Integrated Impact Assessment Report for Clinical Commissioning Policies

Policy Reference Number	E03X05		
Policy Title	Dornase alfa inhaled therapy for primary ciliary dyskinesia (all ages)		
Accountable Commissioner	Bernie Stocks	**Clinical Lead**	Claire Hogg
Finance Lead	Shekh Motin	**Analytical Lead**	Ceri Townley

Section K - Activity Impact		
Theme	**Questions**	
K1 Current Patient Population & Demography / Growth	K 1.1 What is the prevalence of the disease/condition?	K1.Primary ciliary dyskinesia (PCD) is a rare inherited disorder and has a prevalence of approximately 1:26,000 to 40,000 in Europe,[i] with prevalence as high as 1:2,200 in certain ethnic backgrounds (particularly Asians).[ii] In England the number of patients with PCD is estimated at 700 to 800 in 2015 (of which 350 - 450 children and 350 adults).[iii]
	K1.2 What is the number of patients eligible for this treatment under currently routinely commissioned care arrangements?	K1.2 Dornase alfa is proposed for a subset of the prevalent population that does not respond to conventional treatments, with the aim of preventing further lung function decline (as set out in the policy proposition) or improving

		airway clearance. There could be up to 80 individuals (20 children and 60 adults) that would be eligible for the treatment in the longer term, or roughly 10% of the prevalent population [iv] It is estimated that almost 20 patients may be currently using the treatment (around 16 children and around 3 adults).[v] *[Note: The estimate of around 20 is based on a survey in September 2015 to clinicians indicating use of the treatment. Funding for dornase alfa historically may have come from individual funding requests (IFRs), or the cost may have been absorbed by trusts or through general practice.]*
	K1.3 What age group is the treatment indicated for?	K1.3 This policy would apply to children and adults (all ages).
	K1.4 Describe the age distribution of the patient population taking up treatment?	K1.4 PCD affects both children and adults. The number of adults being seen for the disease accounts for up to 50% of the current patient population (as set out in question K1.1).
	K1.5 What is the current activity for the target population covered under the new policy?	K1.5 PCD patients are currently treated with chest physiotherapy, antibiotics, hyperosmolars, and bronchodilators, and they often require hospitalisations and outpatient appointments.[vi] As set out in K1.2, around 20 patients may currently be using dornase alfa.
	K1.6 What is the projected growth of the disease/condition prevalence (prior to applying the new policy) in 2, 5, and 10 years	K1.6 The prevalence rate of PCD is not increasing, however the population with PCD will grow due to increases in the overall population, and may grow due to better recognition (as set out in K2.2). The future population of those with PCD is estimated at around: [vii] • 710 to 810 patients in 2016/17 • 720 to 820 patients in 2017/18

		• 730 to 830 patients in 2020/21 Of the prevalent population, approximately 80 would be within the eligible population as set out in K1.2, or around 10% of the prevalent population.
	K1.7 What is the associated projected growth in activity (prior to applying the new policy) in 2,5 and 10 years	K1.7 Dornase alfa is currently not routinely commissioned, and the few patients receiving this treatment are funded through a number of means.[viii] If current levels of activity in relation to dornase alfa continue, the activity in future years would remain constant at c. 20 patients per year.
	K1.8 How is the population currently distributed geographically?	K1.8 The population is distributed across England. Since the condition is more common in the Asian community as set out in K1.1 – prevalence might be higher in areas with a higher proportion of Asian population.
K2 Future Patient Population & Demography	K2.1 Does the new policy: move to a non-routine commissioning position / substitute a currently routinely commissioned treatment / expand or restrict an existing treatment threshold / add an additional line / stage of treatment / other?	K2.1 The new policy moves to a not routinely commissioned position, to reflect the clinical panel position that there was not sufficient evidence to routinely commission the use of dornase alfa in those with PCD. It is noted that the absence of evidence is not evidence of no effect.
	K2.2 Please describe any factors likely to affect growth in the patient population for this intervention (e.g. increased disease prevalence, increased survival)	K2.2 PCD is a hereditary condition,[ix] and no specific factors affecting the growth have been identified apart from population growth. The prevalent population might grow due to increased recognition, although this growth rate could not be quantified.[x]
	K 2.3 Are there likely to be changes in geography/demography of the patient population and would this impact on activity/outcomes? If yes, provide	K2.3 No evidence of changes was identified.

