Integrated Impact Assessment Report for Clinical Commissioning Policies			
Policy Reference Number	1716		
Policy Title	Human coagulation factor X for hereditary factor X deficiency (all ages) Proposal Choose an item. (ref A3.1)		
Lead Commissioner	Will Horsley	**Clinical Lead**	Mary Mathias
Finance Lead		**Analytical Lead**	Click here to enter text.

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About this Impact Assessment: instructions for completion and explanatory notes
• Each section is divided into themes. • Each theme sets out a number of questions. • All questions are answered by selecting a drop down option or including free text. • Free text boxes are provided to enable succinct relevant commentary to be added which explains the rationale for response or assumption. Please limit responses to 3 sentences of explanatory text. • Data in this document is either drawn from one of the relevant policy documents or a source for the information is provided. • Where assumptions are included where data is not available, this is specified.

Section A - Activity Impact	
A1 Current Patient Population & Demography / Growth	
A1.1 Prevalence of the disease/condition.	The National Haemophilia database (2016) shows that 241 people have a diagnosis in the UK. An estimated incidence of FX supplied by the company is 4% of the current number of people diagnosed in the UK. The prevalent pool of FX patients is therefore anticipated to increase by 10 patients per annum. *Source: Austin SK, Kavakli K, Norton M et al. (2016) Efficacy, safety and pharmacokinetics of a new high purity factor X concentrate in subjects with hereditary factor X deficiency. Available [online] from:* (Austin et al. 2016).
A1.2 Number of patients currently eligible for the treatment according to the proposed policy commissioning criteria.	Approximately 15% of the prevalent and incident population (36 people) will be eligible to initiate treatment. Data from the National Haemophilia Database indicates that 39 patients were treated for FX deficiency across 17 providers in England in 2016-17 39 received any treatment (age 0 to 76 yrs) in 2016-17 Of which an estimated 25 (age 0 to 65 yrs) are on prophylaxis, of whom 13 are aged 0 to 16 years.
A1.3 Age group for which the treatment is proposed according to the policy commissioning criteria.	**All ages** The policy proposition supports the long-term prophylaxis indication for human coagulation factor X in all age groups. The SPC for human coagulation factor X states that the dose and duration of treatment in adults and children are the same, but the safety and efficacy of human coagulation factor X in children less than 12 years of age has not yet been established. The Ten02 study is a phase III study in children under 12 years with moderate to severe Factor X deficiency. The study was presented in abstract form at the 2017 International Society on Thrombosis and Haemostasis Congress. The unpublished results from

<table>
<tr><td></td><td>Liesner et al. (Ten02) have been taken into account by the policy working group based on a confidential draft of the article which was provided by the company. This will be published in the near future and will be available at the time a commissioning policy is considered for routine commissioning. An overview of the study shows that preliminary data were in line with those observed in the Ten01 study.</td></tr>
<tr><td>A1.4 Age distribution of the patient population eligible according to the proposed policy commissioning criteria</td><td>For the year 2016:
All patients: 0 to 65 years, mean age 20 years.
12 adults, mean age 33 years
13 paediatrics, mean age 8 years.

Source National Haemophilia Database.</td></tr>
<tr><td>A1.5 How is the population currently distributed geographically?</td><td><u>Unevenly</u>
If unevenly, estimate regional distribution by %: This represents all treated patients in 2016 based on provider location; the proportion has not been adjusted for prophylaxis only
<table>
<tr><td>North</td><td>10</td><td>26%</td></tr>
<tr><td>Midlands & East</td><td>13</td><td>33%</td></tr>
<tr><td>London</td><td>16</td><td>41%</td></tr>
<tr><td>South</td><td>0</td><td>0%</td></tr>
</table>
Source: National Haemophilia Database</td></tr>
<tr><td colspan="2">A2 Future Patient Population & Demography</td></tr>
</table>

