NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

CENTRE FOR HEALTH TECHNOLOGY EVALUATION
Highly Specialised Technologies

Consultation on Batch 57 draft remits and draft scopes and summary of comments and discussions at scoping workshops

Topic ID	Technical Adviser	Topic title
856	Raisa Sidhu	Human alpha1-proteinase inhibitor for treating emphysema

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Provisional Title	Human alpha1-proteinase inhibitor for treating emphysema		
Topic Selection ID Number	7693	**Wave / Round**	R131
TA ID Number	856		
Manufacturer	CSL Behring		
Anticipated licensing information	Marketing authorisation received in September 2015: 'Human alpha1-proteinase inhibitor (Respreeza) is indicated for maintenance treatment, to slow the progression of emphysema in adults with documented severe alpha1-proteinase inhibitor deficiency (e.g. genotypes PiZZ, PiZ (null), Pi(null, null), PiSZ). Patients are to be under optimal pharmacologic and non-pharmacologic treatment and show evidence of progressive lung disease (e.g. lower forced expiratory volume per second (FEV1) predicted, impaired walking capacity or increased number of exacerbations) as evaluated by a healthcare professional experienced in the treatment of alpha1-proteinase inhibitor deficiency.'		
Draft remit	To appraise the clinical and cost effectiveness of human alpha1-proteinase inhibitor within its marketing authorisation for treating emphysema.		
Main points from consultation	Following the consultation exercise and the scoping workshop, the Institute is of the opinion that an appraisal of human alpha1-proteinase inhibitor for treating emphysema is <u>appropriate.</u> However, this topic should be routed to the HST programme for evaluation. The proposed remit is <u>not appropriate</u>. It should be amended to: <u>To evaluate the benefits and costs of human alpha1-proteinase inhibitor within its licensed indication for treating emphysema for national commissioning by NHS England.</u> This topic was scoped in 2015 and consultees stated that existing treatments relieve symptoms but do not slow down the progression of disease. Human alpha1-proteinase inhibitor is the first treatment which is licensed for this indication and may slow the progression of the disease. Patient groups are keen to access this therapy via the NHS. Stakeholders stated that HST criteria were met, but in 2015 the technology was not eligible for HST because treatment for this condition is commissioned by CCGs. It has now been confirmed that NHS England will take over the specialised commissioning of this service from CCGs from April 2018.		
Population size	Approximately 540 people in England would be eligible for treatment with human alpha1-proteinase inhibitor. This number is based on a UK registry, which estimates that 670 people in England have emphysema caused by alpha1-antitrypsin deficiency and about 540 of these people (80%) will have clinically		

	significant emphysema that requires treatment. During the scoping workshop, a clinical expert advised that only about 50% of these 540 people would have disease severe enough to warrant treatment with human alpha1-proteinase inhibitor. However, the clinical characteristics of the group needing treatment were not clearly defined and the marketing authorisation is quite broad.
Process (TA/HST)	HST
Proposed changes to remit (in bold)	**To evaluate the benefits and costs of human alpha1-proteinase inhibitor within its marketing authorisation for treating emphysema for national commissioning by NHS England.**
Costing implications of remit change	The company estimate that the annual cost of treatment may be around £50,000 per person. Hence the total annual cost, based on 100% uptake may be around £13.5m. There would also be costs for appointments for IV administration. There are not anticipated to be savings from alternative treatments avoided however if the treatment improves quality of life there is potential for reduced use of secondary care/specialist services.
Timeliness statement	Considering that this product has received a marketing authorisation for use in the UK, publication of timely guidance will not be possible.