	details K2.4 What is the resulting expected net increase or decrease in the number of patients who will access the treatment per year in year 2, 5 and 10?	K2.4 The proposed policy establishes a 'not routinely commissioned' proposal for the relevant population (the specific cohort set out in K1.2). The number of patients who fall outside of the cohort covered by the proposed policy, or for whom exceptionality might be demonstrated is likely to be very small. The number of patients receiving the treatment would therefore be expected to decline as patients currently using the treatment would slowly stop use. Of the c. 20 patients estimated to be receiving the drug currently, around 50% are estimated to be using the drug for a limited time (treatment duration could be up to 2 years)[xi] and 50% are estimated to be using the drug on continuous basis.[xii] Based on the above, the number of patients receiving this treatment would decrease from the current number (as identified in K1.5) to around 10 – 15 patients on the drug by 2016/17. There would be no change to this over the next five years. As compared the 'do nothing' case, there will be a reduction in those accessing the treatment, estimated at: • ~ 0 - 5 fewer patients in 2016/17 than in the do nothing case • ~ 5 - 10 fewer patients in 2017/18 than in the do nothing case • ~ 10 fewer patients in 2020/21 than in the do nothing case
K3 Activity	K3.1 What is the current annual activity for the target population covered under the new policy? Please provide details in accompanying excel sheet K3.2 What will be the new activity should the new / revised policy be implemented in the target population? Please provide details in accompanying excel sheet	K3.1 Currently, patients would use chest physiotherapy, antibiotics, hyperosmolars, and bronchodilators. The current estimate is that around 20 patients use dornase alfa (see K1.5). K3.2 In line with the position set out in K2.4, the number of patients accessing the treatment in each year is estimated at: • ~15 - 20 in 2016/17 • ~10 - 15 in 2017/18

		• ~10 in 2020/21
	K3.3 What will be the comparative activity for the 'Next Best Alternative' or 'Do Nothing' comparator if policy is not adopted? Please details in accompanying excel sheet	K3.3 The 'do nothing' case would be as set out in K1.7.
K4 Existing Patient Pathway	K4.1 If there is a relevant currently routinely commissioned treatment, what is the current patient pathway? Describe or include a figure to outline associated activity. K4.2. What are the current treatment access criteria? K4.3 What are the current treatment stopping points?	K4.1 Primary ciliary dyskinesia (PCD) patients are currently treated with chest physiotherapy, antibiotics, hyperosmolars, and bronchodilators. K4.2 Existing approved treatments are provided based on severity of disorder. K4.3 There are no defined stopping points for the currently routinely commissioned treatments.
K5 Comparator (next best alternative treatment) Patient Pathway	K5.1 If there is a 'next best' alternative routinely commissioned treatment what is the current patient pathway? Describe or include a figure to outline associated activity. K5.2 Where there are different stopping points on the pathway please indicate how many patients out of the number starting the pathway would be expected to finish at each point (e.g. expected number dropping out due to side effects of drug, or number who don't continue to treatment after having test to determine likely success). If possible please indicate likely outcome for patient at each stopping point.	K5.1 Not applicable. K5.2 Not applicable.
K6 New Patient Pathway	K6.1 Describe or include a figure to outline associated activity with the patient pathway for the proposed new policy.	K6.1 & K6.2 Not applicable.

	K6.2 Where there are different stopping points on the pathway please indicate how many patients out of the number starting the pathway would be expected to finish at each point (e.g. expected number dropping out due to side effects of drug, or number who don't continue to treatment after having test to determine likely success). If possible please indicate likely outcome for patient at each stopping point.	
K7 Treatment Setting	K7.1How is this treatment delivered to the patient? o Acute Trust: Inpatient/Daycase/Outpatient o Mental Health Provider: Inpatient /Outpatient o Community setting o Homecare delivery K7.2 Is there likely to be a change in delivery setting or capacity requirements, if so what? *e.g. service capacity*	K7.1 Not applicable. It could be suitable for homecare delivery. K7.2 Not applicable.
K8 Coding	K8.1 In which datasets (e.g. SUS/central data collections etc.) will activity related to the new patient pathway be recorded? K8.2 How will this activity related to the new patient pathway be identified?(e.g. ICD10 codes/procedure codes)	K8.1 Not applicable. K8.2 Not applicable.
K9 Monitoring	K9.1 Do any new or revised requirements need to be included in the NHS Standard Contract Information Schedule? K9.2 If this treatment is a drug, what pharmacy monitoring is required? K9.3 What analytical information /monitoring/ reporting is	K9.1-K9.7 Not applicable.

	required? K9.4 What contract monitoring is required by supplier managers? What changes need to be in place? K9.5 Is there inked information required to complete quality dashboards and if so is it being incorporated into routine performance monitoring? K9.6 Are there any directly applicable NICE quality standards that need to be monitored in association with the new policy? K9.7 Do you anticipate using Blueteq or other equivalent system to guide access to treatment? If so, please outline. *See also linked question in M1 below*	
Section L – Service Impact		
Theme	**Questions**	
L1 Service Organisation	L1.1 How is this service currently organised? (i.e. tertiary centres, networked provision) L1.2 How will the proposed policy change the way the commissioned service is organised?	L1.1 Service currently provided at four PCD management centres in England. L1.2 Not applicable.
L2 Geography & Access	L2.1 Where do current referrals come from? L2.2 Will the new policy change / restrict / expand the sources of referral? L2.3 Is the new policy likely to improve equity of access? L2.4 Is the new policy likely to improve equality of access / outcomes?	L2.1 PCD Diagnostic Centres. L2.2-2.4 Not applicable.

