REPORT FROM CLINICAL PANEL

Title: **E03X05**
Dornase alfa inhaled therapy for adults and children with primary ciliary dyskinesia
CRG: Paediatric Medicine
NPOC: Women and Children
Lead: Bernie Stocks

Date: 21 October 2015

The Panel were presented a policy proposal for routine commissioning

Question	Conclusion of the panel	If there is a difference between the evidence review and the policy please give a commentary
<u>The population</u> 1. What are the eligible and ineligible populations defined in the policy and are these consistent with populations for which evidence of effectiveness is presented in the evidence review?	The eligible population(s) defined in the policy are the same or similar to the population(s) for which there is evidence of effectiveness considered in the evidence review.	The evidence of effectiveness is very weak. There is insufficient evidence to identify a population likely to benefit from Dornase Alpha. (Dornase Alpha is not licensed for use in ciliary dyskinesia. It is licensed for use in cystic fibrosis
<u>Population subgroups</u> 2. Are any population subgroups defined in the policy and if so do they match the subgroups for which there is evidence presented in the evidence review?	The population subgroups defined in the policy are the same or similar as those for which there is evidence in the evidence review.	The evidence was too weak to demonstrate effectiveness in any patient subgroup or cohort of patients.

<u>Outcomes - benefits</u> 3. Are the clinical benefits demonstrated in the evidence review consistent with the eligible population and/or subgroups presented in the policy?	The clinical benefits demonstrated in the evidence review do not support the eligible population and/or subgroups presented in the policy.	Some improvements in symptoms were reported in the case reports, but there was notably very little evidence on lung function and no evidence in the case reports beyond 4 weeks. Very limited evidence of effectiveness, only very small case studies.
<u>Outcomes – harms</u> 4. Are the clinical harms demonstrated in the evidence review reflected in the eligible population and/or subgroups presented in the policy?	The clinical harms demonstrated in the evidence review are reflected in the eligible population and/or subgroups presented in the policy.	No harms were reported.
<u>The intervention</u> 5. Is the intervention described in the policy the same or similar as the intervention for which evidence is presented in the evidence review?	-	This couldn't be answered based on the lack of available evidence.
<u>The comparator</u> 1. Is the comparator in the policy the same as that in the evidence review?	-	Not applicable. There are no comparators in the evidence available.

2. Are the comparators in the evidence review the most plausible comparators for patients in the English NHS and are they suitable for informing policy development?	-	Not applicable.

Overall conclusions of the panel

The policy does not reflect the findings of the clinical evidence review, which shows that there is insufficient evidence on which to routinely commission Dornase Alfa. Ciliary Dyskinesia is a relatively rare condition but the panel view is that recognising this that the research evidence remains disproportionately and extremely limited. The panel's decision is that this policy should be changed to a 'not routinely commissioned' position and progressed to stakeholder testing and consultation on this basis.

Report approved by:

David Black
Deputy National Clinical Director Specialised Commissioning
7th December 2015