



SUBMISSION OF COMMENTS ON GUIDELINE ON THE CLINICAL DEVELOPMENT OF MEDICINAL PRODUCTS FOR THE TREATMENT OF CYSTIC FIBROSIS

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COMMENTS FROM: International Pediatric Transplant Association (IPTA)

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GENERAL COMMENTS

Thank you for the opportunity to review this document. We think it is a very reasonable guideline for assessment of efficacy of new therapies for CF. Our one substantive suggestion is that an endpoint which examines the relationship between of the identity of airway colonizing organisms and outcome be included. This may be particularly important in the assessment of new antibiotic therapies.

A few minor comments regarding the background information is outlined below.

SPECIFIC COMMENTS ON TEXT

GUIDELINE SECTION TITLE

Table with 3 columns: Line no + paragraph no., Comment and Rationale, Proposed change (if applicable). Contains two rows of specific comments on text.

1 Where available

Page 8, sec. 1.2.4, paragraph 2	The statement about transplantation is no longer accurate as heart/lung transplant for CF is much less frequently used (most centers offer bilateral lung transplant). Based on the most recent ISHLT registry reports (J Heart Lung Transplant 2007;26:782-95, J Heart Lung Transplant 2007;26:1223-8), heart/lung transplant is much less frequently used in general. Lung transplant for CF is rarely performed in the US at this time. Overall outcomes for patients receiving bilateral lung transplant for CF are as good as or better than recipients with other underlying diseases. Although there have been some recent controversial publications about whether lung transplant for CF provides benefit for pediatric recipients, at this time lung transplant is still considered a viable option for CF patients of any age when end stage lung disease develops.	Many patients are finally listed for lung or less frequently heart/lung transplantation. If they are transplanted the outcome is the same as for patients with other chronic lung diseases.

Please feel free to add more rows if needed.

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