A2.1 Projected changes in the disease/condition epidemiology, such as incidence or prevalence (prior to applying the new policy) in 2, 5, and 10 years?	**Increasing** The prevalence of factor X deficiency is between 1 and 4 people per 1,000,000 population. The number of diagnosed cases currently registered by the **UK** haemophilia database is 241. An incidence rate of 10 new cases per year has been provided by the company. Applying these assumptions to the ONS projected population data gives a growth rate of; 241 people in 2018/19 (Year 1) 251 people in 2019/20 (Year 2) 282 people in year 2022/23 (Year 5) 343 people in year 2027/28 (Year 10) *Source: Resource impact template. References are given in A1.1 above.* Note, this refers to all factor X deficiency of which a minority require treatment for severe disease. Between 2010 and 2016 the number of diagnoses in the UK increased from 180 to 241(33% increase). The number of patients treated over the same period increased from 32 to 35 (annual range 28 to 37), and increase of 9%. The proportion of diagnosed patients treated in any given year has remained stable in the range 15 to 18%. Based on this data we estimate total number of treated patients in England to be 40 in 2 yrs, 42 in 5 yrs and 45 in 10 yrs of which we estimate prophylaxis treatment numbers to be 26, 27 and 29 respectively.

ENGLAND	Treated	Prophylaxis
0 yrs	39	25
2 yrs	40	26
5 yrs	42	27
10 yrs	45	29

<table>
<tr><td>A2.2 Are there likely to be changes in demography of the patient population and would this impact on activity/outcomes?</td><td><u>No</u>
No change in the distribution of patients across England is expected. The eligible population for treatment with human coagulation factor X is those patients with severe factor X deficiency. It is conceivable that patients might specifically migrate to England for treatment if it was routinely commissioned. This has not been accounted for. No other changes expected from the available data.</td></tr>
<tr><td>A2.3 Expected net increase or decrease in the number of patients who will be eligible for the service, according to the proposed service specification commissioning criteria, per year in years 2-5 and 10?

Are these numbers in line with ONS growth assumptions for the age specific population? If not please justify the growth assumptions made.</td><td><table><tr><td>YR2 +/-</td><td>+1</td></tr><tr><td>YR3 +/-</td><td>+2</td></tr><tr><td>YR4 +/-</td><td>+2</td></tr><tr><td>YR5 +/-</td><td>+3</td></tr><tr><td>YR10 +/-</td><td>+6</td></tr></table>Modelled using recent historical data from the National Haemophilia Database</td></tr>
<tr><td colspan="2">A3 Activity</td></tr>
<tr><td>A3.1 What is the purpose of new policy?</td><td><u>Confirm routine commissioning position of an additional new treatment</u>
<u>Remove and replace a currently routinely commissioned treatment</u>
The purpose of the new policy is to propose human coagulation factor X as a prophylaxis treatment option for people of all ages with hereditary</td></tr>
</table>

	Factor X deficiency.
A3.2 What is the annual activity associated with the existing pathway for the eligible population?	enter number. England, 2016-17: 39 patients with factor X deficiency received any treatment. *Source:* NHD NHD records 230 patients registered at an English centre with factor X deficiency for financial year 2016/2017 (104 males/126 females). 39 of these patients treated across 17 providers in England. Treatment is either on-demand (following actual or expected bleeding episode) or prophylaxis (to prevent bleeds). Some evidence of patient clustering as expected with a rare inherited disorder.
A3.3 What is the estimated annual activity associated with the proposed policy proposition pathway for the eligible population?	No change expected, simply the substitution of one IV treatment for another.
A3.4 What is the estimated annual activity associated with the next best alternative comparator pathway for the eligible population? If the only alternative is the existing pathway, please state 'not applicable' and move to A4.	No difference in activity expected. Substitution of one treatment for another.
A4 Existing Patient Pathway	
A4.1 **Existing pathway:** Describe the relevant currently routinely commissioned:	Standard treatment is replacement therapy using of plasma-derived prothrombin complex concentrate. The current model of care ensures that

