NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

CENTRE FOR HEALTH TECHNOLOGY EVALUATION
Highly Specialised Technologies

Consultation on Batch 44b draft remit and draft scope and summary of comments and discussions at scoping workshops

ID	Topic
868	Migalastat for treating Fabry disease

Provisional Title	Migalastat for treating Fabry disease		
Topic Selection ID Number	7281	**Wave / Round**	R104
HST ID Number	868		
Manufacturer	Amicus Therapeutics		
Anticipated licensing information	***CONFIDENTIAL INFORMATION REMOVED***		
Draft remit	To evaluate the benefits and costs of migalastat within its licensed indication for treating Fabry disease for national commissioning by NHS England.		
Main points from consultation	Following the consultation exercise and the scoping workshop, the Institute is of the opinion that an evaluation of migalastat for treating Fabry disease is appropriate. The proposed remit is appropriate. The stakeholders indicated that people with Fabry disease are interested in the development of this treatment, as it is administered orally, whereas enzyme replacement therapy (the current treatment) is administered by IV injection. However, people with Fabry disease are concerned that if they do decide to switch to migalastat then they might not be allowed to switch back. As a result, they would want assurances they can return to enzyme replacement if migalastat was not clinically effective for them.		
Population size	Approximately 152–228 people in England would be eligible for treatment with migalastat. The company expects only half of patients currently receiving enzyme replacement therapy every 2 weeks to switch to an oral tablet every other day. According to stakeholders there are approximately 450 patients with Fabry disease in the UK, with around 380 in England. If according to the company 40–60% of these will have an amenable mutation, then between 152 and 228 people in England will be eligible for treatment with migalastat. Note, however, that the Fabry population is growing at approximately 15% per year because of screening initiatives. Clinical experts indicated that people with Fabry disease are interested in the treatment, although they are cautious about the fact that if they switch from one treatment to another, they may not be allowed to switch back.		
Process (MTA/STA/HST)	HST		
Proposed changes to remit (in bold)	None		
Costing	The number of people with Fabry disease in England is		

implications of remit change	estimated at around 380. Around 40–60% are expected to have an amenable mutation, therefore between 152 and 228 people in England will be eligible for treatment with migalastat. The unit cost of migalastat is unknown, but any cost impact will depend on its cost relative to current treatment options. The cost per fortnightly dose of the comparator drugs is around £5,000. Since migalastat can be taken orally, some administration cost savings are expected, since current enzyme replacement therapies are administered by IV infusion.
Timeliness statement	Assuming that the anticipated date of the marketing authorisation is the latest date that we are aware of and the expected referral date of this topic, issuing timely guidance for this technology will be possible.