delivery
- Contracting agents
 - Handling charges
- Distribution chain
 - Patients home
 - Local pharmacies
 - Hospital /htc's
 - Pharma company

Strategies for New Therapies

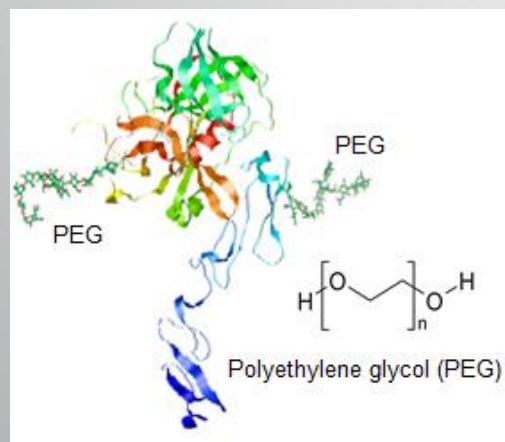


Half-life extension

PEGylated Liposomes

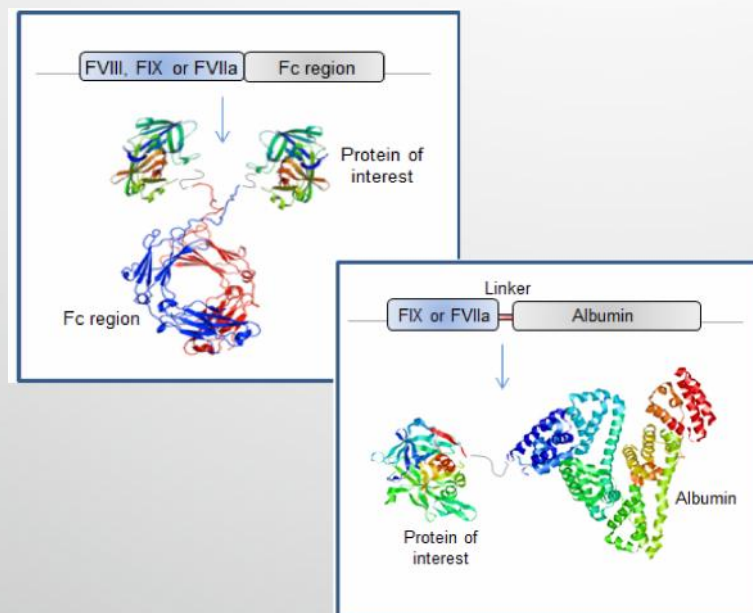


PEGylation
- Random
- Site specific



Modification of amino acid sequence
Fusion protein

- Fc fragment
- Albumin





Other Procurement Options

HTA's

- Cost-effectiveness vs. Budget impact
- Limited ability to assessment

Hep C

Re-imburement schemes

- Cost per treatment
- Cost per patient
- Cost per SVR
- Cancer
 - Budget based on pre defined outcomes

Future of Haemophilia Procurement



- Current tender systems
 - Standard half-life vs. Long-acting
 - Very successful
- Cost per patient
 - Review of all patient usage
 - Specific budget
 - Issues as patient grows
 - Limited focus on outcomes for the patient

Future of Haemophilia Procurement



- Cost to a minimum trough level
 - Individual tailored dosing
 - Regular assessment of patients
 - Adjusts for patients age
 - Change focus on current concepts of prophylaxis
 - 3 – 5%
 - Adjustments for surgery for older patients built-in
 - Mechanism for inhibitor treatments
 - Overall better outcomes for the patient

Future of Haemophilia Procurement



- Cost based on outcomes
 - Individual tailored dosing by lifestyle
 - Regular assessment of patients
 - Adjusts for patients age, activities, trough and bleeding phenotype levels
 - New concept of care
 - Children 0-5 years - trough level 3%
 - Children 5-13 years - trough level 5%
 - Teenagers
 - Active - trough level 5%
 - Very Active - trough level 12%
 - Adult Minimally Active - trough level 1%
 - Adult Very Active - trough level 5%
 - Inhibitor Treatment
 - Haemophilia Service
 - Clinician / Nurse/ Physiotherapy / Psychology/ Personal Trainer
 - Best Outcomes for the patient

Preparation for New Models of Procurement



- Haemophilia ideally placed for new models of care
- National tender systems
- Expert groups for procurement
- Organised care
 - Registries
 - Comprehensive care centres
 - Treatment monitoring
- Excellent clinician /patient co-operation
- Educated and engaged patient population