• Treatment or intervention • Patient pathway • Eligibility and/or uptake estimates.	patients have access to an accredited comprehensive care centre (CCC) or can access services through a managed clinical network which includes at least one CCC. 39/230 (17%) patients in England were treated for factor X deficiency 2016/17. All patients diagnosed with factor X deficiency are believed to be registered with the National Haemophilia Database. The decision to offer treatment is based on individual risk assessment such as level of deficiency (severe, moderate, mild) and lifestyle factors. Similarly the decision to commence regular prophylactic treatment is an individualised assessment which also includes patient education, training and support. Evidence indicates that about 10% of factor X deficiency patients were treated with a prophylactic regimen in 2016-17. *Source: NHD & NHS Standard Contract for Haemophilia (all ages)*
A4.2. What are the current treatment access and stopping criteria?	Those who require prophylaxis treatment, for example, for perioperative management of bleeding, or to prevent spontaneous bleeding episodes - are usually cases of moderate to severe factor X deficiency. Access is also required on-demand; to treat a bleed at the time it occurs. This treatment is usually in mild to moderate cases. This is a lifelong bleeding disorder. *Source:* Shapiro (2017), Mumford et al (2014) People with Factor X deficiency are managed in specialist clinical haematology haemophilia centres. Prothrombin complex concentrate (PCC), the current treatment option used in the UK for people with hereditary factor X deficiency, contains factor II, VII, IX, and X Because PCCs contain relatively small amounts of factor X, the volumes of PCC needed to achieve haemostasis (normal levels of blood clotting) are larger than an equivalent dose of factor X concentrate. Infusion with PCCs can take between 8 and 70 minutes depending on the PCC and required dose. *Source:* Policy proposition document section 6.
A4.3 What percentage of the total eligible population is expected to: a) Be clinically assessed for treatment	For the population relating to the policy proposition a) 100%: All people suspected of having Factor X deficiencies are

b) Be considered to meet an inclusion criteria following assessment c) Choose to initiate treatment d) Comply with treatment e) Complete treatment?	tested and assessed for treatment b) 10% c) 100% d) 100%) e) 100% This is a life-long treatment *Source: NHD Statistics and current practice* Click here to enter text.
A5 Comparator (next best alternative treatment) Patient Pathway (NB: comparator/next best alternative does not refer to current pathway but to an alternative option)	
A5.1 **Next best comparator**: Is there another 'next best' alternative treatment which is a relevant comparator? *If yes, describe relevant* • *Treatment or intervention* • *Patient pathway* • *Actual or estimated eligibility and uptake*	**YES** As described, the current standard of care for factor X deficiency who require treatment is PCC. This is offered to about two-thirds of treated patients as prophylaxis. Other treated patients are assumed to be treated only short-term for actual or expected (e.g. surgery) bleeding episodes. Treatment will be initiated and monitored by experienced clinicians at larger treatment centres. The new treatment is likely to replace PCC for all prophylaxis patients due to greater convenience and reduced risk of long-term complications.
A5.2 What percentage of the total eligible population is estimated to: a) Be clinically assessed for treatment b) Be considered to meet an exclusion criteria following assessment c) Choose to initiate treatment d) Comply with treatment e) Complete treatment?	Total estimated eligible a) 39 out of 230 diagnosed, approx. 17% of which 25 (approx. 10%) were treated with prophylaxis b) 0 c) Estimate 100%. However it is conceivable that some patients not currently receiving prophylaxis may opt to receive prophylaxis with Coagadex due to more convenient administration. d) 100%: Non-compliant patients have treatment withdrawn. e) 100%: This is a life-long treatment

A6 New Patient Pathway	
A6.1 What percentage of the total eligible population is expected to: a) Be clinically assessed for treatment b) Be considered to meet an exclusion criteria following assessment c) Choose to initiate treatment d) Comply with treatment e) Complete treatment?	Per A4.3 above Population relating to the policy proposition a) 100% b) 0% c) 100% d) 100% e) 100% *Source:*
A6.2 Specify the nature and duration of the proposed new treatment or intervention.	**Life long** *Source:* *Company submission – part 3 & policy proposition section 8*
A7 Treatment Setting	
A7.1 How is this treatment delivered to the patient?	*Select all that apply:* Emergency/Urgent care attendance ☐ Acute Trust: inpatient ☐ Acute Trust: day patient ☐

	<table><tr><td>Acute Trust: outpatient</td><td>☐</td></tr><tr><td>Mental Health provider: inpatient</td><td>☐</td></tr><tr><td>Mental Health provider: outpatient</td><td>☐</td></tr><tr><td>Community setting</td><td>☐</td></tr><tr><td>Homecare</td><td>☒</td></tr><tr><td>Other</td><td>☐</td></tr></table> Treatment will be arranged via homecare for self-administration. Patients will not normally be in possession of more than 3 month's supply of medication.
A7.2 What is the current number of contracted providers for the eligible population by region?	<table><tr><td></td><td>All Haemophilia Centres</td><td>Comprehensive Comp Care Centres</td></tr><tr><td>NORTH</td><td>10</td><td>8</td></tr><tr><td>MIDLANDS & EAST</td><td>9</td><td>5</td></tr><tr><td>LONDON</td><td>4</td><td>4</td></tr><tr><td>SOUTH</td><td>14</td><td>5</td></tr></table>

