REPORT FROM CLINICAL PANEL

Title: **A01X01/01**
Ivacaftor for children aged 2-5 years with cystic fibrosis (named mutations)
CRG:
NPOC: Internal Medicine
Lead: Ursula Peaple

Date: 2 December 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.	
<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?	There is a difference between the population subgroups defined in the policy and the populations for there is evidence in the evidence review.	The evidence for efficacy is in children >6 years old. Evidence doesn't exist for children aged 2-5 but there is biological plausibility.

<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 support the eligible population and/or subgroups presented in the policy.	
<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.	
<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?	The intervention described in the policy the same or similar as in the evidence review.	
<u>The comparator</u> 1. Is the comparator in the policy the same as that in the evidence review?	Not applicable	The studies supporting the use of Ivacaftor in this cohort are non-comparator.

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 reflects the findings of the clinical evidence review and should progress

The clinical panel requested that the policy ensure alignment with the stopping criteria for >6 year olds where relevant. They noted the importance of ongoing data collection and reporting.

They also noted the importance of compliance and that the policy proposition has made arrangements to address this.

Report approved by:

Jeremy Glyde
Clinical Effectiveness Team
16th December 2015