L3 Implementation	L3.1 Is there a lead in time required prior to implementation and if so when could implementation be achieved if the policy is agreed? L3.2 Is there a change in provider physical infrastructure required? L3.3 Is there a change in provider staffing required? L3.4 Are there new clinical dependency / adjacency requirements that would need to be in place? L3.5 Are there changes in the support services that need to be in place? L3.6 Is there a change in provider / inter-provider governance required? (e.g. ODN arrangements / prime contractor) L3.7 Is there likely to be either an increase or decrease in the number of commissioned providers? L3.8 How will the revised provision be secured by NHS England as the responsible commissioner? (e.g. publication and notification of new policy, competitive selection process to secure revised provider configuration)	L3.1-3.8 Not applicable.
L4 Collaborative Commissioning	L4.1 Is this service currently subject to or planned for collaborative commissioning arrangements? (e.g. future CCG lead, devolved commissioning arrangements)?	L4. No.
Section M – Finance Impact		
Theme	**Questions**	

M1 Tariff	M1.1 Is this treatment paid under a national prices, and if so which?	M1.1 No.
	M1.2 Is this treatment excluded from national prices?	M1.2 Dornase alfa is an excluded High Cost Drug under the national tariff.
	M1.3 Is this covered under a local price arrangements (if so state range), and if so are you confident that the costs are not also attributable to other clinical services?	M1.3 The drug would be paid under local pricing. The cost of the drug is estimated at around £496 per month (excl. VAT), based on the list price.[xiii] The yearly cost per patient is set out in M2.1, and the VAT position is noted in M1.5.
	M1.4 If a new price has been proposed how has this been derived / tested? How will we ensure that associated activity is not additionally / double charged through existing routes?	M1.4 No new price is proposed.
	M1.5 Is VAT payable (Y/N) and if so has it been included in the costings?	M1.5 If homecare delivery was used, VAT would be recoverable.
	M1.6 Do you envisage a prior approval / funding authorisation being required to support implementation of the new policy?	M1.6 No.
M2 Average Cost per Patient	M2.1 What is the revenue cost per patient in year 1?	M2.1 The revenue cost per patient per year would be nil as the decision is to not routinely commission. For reference, the cost per patient per year is estimated to comprise mainly the cost of the drug itself. In Year 1, the cost per patient could be ~£5,950 for the drug plus homecare delivery (assumed at £900 per year based on 12 deliveries per year).[xiv]
	M2.2 What is the revenue cost per patient in future years (including follow up)?	M2.2 After Year 1, for those using the drug on an ongoing basis the cost could be similar to the first year at around ~£5,950 p.a. plus homecare delivery (assumed at £900 per year).

		For those using the treatment to rescue lung function, the drug may only be taken for 3-6 months in total. As such, the cost in the second year could vary, ranging between c. £0 and £5,950, plus homecare.[xv]
M3 Overall Cost Impact of this Policy to NHS England	M3.1 Indicate whether this is cost saving, neutral, or cost pressure to NHS England? M3.2 Where this has not been identified, set out the reasons why this cannot be measured?	M3.1 Cash neutral or slightly cash releasing. Compared to the 'do nothing' case as described in K1.7, there could be cash released of up to around £35,000 in 2016/17 (Year 1), or up to around £70,000 per year when the cohort reaches its new steady state under the policy (based on an estimated decrease to 10 patients using the drug every year, which could be achieved by 2017/18, as described in K3.2 and K2.4). The cash released would vary based on the exact number of those funded on different routes and the treatment duration required. M3.2 Not applicable.
M4 Overall cost impact of this policy to the NHS as a whole	M4.1 Indicate whether this is cost saving, neutral, or cost saving for other parts of the NHS (e.g. providers, CCGs) M4.2 Indicate whether this is cost saving, neutral, or cost pressure to the NHS as a whole? M4.3 Where this has not been identified, set out the reasons why this cannot be measured? M4.4 Are there likely to be any costs or savings for non NHS commissioners / public sector funders?	M4.1 Cost neutral. M4.2 Cash neutral or releasing as set out in M3.1 M4.3 Not applicable. M4.4 None identified.
M5 Funding	M5.1 Where a cost pressure is indicated, state known source of funds for investment, where identified *e.g. decommissioning less clinically or cost-effective services*	M5.1 Not applicable.
M6 Financial Risks	M6.1 What are the material financial risks to implementing this policy?	M6.1 The risks to implementing this policy are around the number of patients that are currently using dornase alfa on an ongoing basis. In particular, if the