A7.3 Does the proposition require a change of delivery setting or capacity requirements?	**No** However treatment could be commissioned only via a Comprehensive Care Centre.
A8 Coding	
A8.1 Specify the datasets used to record the new patient pathway activity. *expected to be populated for all commissioned activity	*Select all that apply:* Aggregate Contract Monitoring * ☐ Patient level contract monitoring ☒ Patient level drugs dataset ☒ Patient level devices dataset ☐ Devices supply chain reconciliation dataset ☐ Secondary Usage Service (SUS+) ☐ Mental Health Services DataSet (MHSDS) ☐ National Return** ☐ Clinical Database** ☐ Other** ☒ **If National Return, Clinical database or other selected, please specify: Use of Factor X is already recorded in some detail by the UKHCDO with data delivered through Haemtrak/MDSAS

<table>
<tr>
<td>A8.2 Specify how the activity related to the new patient pathway will be identified.</td>
<td>
Select all that apply:
<table>
<tr><td>OPCS v4.8</td><td>☐</td></tr>
<tr><td>ICD10</td><td>☐</td></tr>
<tr><td>Treatment function code</td><td>☐</td></tr>
<tr><td>Main Speciality code</td><td>☐</td></tr>
<tr><td>HRG</td><td>☒</td></tr>
<tr><td>SNOMED</td><td>☐</td></tr>
<tr><td>Clinical coding / terming methodology used by clinical profession</td><td>☒</td></tr>
</table>
Activity is documented in the UKHCDO database. Previous HRG codes were: PN59 Paediatric coagulation disorder; SA02 Coagulation defect.
</td>
</tr>
<tr>
<td>A8.3 Identification Rules for Drugs:
How are drug costs captured?</td>
<td><u>Already specified in current NHS England Drugs List document</u>
If the drug has already been specified in the current NHS England Drug List please specify drug name and drug indication:
Human coagulation factor X (Coagadex) – Blood related products (BNF category 2.11) Not routinely commissioned – directly commissioned through NHSE.</td>
</tr>
<tr>
<td>A8.4 Identification Rules for Devices:
How are device costs captured?</td>
<td><u>Not applicable</u></td>
</tr>
<tr>
<td>A8.5 Identification Rules for Activity:
How are activity costs captured?</td>
<td><u>Already correctly captured by an existing specialised service line (NCBPS code within the PSS Tool)</u>
If activity costs are already captured please specify the specialised service code and description (e.g. NCBPS01C Chemotherapy).</td>
</tr>
</table>

<table>
<tr><td></td><td>NCBPS03Z Haemophilia
If activity costs are already captured please specify whether this service needs a separate code. <u>No</u>
If the activity is captured but the service line needs amendment please specify whether the proposed amendments have been documented and agreed with the Identification Rules team.
N/A
If the activity is not captured please specify whether the proposed identification rules have been documented and agreed with the Identification Rules team. <u>N/A</u></td></tr>
<tr><td colspan="2">A9 Monitoring</td></tr>
<tr><td>A9.1 Contracts
Specify any new or revised data flow or data collection requirements, needed for inclusion in the NHS Standard Contract Information Schedule.</td><td><u>None</u>
All patients should be recorded in national haemophilia database, and there is a new indicator to indicate prophylactic regimens.
Haemtrack™ could be a mandatory component to support commissioning</td></tr>
<tr><td>A9.2 Excluded Drugs and Devices (not covered by the Zero Cost Model)
For treatments which are tariff excluded drugs or devices not covered by the Zero Cost Model, specify the pharmacy or device monitoring required, for example reporting or use of prior approval systems.</td><td>Select all that apply:<table><tr><td>Drugs or Device MDS</td><td>☒</td></tr><tr><td>Blueteq</td><td>☐</td></tr><tr><td>Other prior approval</td><td>☐</td></tr></table></td></tr>
<tr><td>A9.3 Business intelligence
Is there potential for duplicate reporting?</td><td><u>No</u></td></tr>
</table>

A9.4 **Contract monitoring** Is this part of routine contract monitoring?	**Yes** All utilisation under any contracts would be reported through Haemtrak/MDSAS to UKHCDO and to CMU.
A9.5 **Dashboard reporting** Specify whether a dashboard exists for the proposed intervention?	**No**
A9.6 **NICE reporting** Are there any directly applicable NICE or equivalent quality standards which need to be monitored in association with the new policy?	**No**
Section B - Service Impact	
B1 Service Organisation	
B1.1 Describe how the service is currently organised? (i.e. tertiary centres, networked provision etc.)	Local clinical networks already exist lead by Comprehensive Care Centres. Use of human coagulation factor X may be restricted to these providers only. *Source:*
B1.2 Will the proposition change the way the commissioned service is organised?	**No** There are currently a small number of Haemophilia Centres which appear to be providing treatment for factor X deficiency which are not a Comprehensive Care Centre and this could impact on patients. In most of these cases there is a CCC in close proximity. *Source:*
B1.3 Will the proposition require a new approach to the organisation	**No change to delivery of care**

<table>
<tr><td>of care?</td><td></td></tr>
<tr><td colspan="2">B2 Geography & Access</td></tr>
<tr><td>B2.1 Where do current referrals come from?</td><td>Select all that apply:
<table><tr><td>GP</td><td>☐</td></tr><tr><td>Secondary care</td><td>☐</td></tr><tr><td>Tertiary care</td><td>☒</td></tr><tr><td>Other</td><td>☐</td></tr></table>Please specify:
Haemophilia centres</td></tr>
<tr><td>B2.2 What impact will the new policy have on the sources of referral?</td><td><u>No impact</u>
Please specify:
Click here to enter text.</td></tr>
<tr><td>B2.3 Is the new policy likely to improve equity of access?</td><td><u>Decrease</u>
Restriction on commissioned providers for issuing prophylactic treatment of factor X deficiency, although in most cases there is an alternative provider in close proximity to the existing provider. Most patients would not be affected by any change.</td></tr>
<tr><td>B2.4 Is the new policy likely to improve equality of access and/or outcomes?</td><td><u>Decrease</u>
Please see B2.3 above.</td></tr>
</table>

B3 Implementation	
B3.1 Will commissioning or provider action be required before implementation of the proposition can occur?	**No action required** Comprehensive Care Centres are already designated.
B3.2 **Time to implementation:** Is a lead-in time required prior to implementation?	**No - go to B3.4**
B3.3 **Time to implementation:** If lead-in time is required prior to implementation, will an interim plan for implementation be required?	**No - go to B3.4**
B3.4 Is a change in provider physical infrastructure required?	**No**
B3.5 Is a change in provider staffing required?	**No**
B3.6 Are there new clinical dependency and/or adjacency requirements that would need to be in place?	**No**
B3.7 Are there changes in the support services that need to be in place?	**No**
B3.8 Is there a change in provider and/or inter-provider governance required? (e.g. ODN arrangements / prime contractor)	**No**

B3.9 Is there likely to be either an increase or decrease in the number of commissioned providers? If yes, specify the current and estimated number of providers required in each region

No change : figures represent impact of restricting prescribing from all Haemophilia providers to Comprehensive Care Centres only (England)

Region	Current no. of providers	Future State expected range	Provisional or confirmed
North	10	8	C
Midlands & East	9	5	C
London	6	4	C
South	14	5	C
Total	39	22	C

B3.10 Specify how revised provision will be secured by NHS England as the responsible commissioner.

Publication and notification of new policy	☒
Market intervention required	☐
Competitive selection process to secure increase or decrease provider configuration	☐
Price-based selection process to maximise cost effectiveness	☐
Any qualified provider	☐
National Commercial Agreements e.g. drugs, devices	☒
Procurement	☐
Other	☐

B4 Place-based Commissioning	
B4.1 Is this service currently subject to, or planned for, place-based commissioning arrangements? (e.g. future CCG lead, devolved commissioning arrangements, STPs)	**No**

Section C - Finance Impact

C1 Tariff/Pricing

C1.1 How is the service contracted and/or charged?