Associated with Implementing this Policy		number of current patients using dornase alfa on a continuous basis is higher, this will lead to lower cash released. If a greater number of current patients are using the treatment for rescue, this could lead to greater cash release as existing patients discontinue use, and are not replaced with new patients to the same extent. The current funding routes used by patients on dornase alfa is part of this risk.
	M6.2 Can these be mitigated, if so how?	M6.2 Not applicable.
	M6.3 What scenarios (differential assumptions) have been explicitly tested to generate best case, worst case and most likely total cost scenarios?	M6.3 Not applicable.
M7 Value for Money	M7.1 What evidence is available that the treatment is cost effective? *e.g. NICE appraisal, clinical trials or peer reviewed literature*	M7.1 & M7.2 None.
	M7.2 What issues or risks are associated with this assessment? *e.g. quality or availability of evidence*	
M8 Cost Profile	M8.1 Are there non-recurrent capital or revenue costs associated with this policy? *e.g. Transitional costs, periodical costs*	M8.1 Not applicable.
	M8.2 If so, confirm the source of funds to meet these costs.	M8.2 Not applicable.

[i] Kuehni et. al. (2010) "Factors influencing age at diagnosis of primary ciliary dyskinesia in European children", *European Respiratory Journal* in relation to the Caucasian population. Higher estimates for the Asian population. O'Callaghan et.al. (2009) "High prevalence of primary ciliary dyskinesia in a British Asian population", *Archives of Disease in Childhood*.

[ii] Hirst et al. (2014) "Culture of Primary Ciliary Dyskinesia Epithelial Cells at Air-Liquid Interface Can Alter Ciliary Phenotype but Remains a Robust and Informative Diagnostic Aid"

[iii] Based on discussions with the policy working group, PCD may be underdiagnosed, especially in the adult population. The estimate of 700 to 800 patients relates to the current number of patients (2015). Based on discussions and correspondence with the policy working group.

[iv] Correspondence with the policy working group.

[v] The figures noted are estimates based on discussions with the policy working group. The estimate of 19 individuals (16 children and 3 adults) is based on a survey in September 2015 to clinicians indicating use of the treatment. Funding for dornase alfa historically may have come from individual funding requests (IFRs), or the cost may have been absorbed by trusts or through general practice.

[vi] See the policy proposition for further details on the patient pathway. Activity levels specific to the condition PCD were not identified in the SUS data as relevant ICD-10 codes were broad in nature, and would potentially capture activity for non-related conditions.

[vii] These figures are based on the estimated current number of patients with PCD. As PCD is hereditary and carried into adulthood, the ONS projections of overall population growth are used to estimate the population in future years. Based on discussions with the policy working group, PCD may be underdiagnosed, especially in the adult population. Increases in diagnostic rates could affect the number of identified patients; however it has not been possible to estimate the impact of such factors. Figures are rounded.

[viii] Please refer to K1.2.

[ix] Please see the policy proposition for further detail.

[x] Based on discussions and correspondence with the policy working group.

[xi] Patients currently receiving dornase alfa to rescue lung function (treatment duration of 3 to 6 months, but which can last up to 24 months) would discontinue use once their treatment duration ends. Based on discussions with the policy working group.

[xii] Half of the patients currently treated are assumed to be receiving dornase alfa on a continuous basis. Based on discussions and email correspondence with the policy working group.

[xiii] Based on 2.5 mg (in one single-use ampoule) inhaled once daily using a recommended nebulizer based on use for Cystic Fibrosis patients. Excluding VAT. Source: eMC Dictionary of Medicines and Devices Browser [Online]. Available at http://dmd.medicines.org.uk/DesktopDefault.aspx?AMPP=3769411000001106&toc=nofloat [Accessed: 09/11/2015].

[xiv] Based on discussions with NHS England, homecare costs are estimated at around £50-£100 per delivery. These costs are thought to be broadly standard per delivery. Home delivery enables the recovery of VAT on most drugs. Homecare would typically provide the patients with all required support, based on discussions with the policy working group. The estimated cost of the drug is based on a monthly cost of £496 per month (exlc. VAT) based on the Dictionary of Medicines price (see the previous footnote).

[xv] Inflation and efficiency factors have not been included.