Only specify for the relevant section of the patient pathway

Drugs	Not separately charged – part of local or national tariffs	☐
	Excluded from tariff – pass through	☐
	Excluded from tariff - other	☒
Devices	Not separately charged – part of local or national tariffs	☐
	Excluded from tariff (excluding ZCM) – pass through	☐
	Excluded from tariff (excluding ZCM) – other	☐
	Via Zero Cost Model	☐
Activity	Paid entirely by National Tariffs	☐
	Paid entirely by Local Tariffs	☐
	Partially paid by National Tariffs	☐
	Partially paid by Local Tariffs	☐
	Part/fully paid under a Block arrangement	☐

<table>
<tr><td></td><td>
<table>
<tr><td></td><td>Part/fully paid under Pass-Through arrangements</td><td>☐</td></tr>
<tr><td></td><td>Part/fully paid under Other arrangements</td><td>☒</td></tr>
</table>
</td></tr>
<tr><td>C1.2 Drug Costs
Where not included in national or local tariffs, list each drug or combination, dosage, quantity, list price including VAT if applicable and any other key information e.g. Chemotherapy Regime.
NB discounted prices or local prices must not be included as these are subject to commercial confidentiality and must not be disclosed.</td><td>The list price of human coagulation factor X is £4.20 per IU. The list price cost of each formulation of human coagulation factor X is:
<table>
<tr><td>500 IU powder and solvent for solution for injection</td><td>£2,100</td></tr>
<tr><td>250 IU powder and solvent for solution for injection</td><td>£1,050</td></tr>
<tr><td>25 IU/kg at onset of bleeding</td><td>£105</td></tr>
</table>
The prices above do not include VAT because human coagulation factor X is a plasma based product which is not subject to VAT</td></tr>
<tr><td>C1.3 Device Costs
Where not included in national or local tariff, list each element of the excluded device, quantity, list or expected price including VAT if applicable and any other key information.
NB: Discounted prices or local prices must not be included as these are subject to commercial confidentiality and must not be disclosed.</td><td>N/A</td></tr>
<tr><td>C1.4 Activity Costs covered by National Tariffs
List all the HRG codes, HRG descriptions, national tariffs (excluding MFF), volume and other key costs (e.g. specialist top up %)</td><td></td></tr>
<tr><td>C1.5 Activity Costs covered by Local Tariff</td><td></td></tr>
</table>

<table>
<tr><td>List all the HRGs (if applicable), HRG or local description, estimated average tariff, volume and any other key costs. Also indicate whether the Local Tariff(s) is/are newly proposed or established and if newly proposed how is has been derived, validated and tested.</td><td></td></tr>
<tr><td>C1.6 Other Activity Costs not covered by National or Local Tariff
Include descriptions and estimates of all key costs.</td><td>None identified.</td></tr>
<tr><td>C1.7 Are there any prior approval mechanisms required either during implementation or permanently?</td><td><u>Yes</u>
Please specify: Currently an IFR process is in place.</td></tr>
<tr><td colspan="2">C2 Average Cost per Patient</td></tr>
<tr><td>C2.1 What is the estimated cost per patient to NHS England, in years 1-5, including follow-up where required?

Are there any changes expected in year 6-10 which would impact the model?</td><td>
<table>
<tr><td></td><td>Long term prophylaxis (all ages) List price human coagulation factor X</td></tr>
<tr><td>YR1</td><td>491,112</td></tr>
<tr><td>YR2</td><td>491,112</td></tr>
<tr><td>YR3</td><td>491,112</td></tr>
<tr><td>YR4</td><td>491,112</td></tr>
<tr><td>YR5</td><td>491,112</td></tr>
</table>
There is a discounted price applicable therefore actual costs per patient are likely to be lower.

Using the assumptions in C1.4 above, the estimated total cost of other treatments for people on long term prophylaxis (assumed mean patient</td></tr>
</table>

weight is 50kg) is:

Treatment	Drug costs (List prices) £
PCC (Beriplex)	77,563
PCC (Octaplex)	63,343

Please note the above treatments are plasma based products and not subject to VAT.

C3 Overall Cost Impact of this Policy to NHS England

C3.1 Specify the budget impact of the proposal on NHS England in relation to the relevant pathway.

Cost pressure

Please specify:

Using the assumptions given in the sections above, the estimated resource impact **at list prices** is shown in the table below. The figures relate to drug costs only.

Estimated cost impact – list prices.

Year	Net resource impact £000
Year 1	12,403
Year 2	12,260
Year 5	15,296
Year 10	17,920

C3.2 If the budget impact on NHS England cannot be identified set out the reasons why this cannot be measured.	N/A. Please see C3.1.
C3.3 If the activity is subject to a change of commissioning responsibility, from CCG to NHS England, has a methodology for the transfer of funds been identified, and calculated?	No change of commissioning responsibility.
C4 Overall cost impact of this policy to the NHS as a whole	
C4.1 Specify the budget impact of the proposal on other parts of the NHS.	Budget impact for CCGs: **No impact on CCGs** Budget impact for providers: **No impact on providers** Please specify: The additional cost impact for drug treatment would fall under specialised commissioning (see table below). It is not anticipated that there will be significant cost pressure to providers over and above existing options. **Estimated cost impact – list prices. See C3.1**
C4.2 Taking into account responses to C3.1 and C4.1, specify the budget impact to the NHS as a whole.	**Cost pressure** Please specify: The figures in C3.1 show that there is resource impact to the commissioner (NHSE) from implementing the policy. The cost of human coagulation factor X is at list price, therefore the actual resource impact is likely to be lower.

C4.3 Where the budget impact is unknown set out the reasons why this cannot be measured	N/A please see C3.1 above.
C4.4 Are there likely to be any costs or savings for non-NHS commissioners and/or public sector funders?	**NO**
C5 Funding	
C5.1 Where a cost pressure is indicated, state known source of funds for investment, where identified, e.g. decommissioning less clinically or cost-effective services.	CPAG prioritisation reserve.
C6 Financial Risks Associated with Implementing this Policy	
C6.1 What are the material financial risks to implementing this policy?	While the number of people currently receiving long-term prophylaxis is known from the UKHCDO, bleed frequency and type is highly unpredictable. The frequency of treatment is therefore expected to fluctuate annually. The ideal dose/kg and frequency of dosing per person depends on body surface area and age. It is also difficult to predict whether long-term prophylactic use would increase following introduction of human coagulation factor X.
C6.2 How can these risks be mitigated?	UKHCDO data could be used to ensure human coagulation factor X is used at the correct point in the pathway, and trend analysis could be used to assess whether the correct questions are being asked to ensure proper use within the policy. The policy could be approved after publication of relevant clinical effectiveness evidence.

C6.3 What scenarios (differential assumptions) have been explicitly tested to generate best case, worst case and most likely total cost scenarios?	
C6.4 What scenario has been approved and why?	Please see C6.3 above. All prophylaxis patients in England move to treatment with human factor X
C7 Value for Money	
C7.1 What published evidence is available that the treatment is cost effective as evidenced in the evidence review?	**<u>There is no published evidence of cost-effectiveness</u>** Please specify: The clinical evidence review for this technology found no studies relating to cost effectiveness.
C7.2 Has other data been identified through the service specification development relevant to the assessment of value for money?	

Available pricing data suggests the treatment is equivalent cost compared to current/comparator treatment	☐
Available pricing data suggests the treatment is lower cost compared to current/comparator treatment	☐
Available clinical practice data suggests the new treatment has the potential to improve value for money	☐
Other data has been identified	☒
No data has been identified	☐
The data supports a high level of certainty about the impact on	☐

	value | The data does not support a high level of certainty about the impact on value | ☐ **Studies in children under 12 years**: At the time of this review there were no published studies investigating human coagulation factor X for the management of hereditary factor X deficiency in children under 12 years. The Ten02 study is an open-label, non-randomised phase III study in children aged under 12 years with moderate to severe factor X deficiency. The study investigated the efficacy and safety of routine prophylaxis with human coagulation factor X over 6 months and was presented in abstract form at the 2017 International Society on Thrombosis and Haemostasis Congress. The unpublished results from Liesner et al. (Ten02) have been taken into account by the policy working group based on a confidential draft of the article which was provided by the company. This will be published in the near future and will be available at the time a commissioning policy is considered for routine commissioning. The EPAR notes that the applicant provided an overview of the current status of the Ten02 study in their application, and that preliminary data were in line with those observed in the Ten01 study. The European public assessment report (EPAR) for human coagulation factor X (Coagadex) was also used to supplement the published data.
C8 Cost Profile	
C8.1 Are there non-recurrent capital or revenue costs associated with this policy?	**No**
C8.2 If yes, confirm the source of funds to meet these costs.	Not applicable